作成日：2024 年 7 月 30 日

ゾルゲンスマ点滴静注 特定使用成績調査（COAV101A11401，脊髄性筋萎縮症）の中間集計結果

最新情報に基づき，薬剤を適正かつ安全にご使用いただくために，ゾルゲンスマ点滴静注 特定使用成績調査（COAV101A11401，脊髄性筋萎縮症）の中間集計結果を，ノバルティスファーマ株式会社 医療関係者向け情報サイト（DR’s Net）に掲載致しました。

＜留意点＞

- 2020 年 5 月～2024 年 5 月の間で収集された情報です
- 中間の結果であるため，今後，結果は更新されます
- 安全性情報を掲載しています

添付文書 2024 年 5 月改訂（第 6 版）※　抜粋

【効能又は効果】

脊髄性筋萎縮症

ただし、抗 AAV9 抗体が陰性の患者に限る

【用法及び用量】

通常、体重 2.6kg 以上の患者（2 歳未満）には、1.1×10^{14} ベクターゲノム（vg）/kg を 60 分かけて静脈内に単回投与する。本品の再投与はしないこと。本品の投与液量は下記表に従い体重に基づき算出する。

患者の体重（kg）	投与液量（mL）注）
2.6 – 3.0	16.5
3.1 – 3.5	19.3
3.6 – 4.0	22
4.1 – 4.5	24.8
4.6 – 5.0	27.5
5.1 – 5.5	30.3
5.6 – 6.0	33
6.1 – 6.5	35.8
6.6 – 7.0	38.5
7.1 – 7.5	41.3
7.6 – 8.0	44
8.1 – 8.5	46.8
8.6 – 9.0	49.5
9.1 – 9.5	52.3
9.6 – 10.0	55
10.1 – 10.5	57.8
10.6 – 11.0	60.5
11.1 – 11.5	63.3
11.6 – 12.0	66
12.1 – 12.5	68.8
12.6 – 13.0	71.5
13.1 – 13.5	74.3

注）投与液量は体重幅の上限値に基づき算出した。

2 歳未満で 13.6kg 以上の患者には、体重に基づき投与液量を算出すること。

※本剤の使用に際しては，最新の添付文書をご参照ください。

当該調査単位期間終了日：2024 年 5 月 23 日

結果の概要

製造販売後調査の標題	ゾルゲンスマ点滴静注　特定使用成績調査（脊髄性筋萎縮症，COAV101A11401）[1)]
調査の課題及び目的	脊髄性筋萎縮症患者を対象としたゾルゲンスマ点滴静注の長期安全性及び 有効性を検討する。
調査デザイン	前向き，多施設共同，単群，全例調査方式，非介入の観察研究
調査項目	患者の臨床的特性，本品の投与状況，プレドニゾロン及び他 SMA 治療薬の投与状況，有害事象 [2)]，有効性（発達マイルストーン，CHOP-INTEND，HINE-2，HFMSE 等）
結果	当該調査開始日（2020 年 5 月 18 日）からデータカットオフ日（2024 年 5 月 23 日）までに 80 例が登録され，80 例の調査票データが収集された。このうち適応外症例はなく，80 例を安全性解析対象症例とした（Table 14.1.0.jpn）。 調査を継続している症例の割合は 93.8%（75 例），調査を中止した症例の割合は 6.3%（5 例）であった。中止理由は Lost to Follow-up（4 例），死亡（1 例）であった。SMA 治療歴は，本品が初回治療となる症例（OAV101 mono）は 31.3%（25 例），ヌシネルセン又はリスジプラムの短期投与後 [4)]に本品に切り替えた症例（Bridge to OAV101）の割合は 37.5%（30 例），ヌシネルセン又はリスジプラムを長期投与後 [4)]に本品に切り替えた症例（Switch to OAV101）は 20.0%（16 例）[5)]，本品投与後に初めてヌシネルセン又はリスジプラムを投与している患者（Add-on 及び Transient Add-on）は 2.6%（2 例），ヌシネルセン又はリスジプラムを投与後に本品を投与し，再びヌシネルセン又はリスジプラムを投与している症例（Combo w/OAV101）が 8.8%（7 例）[5)]であった。本調査登録後のフォローアップ期間は 1 年未満が 4 例，1 年以上 2 年未満が 24 例，2 年以上が 52 例であった（Table 14.1.1.age3.jpn）。 Demography & Medical history SMA と遺伝子診断された時点の年齢（月齢）の中央値（範囲）は 6.0 ヵ月（0 ヵ月～24 ヵ月）であった。生存運動ニューロン 2（survival motor neuron 2, SMN2）遺伝子コピー数は，2 コピーが 50.0%，3 コピーが 50.0%であった（Table 14.1.3.age3.jpn）。男児の割合は 52.5%，女児の割合は 47.5%であった（Table 14.1.2.age3.jpn）。 OAV101 Treatment 本品投与時の年齢（月齢）[3)]の中央値（範囲）は 10.0 ヵ月（0 ヵ月～24 ヵ月）であった（Table 14.1.6.age3.jpn）。データカットオフ時点の年齢（月齢）の中央値（範囲）は 45.63 ヵ月（17.3 ヵ月～71.4 ヵ月）であった（Table 14.1.2.age3.jpn）。 本品投与後にヌシネルセン又はリスジプラムを投与した症例は 11.3%（9 例）であった（ヌシネルセン 1 例，リスジプラム 8 例）[5)]。追加した理由では薬効欠如（6 例），有害事象（1 例），利用可能な代替治療（1 例），その他（1 例） であった（Table 14.1.1.age3.jpn, Listing 16.4.jpn, Table 14.1.7.txn.jpn, Table 14.1.9.txr.jpn）。なお，いずれの症例も本品投与後に発達マイルストーンの消失は確認されていない（Listing 16.4.jpn）。 グルココルチコステロイドを投与した症例は 77 例であり，その全例がプレドニゾロンを投与していた。プレドニゾロンの投与期間の中央値（範囲）は 2.71 ヵ月（1.0 ヵ月～7.5 ヵ月）であった（Table 14.1.10.cpy.jpn）。 Safety 80 例全例に有害事象が発現し，最も多く報告された有害事象は発熱 81.3%（65 例），次いでアスパラギン酸アミノトランスフェラーゼ（AST）増加が 66.3%（53 例），及びアラニンアミノトランスフェラーゼ（ALT）増加が 65.0%（52 例）であった（Table 14.3.1.1.oav.age3.jpn）。

	初発の有害事象の発現時期は，本品投与後 2 週間以内が多く，80 例中 77 例で認められた（Table 14.3.1.1.2.oav.aeptin.jpn）。 また，CTCAE Grade3 以上の有害事象の発現割合は，78.8%（63 例）であった（Table 14.3.1.0.oav.age3.jpn）。 副作用の発現割合は 98.8%（79 例）であり，最も多く報告された副作用は発熱が 80.0%（64 例），次いで AST 増加が 66.3%（53 例），及び ALT 増加が 62.5%（50 例）であった（Table 14.3.1.4.oav.age3.jpn）。 重篤な有害事象の発現割合は 63.8%（51 例）であり，最も多く報告された重篤な有害事象は，肺炎が 13.8%（11 例），次いで誤嚥性肺炎が 12.5%（10 例），及び AST 増加が 11.3%（9 例）であった（Table 14.3.1.3.oav.age3.jpn）。 重篤な副作用の発現割合は 22.5%（18 例）であった。最も多く報告された重篤な副作用は，AST 増加が 11.3%（9 例），次いで ALT 増加が 7.5%（6 例），及び血栓性微小血管症（TMA）が 5.0%（4 例）であった（Table 14.3.1.5.oav.age3.jpn）。 死亡した症例は 1 例で主な死因は呼吸不全であった（Listing 16.3.jpn）。本症例は，本品投与後 482 日に心肺停止を発現し，同日死亡した。本品との因果関係は否定された。（Listing 16.1.jpn）。 特に注目すべき有害事象の発現割合は，肝障害が 87.5%（70 例），一過性の血小板減少症が 62.5%（50 例），TMA が 5.0%（4 例），心臓関連有害事象が 32.5%（26 例），神経障害の新規発現が 2.5%（2 例），自己免疫疾患の新規発現が 1.3%（1 例），血液疾患の新規発現が 16.3%（13 例）であった。なお，特に注目すべき有害事象のうち，神経節障害に関連する感覚異常，悪性腫瘍の新規発現の報告は認められなかった（Table 14.3.1.6.1.age3.jpn）。 特に注目すべき副作用の発現割合は，肝障害が 86.3%（69 例），一過性の血小板減少症が 62.5%（50 例），TMA が 5.0%（4 例），心臓関連有害事象が 31.3%（25 例），神経障害の新規発現が 1.3%（1 例），自己免疫疾患の新規発現が 1.3%（1 例），血液疾患の新規発現が 16.3%（13 例）であった（Table 14.3.1.6.2.age3.jpn）。特に注目すべき副作用のうち，重篤な副作用として報告されたのは肝障害が 12 例，一過性の血小板減少症が 5 例，TMA が 4 例，心臓関連有害事象が 4 例（TMA4 例と同一症例），血液疾患の新規発現が 6 例（TMA4 例，一過性の血小板減少症 4 例と同一症例）であった。1 例（TMA，心臓関連有害事象，血液疾患の新規発現）は回復したが後遺症ありで報告されているが，それ以外の症例はすべて回復した（Listing 16.1.2.jpn）。 Effectiveness 有効性の結果は再審査結果通知書受領後の本報告書に記載する。
結論	本調査は実施中であり，本品の長期的な安全性を結論づけることは困難であるものの，報告された副作用の特性は，本品投与後に発現が予測される事象が主であり，安全性検討事項を含め新たな安全性の懸念はなかった。今後も本品の安全性情報を収集し，新たな懸念事項が認められた場合には適切な措置を講じることとする。 有効性の結果は再審査結果通知書受領後の本報告書に記載する。
備考	添付資料 1：解析結果 添付資料 2：AESI 定義

CHOP INTEND：Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders
HFMSE：Hammersmith Functional Motor Scale Expanded
HINE-2：Hammersmith Infant Neurological Examination – Section 2
1) 本調査はSMAと診断された患者を対象とした長期観察の疾患レジストリであるRESTORE（ClinicalTrials.gov Identifier: NCT04174157）の一部として日本で実施している。
2) 本品投与開始後12ヵ月後まではすべての有害事象を収集し，残りの調査期間中は，重篤な有害事象，特に注目すべき有害事象［肝障害，神経節障害に関連する感覚異常，心臓関連有害事象，一過性の血小板減少症，血栓性微小血管症（TMA），遺伝子治療に関連する遅発性の有害事象（悪性腫瘍の新規発現，自己免疫疾患の新規発現，神経障害の新規発現，血液疾患の新規発現）］，非重篤な副作用のみ収集
3) 本品投与時月齢が24ヵ月を超えた結果が示されているが，実際には24ヵ月齢未満で投与されていることを確認している。本品投与時月齢を算出するための情報が不完全である場合，データが補完されている。

4) 短期間；ヌシネルセン：負荷投与まで，リスジプラム：3ヵ月投与まで，長期間；ヌシネルセン：維持期の投与，リスジプラム：3ヵ月を超えた投与
5) 1694-201 は本品投与前及び投与後にリスジプラムを投与していたが，死亡前日までの投与であったため Combo w/OAV101 ではなく，Switch to OAV101 として集計された。

Listing 16.1.2.jpn	Listing of Related Serious Adverse Events Japan OAV101 Treated Patients
Listing 16.1.jpn	Listing of Adverse Events of Special Interest Japan OAV101 Treated Patients
Listing 16.2.jpn	Listing SMA Japan OAV101 Treated Patients that were Screened for SMA as a Newborn
Listing 16.3.1.jpn	Listing of Tracheostomy History Japan OAV101 Treated Patients
Listing 16.3.jpn	Listing of Patient Deaths Japan OAV101 Treated Patients
Listing 16.4.1.jpn	Listing of Laboratory Values at Baseline Japan OAV101 Treated Patients
Listing 16.4.2.jpn	Listing of Clinically Significant Laboratory Values Japan OAV101 Treated Patients
Listing 16.4.jpn	Listing of Lack of Drug Effect Japan OAV101 Treated Patients that Switched Treatment due to Lack of Drug Effect
[redacted]	[redacted]
[redacted]	[redacted]
Listing 16.10.jpn	Listing of Lost to Follow-Up Patients Japan OAV101 Treated Patients
Listing 16.11.jpn	Listing of Patients Excluded from the Japan Safety Analysis Set All Japan Enrolled Patients
[redacted]	[redacted]
Listing 16.13.jpn	Listing of Japan OAV101 Treated Patients Japan OAV101 Treated Patients
Table 14.1.0.jpn	Analysis Datasets All Japan Enrolled Patients
Table 14.1.1.age3.jpn	Patient Enrollment and Disposition by Age at OAV101 Infusion Japan Analysis Set
[redacted]	[redacted]
Table 14.1.2.age3.jpn	Patient Demographics by Age at OAV101 Infusion Japan OAV101 Treated Patients
[redacted]	[redacted]
Table 14.1.3.age3.jpn	SMA Medical History by Age at OAV101 Infusion Japan OAV101 Treated Patients
[redacted]	[redacted]
Table 14.1.6.age3.jpn	OAV101 Treatment by Age at OAV101 Infusion Japan OAV101 Treated Patients
[redacted]	[redacted]
Table 14.1.6.g5tx.jpn	OAV101 Treatment by Therapy at OAV101 Infusion Japan OAV101 Treated Patients
[redacted]	[redacted]
Table 14.1.7.cpy.jpn	Nusinersen Treatment by Number of Copies of the SMN2 Gene Japan OAV101 Treated Patients
[redacted]	[redacted]
Table 14.1.7.txn.jpn	Nusinersen Treatment by Therapy Japan OAV101 Treated Patients
[redacted]	[redacted]
Table 14.1.8.age3.jpn	AAV9 Antibody Testing Results by Age at OAV101 Infusion Japan OAV101 Treated Patients
Table 14.1.9.cpy.jpn	Risdiplam Treatment by Number of Copies of the SMN2 Gene Japan OAV101 Treated Patients
[redacted]	[redacted]
Table 14.1.9.txr.jpn	Risdiplam Treatment by Therapy Japan OAV101 Treated Patients
[redacted]	[redacted]

Table 14.1.10.cpy.jpn	Glucocorticosteroid Treatment by Number of Copies of the SMN2 Gene Japan OAV101 Treated Patients
[redacted]	[redacted]
Table 14.3.1.0.oav.age3.jpn	Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion Japan OAV101 Treated Patients
Table 14.3.1.1.2.oav.aeptin.jpn	OAV101 Treatment Emergent Adverse Events Incidence of First Event Japan OAV101 Treated Patients
Table 14.3.1.1.oav.age3.jpn	OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.2.1.oav.age3.jpn	OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.2.2.oav.age3.jpn	OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.3.oav.age3.jpn	Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.4.oav.age3.jpn	OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.5.oav.age3.jpn	OAV101 Related Serious Treatment Emergent Adverse Events by Age at OAV101 Infusion Japan Safety Analysis Set

Table 14.3.1.6.1.age3.jpn	Events of Special Interest by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.6.2.age3.jpn	OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.6.3.age3.jpn	Serious Events of Special Interest by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.1.6.4.age3.jpn	OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.2.age3.jpn	Clinically Significant Abnormal Laboratory Results any Time During Follow-up by Age at OAV101 Infusion Japan Safety Analysis Set
Table 14.3.3.jpn	Patients with Elevated AST or ALT After OAV101 Infusion Japan Safety Analysis Set

Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 70	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 70	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Proteinuria/ Proteinuria/ Renal and urinary disorders	[redacted] 7	[redacted] Yes/ -	Grade 3/ Related to OAV101/ None/ Not recovered/not resolved	Yes/ 2, 3, 6

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Transient hrombocytopenia	Hematuria/ Haematuria/ Renal and urinary disorders	[redacted] 7	[redacted] No/ 35	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	[redacted] 7	[redacted] No/ 42	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE					vomit/ Vomiting/ Gastrointestinal disorders	[redacted] 3	[redacted] No/ 5	Grade 2/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]				fever/ Pyrexia/ General disorders and administration site conditions	[redacted] 4	[redacted] No/ 5	Grade 2/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	Increased ALT/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 359	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	Increased AST/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 359	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 158	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 162	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 72	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			New Incidence of Hematological Disorder	haemolytic Anemia/ Haemolytic anaemia/ Blood and lymphatic system disorders	[redacted] 6	[redacted] No/ 30	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	[redacted] 6	[redacted] No/ 31	Grade 4/ Related to OAV101/ None/ Recovered/resolved with sequelae	Yes/ 2, 3, 4, 6
RESTORE				Transient Thrombocytopenia	platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 34	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 4, 6

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

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RESTORE	[redacted]				acute kidney injury/ Acute kidney injury/ Renal and urinary disorders	[redacted] 7	[redacted] Yes/ -	Grade 4/ Related to OAV101/ None/ Not recovered/not resolved	Yes/ 2, 3, 4, 6
RESTORE				Cardiac	Cardiac failure congestive/ Cardiac failure congestive/ Cardiac disorders	[redacted] 15	[redacted] No/ 21	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE				Hepatotoxicity	AST elevated./ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 8	[redacted] No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Pyrexia/ Pyrexia/ General disorders and administration site conditions	[redacted] 4	[redacted] No/ 5	Grade 2/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/liver enzymes increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 22	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

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RESTORE	[redacted]			Hepatotoxicity	ALT increased/liver enzymes increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 22	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Ferritin high/ Serum ferritin increased/ Investigations	[redacted] 6	[redacted] No/ 22	Grade 2/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST elevated/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 7	Grade 1/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

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RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 13	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 17	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Transient Thrombocytopenia	PLT decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 17	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	Thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	[redacted] 7	[redacted] No/ 34	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3
RESTORE				Hepatotoxicity	elevated liver enzyme/ Hepatic enzyme increased/ Investigations	[redacted] 6	[redacted] No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 6	[redacted] No/ 20	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 4	[redacted] No/ 9	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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Listing 16.1.2.jpn
Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]				Renal impairment/ Renal impairment/ Renal and urinary disorders	[redacted] 11	[redacted] No/ 16	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	Thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	[redacted] 11	[redacted] No/ 16	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE					Acute liver disorder(Grade 4)	[redacted] 5	[redacted] No/ 78	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Listing of Related Serious Adverse Events
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]				Multi organ failure/ Multiple organ dysfunction syndrome/ General disorders and administration site conditions	[redacted] 51	[redacted] No/ 71	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2
RESTORE				Hepatotoxicity	Acute liver failure/ Acute hepatic failure/ Hepatobiliary disorders	[redacted] 52	[redacted] No/ 73	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3

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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	plt decreased/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 13	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 70	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 70	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 189	[redacted] No/ 203	Grade 2/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 189	[redacted] No/ 203	Grade 2/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 230	[redacted] No/ 267	Grade 2/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 230	[redacted] No/ 267	Grade 2/ Unrelated/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE	[redacted]	[redacted]	[redacted]	Cardiac	Hypertension/ Hypertension/ Vascular disorders	[redacted] 7	[redacted] No/ 28	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Hematuria/ Haematuria/ Renal and urinary disorders	[redacted] 7	[redacted] No/ 35	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	[redacted] 7	[redacted] No/ 42	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6

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Japan OAV101 Treated Patients

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RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	Bleeding from the peritoneal tube insertion site/ Medical device site haemorrhage/ General disorders and administration site conditions	[redacted] 15	[redacted] No/ 16	Grade 3/ Unrelated/ None/ Recovered/resolved	No
RESTORE				New Incidence of Neurological Disorder	Cerebral atrophy/ Cerebral atrophy/ Nervous system disorders	[redacted] 21	[redacted] No/ 210	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Increased ALT/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 359	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Japan OAV101 Treated Patients

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RESTORE	[redacted]			Hepatotoxicity	Increased AST/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 359	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Transient Thrombocytopenia	decreased platelet count/ Platelet count decreased/ Investigations	[redacted] 7	[redacted] No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 158	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 162	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Transient Thrombocytopenia	Platelet decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	hepatic function abnormal/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 4	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 72	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE	[redacted]	[redacted]	[redacted]	Cardiac	NT-proBNP increased/ N-terminal prohormone brain natriuretic peptide increased/ Investigations	[redacted] 5	[redacted] No/ 20	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 105	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	hepatic enzyme increased/ Hepatic enzyme increased/ Investigations	[redacted] 4	[redacted] No/ 395	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 6	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			New Incidence of Hematological Disorder	haemolytic Anemia/ Haemolytic anaemia/ Blood and lymphatic system disorders	[redacted] 6	[redacted] No/ 30	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	[redacted] 6	[redacted] No/ 31	Grade 4/ Related to OAV101/ None/ Recovered/resolved with sequelae	Yes/ 2, 3, 4, 6
RESTORE				Transient Thrombocytopenia	platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 34	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 4, 6

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Cardiac	CPK increased/ Blood creatine phosphokinase increased/ Investigations	[redacted] 6	[redacted] No/ 57	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 330	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 330	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	hematuria/ Haematuria/ Renal and urinary disorders	[redacted] 7	[redacted] No/ 169	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Cardiac	hypertension/ Hypertension/ Vascular disorders	[redacted] 8	[redacted] Yes/ -	Grade 2/ Related to OAV101/ None/ Not recovered/not resolved	No
RESTORE				Cardiac	cardiac troponin T increased/ Troponin T increased/ Investigations	[redacted] 14	[redacted] No/ 57	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Cardiac	cardiac troponinI increased/ Troponin I increased/ Investigations	[redacted] 14	[redacted] No/ 57	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Cardiac	Cardiac failure congestive/ Cardiac failure congestive/ Cardiac disorders	[redacted] 15	[redacted] No/ 21	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3, 6

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 14	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	CPK increased/ Blood creatine phosphokinase increased/ Investigations	[redacted] 7	[redacted] No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	[redacted] 7	[redacted] No/ 14	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 56	[redacted] No/ 133	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 56	[redacted] No/ 133	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Transient Thrombocytopenia	platelet decreased/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 11	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 2	[redacted] No/ 127	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Plate decreased/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 16	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST elevated./ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT elevated./ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 6	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT elevated/ Alanine aminotransferase increased/ Investigations	[redacted] 22	[redacted] No/ 114	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST elevated./ Aspartate aminotransferase increased/ Investigations	[redacted] 22	[redacted] No/ 114	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST elevated./ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	ALT elevated/ Alanine aminotransferase increased/ Investigations	[redacted] 8	[redacted] No/ 15	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 8	[redacted] No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Cardiac	Elevated troponin I level/ Troponin I increased/ Investigations	[redacted] 8	[redacted] No/ 57	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				New Incidence of Hematological Disorder	anemia/ Anaemia/ Blood and lymphatic system disorders	[redacted] 392	[redacted] No/ 448	Grade 1/ Unrelated/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST increased/liver enzymes increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 22	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	ALT increased/liver enzymes increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 22	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	ALT elevated/ Alanine aminotransferase increased/ Investigations	[redacted] 43	[redacted] No/ 85	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST elevated/ Aspartate aminotransferase increased/ Investigations	[redacted] 43	[redacted] No/ 85	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST elevated/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 7	Grade 1/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Hepatotoxicity	ALT elevated/ Alanine aminotransferase increased/ Investigations	[redacted] 70	[redacted] No/ 252	Grade 1/ Unrelated/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST elevated/ Aspartate aminotransferase increased/ Investigations	[redacted] 70	[redacted] No/ 252	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 57	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 15	[redacted] No/ 71	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

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RESTORE	[redacted]			Cardiac	CPK increased/ Blood creatine phosphokinase increased/ Investigations	[redacted] 5	[redacted] No/ 8	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 13	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Transient Thrombocytopenia	PLT decreased/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 16	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 17	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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RESTORE	[redacted]			Cardiac	troponin I increased/ Troponin I increased/ Investigations	[redacted] 13	[redacted] No/ 19	Grade 1/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	elevation of hepatic devitalizing enzymes/ Hepatic enzyme increased/ Investigations	[redacted] 138	[redacted] No/ 341	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Elevated AST (liver disorder)/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 436	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Japan OAV101 Treated Patients

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RESTORE	[redacted]			Hepatotoxicity	Elevated ALT (liver disorder)/ Alanine aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 226	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 9	[redacted] No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	increased/ALT increased again/elevations in serum amino transferases concentrations"/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 71	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	increased/AST increased again/elevations in serum amino transferases concentrations/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 71	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 8	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity, Cardiac	Hepatomegaly -2 transverse finger palpable, no splenomegaly/ Hepatomegaly/ Hepatobiliary disorders	[redacted] 4	[redacted] No/ 4	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	Hepatic function disorder (AST, ALT increased)/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 6	[redacted] No/ 15	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 6	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Hepatic function abnormal/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 29	[redacted] No/ 657	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Bleeding from gastric mucosa/ Gastric haemorrhage/ Gastrointestinal disorders	[redacted] 36	[redacted] No/ 64	Grade 1/ Unrelated/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
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Japan OAV101 Treated Patients

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RESTORE	[redacted]			New Incidence of Hematological Disorder	eosinophilia/ Eosinophilia/ Blood and lymphatic system disorders	[redacted] 1343	[redacted] Yes/ -	Grade 3/ Unrelated/ None/ Not recovered/not resolved	Yes/ 3
RESTORE				Transient Thrombocytopenia	Plt decreased/ Platelet count decreased/ Investigations	[redacted] 4	[redacted] No/ 11	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 11	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Japan OAV101 Treated Patients

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RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 67	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	platelet decreased/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 8	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

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RESTORE	[redacted]			Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 10	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 19	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	hepatic enzyme increased/ Hepatic enzyme increased/ Investigations	[redacted] 3	[redacted] No/ 21	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

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RESTORE	[redacted]			Transient Thrombocytopenia	Platelet decreased/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	troponin I increased/ Troponin I increased/ Investigations	[redacted] 6	[redacted] No/ 28	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	troponin I increased/ Troponin I increased/ Investigations	[redacted] 42	[redacted] No/ 55	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Cardiac	CK increased/ Blood creatine phosphokinase increased/ Investigations	[redacted] 8	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 8	[redacted] No/ 43	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 29	[redacted] No/ 43	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 3	[redacted] No/ 22	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 22	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	PLT decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Troponin I increased/ Troponin I increased/ Investigations	[redacted] 8	[redacted] No/ 71	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	elevated AST/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 127	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	elavated ALT/ Alanine aminotransferase increased/ Investigations	[redacted] 43	[redacted] No/ 127	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 108	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 108	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				New Incidence of Autoimmune Disorder	hypersensitivity/ Hypersensitivity/ Immune system disorders	[redacted] 14	[redacted] No/ 45	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Troponin I increased/ Troponin I increased/ Investigations	[redacted] 2	[redacted] No/ 168	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 13	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Nocturnal Dyspnea/ Nocturnal dyspnoea/ Respiratory, thoracic and mediastinal disorders	[redacted] 504	[redacted] No/ 506	Grade 1/ Unrelated/ None/ Recovered/resolved	Yes/ 3

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Japan OAV101 Treated Patients

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RESTORE	[redacted]			Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 28	[redacted] No/ 65	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 43	[redacted] No/ 57	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 50	[redacted] No/ 57	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Japan OAV101 Treated Patients

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RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 4	[redacted] No/ 22	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 89	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 32	[redacted] No/ 89	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 50	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 50	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	Hepatic function disorder/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 407	[redacted] No/ 435	Grade 2/ Unrelated/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 19	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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RESTORE	[redacted]			Cardiac	Cardiac troponin I increased/ Troponin I increased/ Investigations	[redacted] 6	[redacted] No/ 33	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 61	[redacted] No/ 784	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 166	[redacted] No/ 196	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 122	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 122	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Cardiac	Creatine phosphokinase increased/ Blood creatine phosphokinase increased/ Investigations	[redacted] 100	[redacted] No/ 162	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 14	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Plt decreased/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 21	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 20	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 20	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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RESTORE	[redacted]			Cardiac	elevated Troponin-I/ Troponin I increased/ Investigations	[redacted] 11	[redacted] No/ 41	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 27	[redacted] No/ 83	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 27	[redacted] No/ 83	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increase/ Aspartate aminotransferase increased/ Investigations	[redacted] 8	[redacted] No/ 89	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 8	[redacted] No/ 229	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Cardiac	CK-MB increased/ Blood creatine phosphokinase MB increased/ Investigations	[redacted] 27	[redacted] No/ 166	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increase/ Aspartate aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 132	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	GPT increase/ Alanine aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 132	Grade 3/ Unrelated/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	GGT increased/ Gamma-glutamyltransferase increased/ Investigations	[redacted] 23	[redacted] No/ 28	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	[redacted] 4	[redacted] No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 228	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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RESTORE	[redacted]			Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 228	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	GGT increased/ Gamma-glutamyltransferase increased/ Investigations	[redacted] 20	[redacted] No/ 53	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 14	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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RESTORE	[redacted]			Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 21	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Cardiac troponin I increased/ Troponin I increased/ Investigations	[redacted] 14	[redacted] No/ 40	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 40	[redacted] No/ 237	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 40	[redacted] No/ 237	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 126	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 63	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 12	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 22	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	PLT decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 17	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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RESTORE	[redacted]			Hepatotoxicity	AST elevation/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	CK elevation/ Blood creatine phosphokinase increased/ Investigations	[redacted] 6	[redacted] No/ 22	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 11	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Tn I elevation/ Troponin I increased/ Investigations	[redacted] 8	[redacted] No/ 83	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 48	[redacted] No/ 83	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	elavation of liver function enzyme(AST/ALT), and y-GTP/ Hepatic enzyme increased/ Investigations	[redacted] 5	[redacted] No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	increased troponin -I/ Troponin I increased/ Investigations	[redacted] 5	[redacted] No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 23	[redacted] No/ 112	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 23	[redacted] No/ 189	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 291	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 7	[redacted] No/ 14	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 291	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	plt decreased/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 120	Grade 4/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 120	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Plt decreased/ Platelet count decreased/ Investigations	[redacted] 7	[redacted] No/ 120	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	CK increased/ Blood creatine phosphokinase increased/ Investigations	[redacted] 78	[redacted] Yes/ -	Grade 2/ Related to OAV101/ None/ Not recovered/not resolved	No

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RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	elevation hepatic enzyme/ Hepatic enzyme increased/ Investigations	[redacted] 8	[redacted] No/ 22	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Cardiac troponin I increased/ Troponin I increased/ Investigations	[redacted] 15	[redacted] No/ 162	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	[redacted] 4	[redacted] No/ 21	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 357	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	Thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	[redacted] 7	[redacted] No/ 34	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Cardiac	arrhythmia/ Arrhythmia/ Cardiac disorders	[redacted] 25	[redacted] No/ 25	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 35	[redacted] No/ 357	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 50	[redacted] No/ 70	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 324	[redacted] No/ 330	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	platelets decreased/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Creatine kinase increased/ Blood creatine phosphokinase increased/ Investigations	[redacted] 6	[redacted] No/ 15	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	elevated liver enzyme/ Hepatic enzyme increased/ Investigations	[redacted] 6	[redacted] No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Cardiac	bradycardia/ Bradycardia/ Cardiac disorders	[redacted] 8	[redacted] No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	platelets decreased/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 10	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 6	[redacted] No/ 20	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 65	[redacted] No/ 79	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 29	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 29	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 16	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	ALT mildly increased/ Alanine aminotransferase increased/ Investigations	[redacted] 56	[redacted] No/ 99	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	AST mildly increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 56	[redacted] No/ 99	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
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Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	ALT elevation/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST elevation/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 13	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 4	[redacted] No/ 9	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3

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Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 638	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 295	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Haemoglobin decreased/ Haemoglobin decreased/ Investigations	[redacted] 6	[redacted] No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6
RESTORE				Cardiac	Blood pressure increased/ Blood pressure increased/ Investigations	[redacted] 7	[redacted] No/ 15	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelets decreased/ Platelet count decreased/ Investigations	[redacted] 7	[redacted] No/ 18	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 7	[redacted] No/ 85	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 6

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Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]	[redacted]	[redacted]	Thrombotic Microangiopathy, Cardiac, New Incidence of Hematological Disorder	Thrombotic microangiopathy/ Thrombotic microangiopathy/ Blood and lymphatic system disorders	[redacted] 11	[redacted] No/ 16	Grade 3/ Related to OAV101/ None/ Recovered/resolved	Yes/ 3
RESTORE				Transient Thrombocytopenia	Hb decreased/ Haemoglobin decreased/ Investigations	[redacted] 11	[redacted] No/ 29	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increase/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 74	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST increase/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 123	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	PLT decrease/ Platelet count decreased/ Investigations	[redacted] 6	[redacted] No/ 11	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Cardiac	Troponin I increase/ Troponin I increased/ Investigations	[redacted] 15	[redacted] No/ 158	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Transient Thrombocytopenia	platelet decrease/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 15	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 7	[redacted] No/ 17	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Hepatic enzyme increased/ Hepatic enzyme increased/ Investigations	[redacted] 7	[redacted] No/ 42	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE	[redacted]			Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 4	[redacted] No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Alanine aminotransferase increased/ Alanine aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 319	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Aspartate aminotransferase increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 5	[redacted] No/ 319	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Japan OAV101 Treated Patients

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RESTORE	[redacted]			Hepatotoxicity	gamma-glutamyl transpeptidase increased/ Gamma-glutamyltransferase increased/ Investigations	[redacted] 15	[redacted] No/ 30	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 139	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 6	[redacted] No/ 139	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	Plt decreased/ Platelet count decreased/ Investigations	[redacted] 9	[redacted] No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Hepatic function abnormal/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 5	[redacted] No/ 14	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 14	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia, New Incidence of Hematological Disorder	Thrombocytopenia/ Thrombocytopenia/ Blood and lymphatic system disorders	[redacted] 6	[redacted] No/ 10	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	Hepatic function disorder/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 29	[redacted] No/ 76	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 2	[redacted] No/ 79	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No

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RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	decreasing platelet count/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 6	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Hepatotoxicity	liver dysfunction/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 5	[redacted] Yes/ -	Grade 3/ Related to OAV101/ None/ Not recovered/not resolved	No
RESTORE	[redacted]	[redacted]	[redacted]	Transient Thrombocytopenia	platlet decreased/ Platelet count decreased/ Investigations	[redacted] 5	[redacted] No/ 12	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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Japan OAV101 Treated Patients

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RESTORE	[redacted]			Transient Thrombocytopenia	platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 13	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 8	[redacted] No/ 57	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 8	[redacted] No/ 57	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

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Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
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5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Transient Thrombocytopenia	platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 4	[redacted] No/ 16	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 29	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 4	[redacted] No/ 29	Grade 3/ Related to OAV101/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Cardiac	Troponin T increased/ Troponin T increased/ Investigations	[redacted] 5	[redacted] Yes/ -	Grade 1/ Related to OAV101/ None/ Not recovered/not resolved	No
RESTORE				Transient Thrombocytopenia	Platelet count decreased/ Platelet count decreased/ Investigations	[redacted] 8	[redacted] No/ 10	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 8	[redacted] No/ 22	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 8	[redacted] No/ 22	Grade 2/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	ALT increased/ Alanine aminotransferase increased/ Investigations	[redacted] 71	[redacted] No/ 83	Grade 3/ Unrelated/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	AST increased/ Aspartate aminotransferase increased/ Investigations	[redacted] 71	[redacted] No/ 83	Grade 2/ Unrelated/ None/ Recovered/resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Hepatotoxicity	Hepatic function abnormal/ Hepatic function abnormal/ Hepatobiliary disorders	[redacted] 4	[redacted] No/ 22	Grade 1/ Related to OAV101/ None/ Recovered/resolved	No
RESTORE				Hepatotoxicity	Acute liver failure/ Acute hepatic failure/ Hepatobiliary disorders	[redacted] 52	[redacted] No/ 73	Grade 4/ Related to OAV101/ None/ Recovered/resolved	Yes/ 2, 3
RESTORE				Cardiac	Cardio-respiratory arrest/ Cardio-respiratory arrest/ Cardiac disorders	[redacted] 269	[redacted] No/ 351	Grade 4/ Unrelated/ None/ Recovered/resolved	Yes/ 2, 3
RESTORE				New Incidence of Neurological Disorder	seizure/ Seizure/ Nervous system disorders	[redacted] 275	[redacted] Yes/ -	Grade 3/ Unrelated/ None/ Not recovered/not resolved	No

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.1.jpn
Listing of Adverse Events of Special Interest
Japan OAV101 Treated Patients

Source	Patient	Therapy[a] Start Date	Therapy[a] Stop Date	Type of AESI	Verbatim Term/ Preferred Term/ SOC	Start Date/ Days from OAV101 Infusion	Stop Date/ Ongoing/ Days from OAV101 Infusion	CTCAE Grade/ Relationship/ Action Taken/ Outcome	Serious/ SAE Criteria
RESTORE	[redacted]			Cardiac	Cardio-respiratory arrest/ Cardio-respiratory arrest/ Cardiac disorders	[redacted] 482	[redacted] No/ 482	Grade 5/ Unrelated/ None/ Fatal	Yes/ 1

[a] A = OAV101, N = Nurinersen, R = Rispladem, B = BSC Therapy
Note: SAE Criteria 1 = Results in death, 2 = It is immediately life-threatening,
3 = It requires in-patient hospitalization or prolongation of existing hospitalization, 4 = It results in persistent or significant disability or incapacity,
5 = Results in a congenital abnormality or birth defect, 6 = It is an important medical event that may jeopardize the patient or may require medical intervention to prevent one of the outcomes listed above.
AESI = Adverse Events of Special Interest, CTCAE = Common Terminology Criteria for Adverse Events (version 4.03), SOC = System Organ Class, SAE = Serious Adverse Event.

Listing 16.2.jpn
Listing SMA
Japan OAV101 Treated Patients that were Screened for SMA as a Newborn

Patient	Therapy Group	SMN2 Copy Number	Displays SMA Symptoms at Diagnosis?	SMA Symptoms	Date of Genetically Confirmed SMA Diagnosis	Age at SMA Diagnosis (Months)	Displays SMA Symptoms at Enrollment?
[redacted]	Bridge to OAV101	2 Copies	Yes	Hypotonia, Pneumonia or respiratory symptoms	[redacted]	1	Yes
[redacted]	Bridge to OAV101	2 Copies	No		[redacted]	1	No
[redacted]	Bridge to OAV101	3 Copies	No		[redacted]	3	No
[redacted]	Bridge to OAV101	3 Copies	Yes	Hypotonia, Limb weakness, Tongue fasciculations, Swallowing or feeding difficulties	[redacted]	2	Yes
[redacted]	Combo w/OAV101	2 Copies	Yes	Hypotonia, Limb weakness	[redacted]	0	Yes
[redacted]	OAV101 mono	3 Copies	No		[redacted]	1	No
[redacted]	OAV101 mono	2 Copies	No		[redacted]	1	Yes
[redacted]	OAV101 mono	3 Copies	No		[redacted]	1	No
[redacted]	OAV101 mono	3 Copies	No		[redacted]	0	No
[redacted]	Switch to OAV101	3 Copies	No		[redacted]	0	No

Listing 16.3.1.jpn
Listing of Tracheostomy History
Japan OAV101 Treated Patients

Patient	Therapy Group	SMN2 Copy Number	Age At OAV101 Therapy (Months)	Time To Tracheostomy From First Therapy (Months)	Age At Tracheostomy (Months)	Age At Data Cut (Months)	Reason for Tracheostomy	Ongoing?
[redacted]	Bridge to OAV101	2 Copies	7	1.1	2.9	54.3	Progression of disease without acute cause	Yes
[redacted]	Bridge to OAV101	2 Copies	7	2.1	5.1	35.5	Pneumonia	Yes
[redacted]	Combo w/OAV101	2 Copies	4	7.8	10.5	29.2	Pneumonia	Yes
[redacted]	OAV101 mono	2 Copies	2	17.2	19.5	32.7	Upper respiratory illness	Yes
[redacted]	Switch to OAV101	2 Copies	23	3.4	8.8	68.4	Progression of disease without acute cause	Yes
[redacted]	Switch to OAV101	2 Copies	24	6.5	8.1	70.7	Upper respiratory illness	Yes
[redacted]	Switch to OAV101	2 Copies	7	14.7	16.6	22.8	Progression of disease without acute cause	Yes
[redacted]	Transient add-on	2 Copies	2	23.1	24.9	48.3	Pneumonia	Yes

Listing 16.3.jpn
Listing of Patient Deaths
Japan OAV101 Treated Patients

Patient	Date of Death	Primary cause of death
[redacted]	[redacted]	Respiratory failure

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 366	ALB	4.9 (g/dL)	3.9	4.9
	Enrollment / [redacted] 366	ALP	718 (U/L)	104	338
	Enrollment / [redacted] 366	ALT	19 (U/L)	3	49
	Enrollment / [redacted] 366	AST	41 (U/L)	9	37
	Enrollment / [redacted] 366	BILD	0 (mg/dL)	0	0.6
	Enrollment / [redacted] 366	BILI	0.4 (mg/dL)	0.1	1.1
	Enrollment / [redacted] 366	HCT	38.9 (%)	33.4	44.9
	Enrollment / [redacted] 366	HGB	13.3 (g/dL)	11.3	15.2
	Enrollment / [redacted] 366	PLAT	294 (10E3/uL)	131	369
	Enrollment / [redacted] 366	PROT	7.2 (g/dL)	6.6	8.2
	Enrollment / [redacted] 366	RBC	4.84 (10E6/uL)	3.76	5
	Enrollment / [redacted] 366	WBC	10.4 (10E3/uL)	3.5	9.1
[redacted]	Enrollment [redacted] / 1	ALB	4.1 (g/dL)	3.9	4.9
	Enrollment [redacted] / 1	ALP	431 (U/L)	38	113
	Enrollment [redacted] / 1	ALT	27 (U/L)	3	49
	Enrollment [redacted] / 1	AST	41 (U/L)	9	37
	Enrollment [redacted] / 1	BILD	0 (mg/dL)	0	0.6
	Enrollment [redacted] / 1	BILI	0.2 (mg/dL)	0.1	1.1
	Enrollment [redacted] / 1	HCT	37 (%)	33.4	44.9
	Enrollment [redacted] / 1	HGB	12 (g/dL)	11.3	15.2
	Enrollment [redacted] / 1	PLAT	379 (10E3/uL)	131	369
	Enrollment [redacted] / 1	PROT	6.7 (g/dL)	6.6	8.2

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 1	RBC	4.63 (10E6/uL)	3.76	5
	Enrollment [redacted] / 1	WBC	10.4 (10E3/uL)	3.5	9.1
	Enrollment / [redacted] 515	ALP	614 (U/L)	106	322
	Enrollment / [redacted] 530	PROT	7.3 (g/dL)	6.6	8.1
	Enrollment / [redacted] 538	ALB	5.1 (g/dL)	4.1	5.1
	Enrollment / [redacted] 538	ALT	28 (U/L)	7	23
	Enrollment / [redacted] 538	AST	40 (U/L)	13	30
	Enrollment / [redacted] 538	BILI	0.58 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 538	HCT	42.3 (%)	35.1	44.4
	Enrollment / [redacted] 538	HGB	13.9 (g/dL)	11.6	14.8
	Enrollment / [redacted] 538	PLAT	372 (10E3/uL)	158	348
	Enrollment / [redacted] 538	RBC	4.98 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] 538	WBC	9.9 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] / 93	ALB	4.7 (g/dL)	4.1	5.1
	Enrollment / [redacted] / 93	ALP	155 (U/L)	38	113
	Enrollment / [redacted] / 93	ALT	17 (U/L)	10	42
	Enrollment / [redacted] / 93	AST	36 (U/L)	13	30
	Enrollment / [redacted] / 93	BILD	0.03 (mg/dL)	0	0.2
	Enrollment / [redacted] / 93	BILI	0.34 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / 93	HCT	42.8 (%)	40.7	50.1
	Enrollment / [redacted] / 93	HGB	13.1 (g/dL)	13.7	16.8

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 93	PLAT	365 (10E3/uL)	158	348
	Enrollment [redacted] / 93	PROT	7.1 (g/dL)	6.6	8.1
	Enrollment [redacted] / 93	RBC	5.21 (10E6/uL)	4.35	5.55
	Enrollment [redacted] / 93	WBC	12 (10E3/uL)	3.3	8.6
	Enrollment [redacted] / 97	ALT	56 (U/L)	10	42
	Enrollment [redacted] / 97	AST	123 (U/L)	13	30
	Enrollment / [redacted] 100	PLAT	146 (10E3/uL)	158	348
	Enrollment / [redacted] 105	ALT	2871 (U/L)	10	42
	Enrollment / [redacted] 105	AST	1856 (U/L)	13	30
	Enrollment / [redacted] 176	ALB	4.4 (g/dL)	3.26	4.76
	Enrollment / [redacted] 176	ALP	687 (IU/L)	400	1550
	Enrollment / [redacted] 176	ALT	16 (IU/L)	12	50.5
	Enrollment / [redacted] 176	AST	35 (IU/L)	24.5	66.5
	Enrollment / [redacted] 176	BILI	0.1 (mg/dL)	0.12	0.59
	Enrollment / [redacted] 176	HCT	35.3 (%)	30.5	41.7
	Enrollment / [redacted] 176	HGB	11.8 (g/dL)	10.2	14.3
	Enrollment / [redacted] 176	PLAT	448 (10E3/uL)	200	740
	Enrollment / [redacted] 176	PROT	6.8 (g/dL)	5.45	7.25
	Enrollment / [redacted] 176	RBC	4.54 (10E6/uL)	3.86	5.3
	Enrollment / [redacted] 176	WBC	7.6 (10E3/uL)	4.4	19.2
	Enrollment / [redacted] 152	ALB	4.1 (g/dL)	3.26	4.76

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 152	ALP	607 (U/L)	400	1550
	Enrollment / [redacted] 152	ALT	25 (U/L)	12	50.5
	Enrollment / [redacted] 152	AST	34 (U/L)	24.5	66.5
	Enrollment / [redacted] 152	BILI	0.2 (mg/dL)	0.12	0.59
	Enrollment / [redacted] 152	HCT	40.2 (%)	30.5	41.7
	Enrollment / [redacted] 152	HGB	14 (g/dL)	10.2	14.3
	Enrollment / [redacted] 152	PLAT	587 (10E3/uL)	200	740
	Enrollment / [redacted] 152	PROT	6 (g/dL)	5.45	7.25
	Enrollment / [redacted] 152	RBC	5.13 (10E6/uL)	3.86	5.3
	Enrollment / [redacted] 152	WBC	16.5 (10E3/uL)	4.4	19.2
	Enrollment / [redacted] 14	ALB	4.2 (g/dL)	3.36	4.74
	Enrollment / [redacted] 14	ALP	204 (U/L)	-	-
	Enrollment / [redacted] 14	ALT	11 (U/L)	9.4	38.4
	Enrollment / [redacted] 14	AST	32 (U/L)	23	56.5
	Enrollment / [redacted] 14	BILI	0.4 (mg/dL)	0.16	0.67
	Enrollment / [redacted] 14	HCT	36.1 (%)	32	42.4
	Enrollment / [redacted] 14	HGB	12.1 (g/dL)	10.5	14.1
	Enrollment / [redacted] 14	PLAT	27.8 (10E4/uL)	16	65
	Enrollment / [redacted] 14	PROT	6.6 (g/dL)	5.7	7.5
	Enrollment / [redacted] 14	RBC	4.55 (10E6/uL)	3.93	5.38
	Enrollment / [redacted] 14	WBC	8.8 (10E3/uL)	4.3	19.6
	Enrollment / [redacted] 17	ALT	13 (U/L)	-	-

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 17	AST	59 (U/L)	-	-
	Enrollment [redacted] / 19	AST	585 (U/L)	-	-
	Enrollment [redacted] / 19	PLAT	14.3 (10E4/uL)	-	-
	Enrollment [redacted] / 21	PLAT	11.5 (10E4/uL)	-	-
	Enrollment [redacted] / 49	ALT	617 (U/L)	-	-
	Enrollment [redacted] / 20	ALB	4.9 (g/dL)	3.27	4.76
	Enrollment [redacted] / 20	ALP	158 (U/L)	-	-
	Enrollment [redacted] / 20	ALT	8 (U/L)	11.5	48
	Enrollment [redacted] / 20	AST	32 (U/L)	24	65.5
	Enrollment [redacted] / 20	BILI	0.5 (mg/dL)	0.12	0.59
	Enrollment [redacted] / 20	HCT	31.1 (%)	30.8	41.7
	Enrollment [redacted] / 20	HGB	10.4 (g/dL)	10.3	14.3
	Enrollment [redacted] / 20	PLAT	57 (10E4/uL)	20	72
	Enrollment [redacted] / 20	PROT	6.8 (g/dL)	5.5	7.3
	Enrollment [redacted] / 20	RBC	4.02 (10E6/uL)	3.88	5.32
	Enrollment [redacted] / 20	WBC	5.4 (10E3/uL)	4.4	19.4
	Enrollment / [redacted] 487	ALB	3.7 (g/dL)	3.5	5
	Enrollment / [redacted] 487	ALT	10 (IU/L)	6	27
	Enrollment / [redacted] 487	AST	24 (IU/L)	13	30
	Enrollment / [redacted] 487	BILI	0.1 (mg/dL)	0.2	1
	Enrollment / [redacted] 487	HCT	32.3 (%)	36	47

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 487	HGB	10.6 (g/dL)	11.3	14.9
	Enrollment / [redacted] 487	PLAT	396 (10E3/uL)	180	340
	Enrollment / [redacted] 487	PROT	6.6 (g/dL)	6.6	8.1
	Enrollment / [redacted] 487	RBC	4.14 (10E6/uL)	3.95	4.65
	Enrollment / [redacted] 487	WBC	5900 (cells/uL)	4300	8000
	Enrollment / [redacted] 355	ALB	4.1 (g/dL)	4.1	5.1
	Enrollment / [redacted] 355	ALP	378 (U/L)	106	322
	Enrollment / [redacted] 355	ALT	40 (U/L)	7	23
	Enrollment / [redacted] 355	AST	39 (U/L)	13	30
	Enrollment / [redacted] 355	BILI	0.39 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 355	HCT	37.8 (%)	35.1	44.4
	Enrollment / [redacted] 355	HGB	12.2 (g/dL)	11.6	14.8
	Enrollment / [redacted] 355	PLAT	326 (10E3/uL)	158	348
	Enrollment / [redacted] 355	PROT	6.7 (g/dL)	6.6	8.1
	Enrollment / [redacted] 355	RBC	4.48 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] 355	WBC	5.9 (10E3/uL)	3.3	8.6
	Enrollment [redacted] / 1	ALB	5.3 (g/dL)	4.1	5.1
	Enrollment [redacted] / 1	ALT	55 (U/L)	10	42
	Enrollment [redacted] / 1	AST	63 (U/L)	13	30
	Enrollment [redacted] / 1	HCT	43.1 (%)	40.7	50.1
	Enrollment [redacted] / 1	HGB	14.1 (g/dL)	13.7	16.8

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 1	PLAT	342 (10E3/uL)	158	348
	Enrollment [redacted] / 1	PROT	7.4 (g/dL)	6.6	8.1
	Enrollment [redacted] / 1	RBC	5.37 (10E6/uL)	4.35	5.55
	Enrollment [redacted] / 1	WBC	7.2 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 238	ALP	237 (U/L)	38	113
	Enrollment / [redacted] 238	ALT	23 (U/L)	7	23
	Enrollment / [redacted] 238	AST	35 (U/L)	13	30
	Enrollment / [redacted] 238	BILI	0.51 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 238	HCT	38.3 (%)	35.1	44.4
	Enrollment / [redacted] 238	HGB	12.1 (g/dL)	11.6	14.8
	Enrollment / [redacted] 238	PLAT	441 (10E3/uL)	158	348
	Enrollment / [redacted] 238	PROT	6.4 (g/dL)	6.6	8.1
	Enrollment / [redacted] 238	RBC	4.7 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] 238	WBC	11.1 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 239	AST	45 (U/L)	13	30
	Enrollment / [redacted] 242	ALT	78 (U/L)	7	23
	Enrollment / [redacted] 242	AST	152 (U/L)	13	30
	Enrollment / [redacted] 242	PLAT	58 (10E3/uL)	158	348
	Enrollment / [redacted] 244	PLAT	47 (10E3/uL)	158	348
	Enrollment / [redacted] 275	AST	514 (U/L)	13	30
	Enrollment / [redacted] 279	ALT	768 (U/L)	7	23

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day		Test	Result (units)	Clinically Significant Limits	
					Very Low	Very High
[redacted]	Enrollment / [redacted]	-19	ALB	4.4 (g/dL)	4	5
	Enrollment / [redacted]	-19	ALT	14 (IU/L)	5	25
	Enrollment / [redacted]	-19	AST	35 (IU/L)	15	50
	Enrollment / [redacted]	-19	BILI	0.3 (mg/dL)	0.1	1.1
	Enrollment / [redacted]	-19	HCT	38.9 (%)	33.4	44.9
	Enrollment / [redacted]	-19	HGB	13.1 (g/dL)	11.3	15.2
	Enrollment / [redacted]	-19	PLAT	246 (10E3/uL)	130	369
	Enrollment / [redacted]	-19	PROT	6.7 (g/dL)	5.7	7.3
	Enrollment / [redacted]	-19	RBC	5.1 (10E6/uL)	3.76	5
	Enrollment / [redacted]	-19	WBC	11.3 (10E3/uL)	3.5	9.1
	Enrollment / [redacted]	105	ALB	4.2 (g/dL)	3.5	4.8
	Enrollment / [redacted]	105	ALT	25 (IU/L)	5	65
	Enrollment / [redacted]	105	AST	32 (IU/L)	15	80
	Enrollment / [redacted]	105	BILI	0.8 (mg/dL)	0.2	1.1
	Enrollment / [redacted]	105	HCT	34.6 (%)	33.4	44.9
	Enrollment / [redacted]	105	HGB	11.4 (g/dL)	11.3	15.2
	Enrollment / [redacted]	105	PLAT	548 (10E3/uL)	130	369
	Enrollment / [redacted]	105	PROT	6 (g/dL)	5	7
	Enrollment / [redacted]	105	RBC	4.36 (10E6/uL)	3.76	5
	Enrollment / [redacted]	105	WBC	6.7 (10E3/uL)	3.5	9.1
	Enrollment / [redacted]	-17	ALB	4.3 (g/dL)	3.4	4.7

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] -17	ALT	17 (IU/L)	9	38
	Enrollment / [redacted] -17	AST	38 (IU/L)	23	57
	Enrollment / [redacted] -17	BILI	0.3 (mg/dL)	0.2	0.7
	Enrollment / [redacted] -17	HCT	37.4 (%)	39.8	51.8
	Enrollment / [redacted] -17	HGB	12.6 (g/dL)	13.5	17.6
	Enrollment / [redacted] -17	PLAT	377 (10E3/uL)	131	362
	Enrollment / [redacted] -17	PROT	6.4 (g/dL)	5.7	7.5
	Enrollment / [redacted] -17	RBC	4.64 (10E6/uL)	4.27	5.7
	Enrollment / [redacted] -17	WBC	9.4 (10E3/uL)	3.9	9.8
	Enrollment / [redacted] -23	ALB	3.7 (g/dL)	3	4.1
	Enrollment / [redacted] -23	ALT	28 (U/L)	11	45
	Enrollment / [redacted] -23	AST	39 (U/L)	20	62
	Enrollment / [redacted] -23	BILI	2.3 (mg/dL)	0.4	3.2
	Enrollment / [redacted] -23	HCT	47.2 (%)	33.4	44.9
	Enrollment / [redacted] -23	HGB	16.4 (g/dL)	11.3	15.2
	Enrollment / [redacted] -23	PLAT	52.6 (10E4/uL)	13	36.9
	Enrollment / [redacted] -23	PROT	5.5 (g/dL)	4.7	6.4
	Enrollment / [redacted] -23	RBC	50.2 (10E6/uL)	37.6	50
	Enrollment / [redacted] -23	WBC	11 (10E3/uL)	3.5	9.1
	Enrollment [redacted] / 4	AST	65 (IU/L)	20	62
	Enrollment [redacted] 70	ALT	69 (IU/L)	11	45
	Enrollment [redacted] 70	AST	78 (IU/L)	20	62

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 77	ALT	80 (IU/L)	11	45
	Enrollment / [redacted] / 77	AST	94 (IU/L)	20	62
	Enrollment [redacted] / 0	ALB	3.7 (g/dL)	3.1	4.3
	Enrollment [redacted] / 0	ALT	32 (U/L)	12	50
	Enrollment [redacted] / 0	AST	39 (U/L)	21	64
	Enrollment [redacted] / 0	BILI	0.8 (mg/dL)	0.3	2.3
	Enrollment [redacted] / 0	HCT	34.9 (%)	39.8	51.8
	Enrollment [redacted] / 0	HGB	12.1 (g/dL)	13.5	17.6
	Enrollment [redacted] / 0	PLAT	34.1 (10E4/uL)	13	36.2
	Enrollment [redacted] / 0	PROT	5.5 (g/dL)	4.9	6.6
	Enrollment [redacted] / 0	RBC	3.9 (10E6/uL)	4.27	5.7
	Enrollment [redacted] / 0	WBC	9.8 (10E3/uL)	3.9	9.8
	Enrollment [redacted] / 1	ALB	3.5 (g/dL)	3	4.1
	Enrollment [redacted] / 1	ALT	20 (U/L)	11	45
	Enrollment [redacted] / 1	AST	29 (U/L)	20	62
	Enrollment [redacted] / 1	BILI	0.6 (mg/dL)	0.4	3.2
	Enrollment [redacted] / 1	HCT	29.3 (%)	33.4	44.9
	Enrollment [redacted] / 1	HGB	9.9 (g/dL)	11.3	15.2
	Enrollment [redacted] / 1	PLAT	38.2 (10E4/uL)	13	36.9
	Enrollment [redacted] / 1	PROT	5.4 (g/dL)	4.7	6.4
	Enrollment [redacted] / 1	RBC	3.02 (10E6/uL)	3.76	5

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 1	WBC	8.8 (10E3/uL)	3.5	9.1
[redacted]	Enrollment / [redacted] 24	ALP	685 (IU/L)	104	338
	Enrollment / [redacted] 24	ALT	23 (IU/L)	0	40
	Enrollment / [redacted] 24	AST	23 (IU/L)	0	37
	Enrollment / [redacted] 24	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 24	HCT	34.1 (%)	39.8	51.8
	Enrollment / [redacted] 24	HGB	11.4 (g/dL)	13.5	17.6
	Enrollment / [redacted] 24	PLAT	422 (10E3/uL)	131	362
	Enrollment / [redacted] 24	PROT	5.3 (g/dL)	5.5	8
	Enrollment / [redacted] 24	RBC	4.21 (10E6/uL)	4.27	5.7
	Enrollment / [redacted] 24	WBC	7.8 (10E3/uL)	3.9	9.8
[redacted]	Enrollment / [redacted] -2	ALB	4.1 (g/dL)	4.2	4.9
	Enrollment / [redacted] -2	ALP	602 (IU/L)	104	338
	Enrollment / [redacted] -2	ALT	17 (IU/L)	0	40
	Enrollment / [redacted] -2	AST	27 (IU/L)	0	37
	Enrollment / [redacted] -2	BILI	0.2 (mg/dL)	0.2	1.2
	Enrollment / [redacted] -2	HCT	36.1 (%)	39.8	51.8
	Enrollment / [redacted] -2	HGB	11.7 (g/dL)	13.5	17.6
	Enrollment / [redacted] -2	PLAT	564 (10E3/uL)	131	362
	Enrollment / [redacted] -2	PROT	6 (g/dL)	5.5	8
	Enrollment / [redacted] -2	RBC	4.48 (10E6/uL)	4.27	5.7

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / -2	WBC	13.6 (10E3/uL)	3.9	9.8
	Enrollment / [redacted] / 266	ALB	4.6 (g/dL)	4.1	5.1
	Enrollment / [redacted] / 266	ALP	602 (U/L)	106	322
	Enrollment / [redacted] / 266	ALT	19 (U/L)	7	23
	Enrollment / [redacted] / 266	AST	44 (U/L)	13	30
	Enrollment / [redacted] / 266	BILI	0.6 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / 266	HCT	34.9 (%)	35.1	44.4
	Enrollment / [redacted] / 266	HGB	11.6 (g/dL)	11.6	14.8
	Enrollment / [redacted] / 266	PLAT	257 (10E3/uL)	158	348
	Enrollment / [redacted] / 266	RBC	4.14 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] / 266	WBC	6.49 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] / 673	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / [redacted] / 673	ALP	640 (U/L)	106	322
	Enrollment / [redacted] / 673	ALT	35 (U/L)	10	42
	Enrollment / [redacted] / 673	AST	55 (U/L)	13	30
	Enrollment / [redacted] / 673	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / 673	HCT	37.4 (%)	40.7	50.1
	Enrollment / [redacted] / 673	HGB	12.7 (g/dL)	13.7	16.8
	Enrollment / [redacted] / 673	PLAT	456 (10E3/uL)	158	348
	Enrollment / [redacted] / 673	PROT	6.9 (g/dL)	6.6	8.1
	Enrollment / [redacted] / 673	RBC	4.46 (10E6/uL)	4.35	5.55

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 673	WBC	11.32 (10E3/uL)	3.3	8.6
[redacted]	Enrollment / [redacted] -22	ALB	4.5 (g/dL)	4.1	5.1
	Enrollment / [redacted] -22	ALP	214 (U/L)	395	1339
	Enrollment / [redacted] -22	ALT	16 (U/L)	10	38
	Enrollment / [redacted] -22	AST	33 (U/L)	13	57
	Enrollment / [redacted] -22	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / [redacted] -22	HCT	39 (%)	32	42.4
	Enrollment / [redacted] -22	HGB	13.2 (g/dL)	10.5	14.1
	Enrollment / [redacted] -22	PLAT	336 (10E3/uL)	158	348
	Enrollment / [redacted] -22	PROT	6 (g/dL)	6.6	8.1
	Enrollment / [redacted] -22	RBC	4.93 (10E6/uL)	4.35	5.55
	Enrollment / [redacted] -22	WBC	6.87 (10E3/uL)	3.3	8.6
	Enrollment [redacted] / 4	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment [redacted] / 4	ALT	13 (U/L)	10	42
	Enrollment [redacted] / 4	AST	20 (U/L)	13	30
	Enrollment [redacted] / 4	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment [redacted] / 4	HCT	34.4 (%)	40.7	50.1
	Enrollment [redacted] / 4	HGB	11.5 (g/dL)	13.7	16.8
	Enrollment [redacted] / 4	PLAT	218 (10E3/uL)	158	348
	Enrollment [redacted] / 4	PROT	6.3 (g/dL)	6.6	8.1
	Enrollment [redacted] / 4	RBC	4.21 (10E6/uL)	4.35	5.55

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 4	WBC	11.16 (10E3/uL)	3.3	8.6
[redacted]	Enrollment / [redacted] 225	ALB	4.2 (g/dL)	3.4	4.7
	Enrollment / [redacted] 225	ALT	14 (IU/L)	9	38
	Enrollment / [redacted] 225	AST	28 (IU/L)	24	57
	Enrollment / [redacted] 225	BILI	0.39 (mg/dL)	0.2	0.7
	Enrollment / [redacted] 225	HCT	35.1 (%)	33	42.4
	Enrollment / [redacted] 225	HGB	10.8 (g/dL)	10.7	14.1
	Enrollment / [redacted] 225	PLAT	280 (10E3/uL)	320	650
	Enrollment / [redacted] 225	PROT	6.3 (g/dL)	5.7	7.5
	Enrollment / [redacted] 225	RBC	4.04 (10E6/uL)	3.93	5.38
	Enrollment / [redacted] 225	WBC	6.6 (10E3/uL)	4.3	19.6
[redacted]	Enrollment / [redacted] 12	ALB	3.6 (g/dL)	4.1	5.1
	Enrollment / [redacted] 12	ALT	36 (IU/L)	7	23
	Enrollment / [redacted] 12	AST	39 (IU/L)	13	30
	Enrollment / [redacted] 12	BILD	0.07 (mg/dL)	0	0.2
	Enrollment / [redacted] 12	BILI	0.22 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 12	HCT	31.6 (%)	35.1	44.4
	Enrollment / [redacted] 12	HGB	10.4 (g/dL)	11.6	14.8
	Enrollment / [redacted] 12	PLAT	518 (10E3/uL)	158	348
	Enrollment / [redacted] 12	PROT	5.4 (g/dL)	6.6	8.1
	Enrollment / [redacted] 12	RBC	3.51 (10E6/uL)	3.86	4.92

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 12	WBC	10.1 (10E3/uL)	3.3	8.6
	Enrollment [redacted] / 9	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment [redacted] / 9	ALT	10 (IU/L)	10	42
	Enrollment [redacted] / 9	AST	22 (IU/L)	13	30
	Enrollment [redacted] / 9	BILD	0.31 (mg/dL)	0	0.2
	Enrollment [redacted] / 9	BILI	13.21 (mg/dL)	0.4	1.5
	Enrollment [redacted] / 9	HCT	42.4 (%)	40.7	50.1
	Enrollment [redacted] / 9	HGB	15 (g/dL)	13.7	16.8
	Enrollment [redacted] / 9	PLAT	725 (10E3/uL)	158	348
	Enrollment [redacted] / 9	PROT	6.4 (g/dL)	6.6	8.1
	Enrollment [redacted] / 9	RBC	4.52 (10E6/uL)	4.35	5.55
	Enrollment [redacted] / 9	WBC	7.1 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 499	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / [redacted] 499	ALP	336 (IU/L)	173	353
	Enrollment / [redacted] 499	ALT	21 (IU/L)	5	31
	Enrollment / [redacted] 499	AST	45 (IU/L)	22	50
	Enrollment / [redacted] 499	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 499	HCT	39.5 (%)	34	40
	Enrollment / [redacted] 499	HGB	13.3 (g/dL)	10.5	13.5
	Enrollment / [redacted] 499	PLAT	391 (10E3/uL)	150	400
	Enrollment / [redacted] 499	PROT	6.5 (mg/dL)	6.5	8.1

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 499	RBC	437 (10E10/L)	15	35
	Enrollment / [redacted] 499	WBC	11600 (cmm)	7000	15000
	Enrollment [redacted] / -1	ALT	85 (U/L)	10	30
	Enrollment [redacted] / -1	AST	73 (U/L)	13	30
	Enrollment [redacted] / -1	BILD	0.1 (mg/dL)	0	0.2
	Enrollment [redacted] / -1	BILI	0.5 (mg/dL)	0.4	1.2
	Enrollment [redacted] / -1	HCT	35.9 (%)	40.7	50.7
	Enrollment [redacted] / -1	HGB	12.2 (g/dL)	13.7	16.8
	Enrollment [redacted] / -1	PLAT	677 (10E3/uL)	158	348
	Enrollment [redacted] / -1	RBC	4.58 (10E6/uL)	4.35	5.55
	Enrollment [redacted] / -1	WBC	7.3 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 211	ALB	4.3 (g/dL)	4.1	5.1
	Enrollment / [redacted] 211	ALP	163 (U/L)	38	113
	Enrollment / [redacted] 211	ALT	12 (U/L)	10	30
	Enrollment / [redacted] 211	AST	25 (U/L)	13	30
	Enrollment / [redacted] 211	BILD	0 (mg/dL)	0	0.2
	Enrollment / [redacted] 211	BILI	0.3 (mg/dL)	0.4	1.2
	Enrollment / [redacted] 211	HCT	37.7 (%)	40.7	50.1
	Enrollment / [redacted] 211	HGB	12.3 (g/dL)	13.7	16.8
	Enrollment / [redacted] 211	PLAT	466 (10E3/uL)	158	348
	Enrollment / [redacted] 211	PROT	6.7 (g/dL)	6.6	8.1

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 211	RBC	4.51 (10E6/uL)	4.35	5.55
	Enrollment / [redacted] 211	WBC	10.5 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 224	ALB	4.6 (g/dL)	4.1	5.1
	Enrollment / [redacted] 224	ALP	167 (U/L)	38	113
	Enrollment / [redacted] 224	ALT	132 (U/L)	10	30
	Enrollment / [redacted] 224	AST	232 (U/L)	13	30
	Enrollment / [redacted] 224	BILD	0.2 (mg/dL)	0	0.2
	Enrollment / [redacted] 224	BILI	0.5 (mg/dL)	0.4	1.2
	Enrollment / [redacted] 224	HCT	35.4 (%)	40.7	50.1
	Enrollment / [redacted] 224	HGB	11.1 (g/dL)	13.7	16.8
	Enrollment / [redacted] 224	PLAT	433 (10E3/uL)	158	348
	Enrollment / [redacted] 224	PROT	6.9 (g/dL)	6.6	8.1
	Enrollment / [redacted] 224	RBC	4.18 (10E6/uL)	4.35	5.55
	Enrollment / [redacted] 224	WBC	13.9 (10E3/uL)	3.3	8.6
	Enrollment [redacted] / 0	ALB	3.6 (g/dL)	4.1	5.1
	Enrollment [redacted] 0	ALP	286 (U/L)	38	113
	Enrollment [redacted] 0	ALT	24 (U/L)	10	42
	Enrollment [redacted] 0	AST	33 (U/L)	13	30
	Enrollment [redacted] 0	HCT	34 (%)	40.7	50.1
	Enrollment [redacted] 0	HGB	11.8 (g/dL)	13.7	16.8
	Enrollment [redacted] 0	PLAT	699 (10E3/uL)	158	348

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 0	PROT	5.3 (g/dL)	6.6	8.1
	Enrollment [redacted] / 0	RBC	3.77 (10E6/uL)	4.35	5.55
	Enrollment [redacted] / 0	WBC	10.7 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 133	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / [redacted] 133	ALP	292 (U/L)	38	113
	Enrollment / [redacted] 133	ALT	19 (U/L)	7	23
	Enrollment / [redacted] 133	AST	23 (U/L)	13	30
	Enrollment / [redacted] 133	BILD	0.1 (mg/dL)	0	0.2
	Enrollment / [redacted] 133	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 133	HGB	11.3 (g/dL)	11.6	14.8
	Enrollment / [redacted] 133	PLAT	414 (10E3/uL)	158	348
	Enrollment / [redacted] 133	PROT	6.6 (g/dL)	6.6	8.1
	Enrollment / [redacted] 133	RBC	4.34 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] 133	WBC	9.6 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 176	AST	37 (U/L)	12	30
	Enrollment / [redacted] 176	WBC	21.1 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 113	ALB	4.8 (g/dL)	4.1	5.1
	Enrollment / [redacted] 113	ALP	305 (U/L)	38	113
	Enrollment / [redacted] 113	ALT	66 (U/L)	7	23
	Enrollment / [redacted] 113	AST	73 (U/L)	13	30
	Enrollment / [redacted] 113	BILD	0.1 (mg/dL)	0	0.2

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 113	BILI	0.5 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 113	HCT	42.9 (%)	35.1	44
	Enrollment / [redacted] 113	HGB	14.3 (g/dL)	11.6	14.8
	Enrollment / [redacted] 113	PLAT	269 (10E3/uL)	158	348
	Enrollment / [redacted] 113	PROT	6.5 (g/dL)	6.6	8.1
	Enrollment / [redacted] 113	RBC	5.11 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] 113	WBC	7.4 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 119	ALT	149 (U/L)	7	23
	Enrollment / [redacted] 119	AST	243 (U/L)	13	30
	Enrollment / [redacted] 119	WBC	3.1 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 141	AST	97 (U/L)	13	30
	Enrollment / [redacted] 144	AST	123 (U/L)	13	30
[redacted]	Enrollment / [redacted] 225	ALB	5.2 (g/dL)	4.1	5.1
	Enrollment / [redacted] 225	ALP	300 (U/L)	38	113
	Enrollment / [redacted] 225	ALT	27 (U/L)	7	23
	Enrollment / [redacted] 225	AST	37 (U/L)	13	30
	Enrollment / [redacted] 225	HCT	38.4 (%)	35.1	44.4
	Enrollment / [redacted] 225	HGB	12.5 (g/dL)	11.6	14.8
	Enrollment / [redacted] 225	PLAT	613 (10E3/uL)	158	348
	Enrollment / [redacted] 225	PROT	7 (g/dL)	6.6	8.1
	Enrollment / [redacted] 225	RBC	4.6 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] 225	WBC	8.1 (10E3/uL)	3.3	8.6

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 229	ALT	39 (U/L)	7	23
	Enrollment / [redacted] 229	AST	90 (U/L)	13	30
	Enrollment / [redacted] 231	ALT	81 (U/L)	7	23
	Enrollment / [redacted] 231	AST	134 (U/L)	13	30
	Enrollment / [redacted] 268	ALT	32 (U/L)	7	23
	Enrollment / [redacted] 268	AST	51 (U/L)	13	30
	Enrollment / [redacted] 64	ALB	4.1 (g/dL)	3.9	4.9
	Enrollment / [redacted] 64	ALP	466 (IU/L)	130	350
	Enrollment / [redacted] 64	ALT	11 (IU/L)	4	43
	Enrollment / [redacted] 64	AST	32 (IU/L)	8	38
	Enrollment / [redacted] 64	BILD	0.1 (mg/dL)	0	0.2
	Enrollment / [redacted] 64	BILI	0.9 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 64	HCT	38.3 (%)	35	48
	Enrollment / [redacted] 64	HGB	12.6 (g/dL)	12	16
	Enrollment / [redacted] 64	PLAT	406 (10E3/uL)	130	406
	Enrollment / [redacted] 64	PROT	6.6 (g/dL)	6.7	8.8
	Enrollment / [redacted] 64	RBC	4.8 (10E6/uL)	3.8	4.8
	Enrollment / [redacted] 64	WBC	7.4 (10E3/uL)	5	8.5
	Enrollment [redacted] / 7	ALB	3.7 (g/dL)	3.9	4.9
	Enrollment [redacted] / 7	ALP	258 (IU/L)	38	113
	Enrollment [redacted] / 7	ALT	11 (IU/L)	4	43

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 7	AST	21 (IU/L)	8	38
	Enrollment [redacted] / 7	BILD	0.2 (mg/dL)	0	0.2
	Enrollment [redacted] / 7	BILI	2.6 (mg/dL)	0.2	1.2
	Enrollment [redacted] / 7	HCT	30.8 (%)	35	48
	Enrollment [redacted] / 7	HGB	10.7 (g/dL)	12	16
	Enrollment [redacted] / 7	PLAT	271 (10E3/uL)	130	400
	Enrollment [redacted] / 7	PROT	5.2 (g/dL)	6.7	8.3
	Enrollment [redacted] / 7	RBC	3.25 (10E6/uL)	3.8	4.8
	Enrollment [redacted] / 7	WBC	6.2 (10E3/uL)	5	8.5
	Enrollment / [redacted] 50	ALB	4.6 (g/dL)	3.9	4.9
	Enrollment / [redacted] 50	ALP	132 (U/L)	38	113
	Enrollment / [redacted] 50	ALT	20 (U/L)	4	43
	Enrollment / [redacted] 50	AST	40 (U/L)	8	38
	Enrollment / [redacted] 50	BILD	0.1 (mg/dL)	0	0.2
	Enrollment / [redacted] 50	BILI	0.6 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 50	HCT	36.1 (%)	35	48
	Enrollment / [redacted] 50	HGB	11.9 (g/dL)	12	16
	Enrollment / [redacted] 50	PLAT	287 (10E3/uL)	130	400
	Enrollment / [redacted] 50	PROT	6.2 (g/dL)	6.7	8.3
	Enrollment / [redacted] 50	RBC	4.34 (10E6/uL)	3.8	4.8
	Enrollment / [redacted] 50	WBC	9.8 (10E3/uL)	5	8.3
	Enrollment / [redacted] 56	ALT	119 (U/L)	4	43

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 56	ALT	266 (U/L)	4	43
	Enrollment / [redacted] 56	AST	298 (U/L)	8	38
	Enrollment / [redacted] 56	AST	700 (U/L)	8	38
	Enrollment / [redacted] 59	ALT	259 (U/L)	4	43
	Enrollment / [redacted] 59	AST	397 (U/L)	8	38
	Enrollment / [redacted] 64	ALT	70 (U/L)	4	43
	Enrollment / [redacted] 64	AST	61 (U/L)	8	38
	Enrollment / [redacted] 77	AST	50 (U/L)	8	38
	Enrollment / [redacted] 14	ALB	3.4 (g/dL)	3.9	4.9
	Enrollment / [redacted] 14	ALP	222 (U/L)	38	113
	Enrollment / [redacted] 14	ALT	18 (U/L)	4	44
	Enrollment / [redacted] 14	AST	20 (U/L)	8	38
	Enrollment / [redacted] 14	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 14	HCT	30.8 (%)	41.3	52.1
	Enrollment / [redacted] 14	HGB	10.3 (g/dL)	13.3	16.6
	Enrollment / [redacted] 14	PLAT	447 (10E3/uL)	172	359
	Enrollment / [redacted] 14	RBC	3.55 (10E6/uL)	4.29	5.7
	Enrollment / [redacted] 14	WBC	14.98 (10E3/uL)	3.58	8.15
	Enrollment / [redacted] 21	PLAT	96 (10E3/uL)	172	359
	Enrollment [redacted] / 1	ALB	4 (g/dL)	4.1	5.1
	Enrollment [redacted] / 1	ALP	389 (U/L)	38	113

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 1	ALT	34 (U/L)	10	42
	Enrollment / [redacted] / 1	AST	41 (U/L)	13	30
	Enrollment / [redacted] / 1	BILD	0.2 (mg/dL)	0	0.2
	Enrollment / [redacted] / 1	BILI	1.7 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / 1	HCT	31.6 (%)	40.7	50.1
	Enrollment / [redacted] / 1	HGB	10.8 (g/dL)	13.7	16.8
	Enrollment / [redacted] / 1	PLAT	388 (10E3/uL)	158	348
	Enrollment / [redacted] / 1	PROT	5.6 (g/dL)	6.6	8.1
	Enrollment / [redacted] / 1	RBC	3.47 (10E6/uL)	4.35	5.55
	Enrollment / [redacted] / 1	WBC	5.9 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] / 1	ALB	4.7 (g/dL)	3.3	5.2
	Enrollment / [redacted] / 1	ALP	475 (IU/L)	171	1330
	Enrollment / [redacted] / 1	ALT	12 (IU/L)	3	27
	Enrollment / [redacted] / 1	AST	28 (IU/L)	11	47
	Enrollment / [redacted] / 1	BILI	0.52 (mg/dL)	0.14	0.95
	Enrollment / [redacted] / 1	HCT	34.3 (%)	32	50
	Enrollment / [redacted] / 1	HGB	11.8 (g/dL)	11.5	15.5
	Enrollment / [redacted] / 1	PLAT	327 (10E3/uL)	130	400
	Enrollment / [redacted] / 1	PROT	6.8 (g/dL)	5.5	8
	Enrollment / [redacted] / 1	RBC	4.21 (10E6/uL)	3.8	5.3
	Enrollment / [redacted] / 1	WBC	7.4 (10E3/uL)	5	10

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 182	ALB	4.8 (g/dL)	4.1	5.1
	Enrollment / [redacted] 182	ALP	246 (U/L)	38	113
	Enrollment / [redacted] 182	ALT	31 (U/L)	10	30
	Enrollment / [redacted] 182	AST	57 (U/L)	13	30
	Enrollment / [redacted] 182	BILI	0.4 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 182	HCT	34.5 (%)	40.7	50.1
	Enrollment / [redacted] 182	HGB	10.7 (g/dL)	13.7	16.8
	Enrollment / [redacted] 182	PLAT	294 (10E3/uL)	158	348
	Enrollment / [redacted] 182	PROT	6.6 (g/dL)	6.6	8.1
	Enrollment / [redacted] 182	RBC	4 (10E6/uL)	4.35	5.55
	Enrollment / [redacted] 182	WBC	10860 (cells/uL)	3300	8600
[redacted]	Enrollment [redacted] / -1	ALB	4.6 (g/dL)	4.1	5.1
	Enrollment [redacted] / -1	ALP	175 (U/L)	38	113
	Enrollment [redacted] / -1	ALT	18 (U/L)	7	23
	Enrollment [redacted] / -1	AST	32 (U/L)	13	30
	Enrollment [redacted] / -1	BILD	0.1 (mg/dL)	0	0.2
	Enrollment [redacted] / -1	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment [redacted] / -1	HCT	38.9 (%)	35.1	44.4
	Enrollment [redacted] / -1	HGB	12.6 (g/dL)	11.6	14.8
	Enrollment [redacted] / -1	PLAT	777 (10E3/uL)	158	348
	Enrollment [redacted] / -1	PROT	6.7 (g/dL)	6.6	8.1
	Enrollment [redacted] / -1	RBC	4.76 (10E6/uL)	3.86	4.92

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] -1	WBC	12.74 (10E3/uL)	3.3	8.6
	Enrollment [redacted] / 5	ALT	491 (U/L)	7	23
	Enrollment [redacted] / 5	AST	781 (U/L)	13	30
	Enrollment [redacted] / 6	ALT	1067 (U/L)	7	23
	Enrollment [redacted] / 6	AST	1678 (U/L)	13	30
	Enrollment / [redacted] 27	ALT	115 (U/L)	7	23
	Enrollment / [redacted] 27	AST	124 (U/L)	13	30
	Enrollment / [redacted] 34	ALT	480 (U/L)	7	23
	Enrollment / [redacted] 34	AST	257 (U/L)	13	30
	Enrollment / [redacted] -24	ALB	3.8 (g/dL)	3.9	5.2
	Enrollment / [redacted] -24	ALP	653 (IU/L)	494	1550
	Enrollment / [redacted] -24	ALT	19 (IU/L)	9	53
	Enrollment / [redacted] -24	AST	33 (IU/L)	23	70
	Enrollment / [redacted] -24	BILD	0.1 (mg/dL)	0	0.2
	Enrollment / [redacted] -24	BILI	0.4 (mg/dL)	0.1	0.6
	Enrollment / [redacted] -24	HCT	34 (%)	32.8	42.7
	Enrollment / [redacted] -24	HGB	11.7 (g/dL)	11.1	14.5
	Enrollment / [redacted] -24	PLAT	428 (10E3/uL)	189	487
	Enrollment / [redacted] -24	PROT	5.9 (g/dL)	5.3	7.7
	Enrollment / [redacted] -24	RBC	4.3 (10E6/uL)	4.08	5.29
	Enrollment / [redacted] -24	WBC	7.8 (10E3/uL)	5.6	14.5

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / -1	ALB	4.1 (g/dL)	3.9	5.2
	Enrollment / [redacted] / -1	ALT	26 (IU/L)	7	53
	Enrollment / [redacted] / -1	AST	51 (IU/L)	17	70
	Enrollment / [redacted] / -1	BILD	0 (mg/dL)	0	0.2
	Enrollment / [redacted] / -1	BILI	0.7 (mg/dL)	0.1	0.9
	Enrollment / [redacted] / -1	HCT	42.3 (%)	32.8	42.7
	Enrollment / [redacted] / -1	HGB	13.2 (g/dL)	11.1	14.5
	Enrollment / [redacted] / -1	PLAT	260 (10E3/uL)	189	487
	Enrollment / [redacted] / -1	PROT	6.3 (g/dL)	5.3	8
	Enrollment / [redacted] / -1	RBC	5.36 (10E6/uL)	4.08	5.29
	Enrollment / [redacted] / -1	WBC	10.4 (10E3/uL)	5.6	14.5
	Enrollment / [redacted] / -9	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / [redacted] / -9	ALT	15 (U/L)	7	23
	Enrollment / [redacted] / -9	AST	47 (U/L)	13	30
	Enrollment / [redacted] / -9	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / -9	HCT	41.8 (%)	35.1	44.4
	Enrollment / [redacted] / -9	HGB	13.9 (g/dL)	11.6	14.8
	Enrollment / [redacted] / -9	PLAT	470 (10E9/L)	150	348
	Enrollment / [redacted] / -9	PROT	6.7 (g/dL)	6.6	8.1
	Enrollment / [redacted] / -9	RBC	520 (10E10/L)	386	492
	Enrollment / [redacted] / -9	WBC	12070 (10E6/L)	3300	8600

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 8	PLAT	511 (10E9/L)	158	348
	Enrollment [redacted] / 9	ALT	43 (U/L)	7	23
	Enrollment [redacted] / 9	AST	50 (U/L)	13	30
	Enrollment / [redacted] 25	ALT	351 (U/L)	7	23
	Enrollment / [redacted] 25	AST	282 (U/L)	13	30
	Enrollment / [redacted] 29	ALT	110 (U/L)	7	23
	Enrollment / [redacted] 29	AST	153 (U/L)	13	30
	Enrollment / [redacted] 32	ALT	63 (U/L)	7	23
	Enrollment / [redacted] 58	ALT	51 (U/L)	7	23
	Enrollment / [redacted] 58	AST	67 (U/L)	13	30
	Enrollment / [redacted] 78	ALT	60 (U/L)	7	23
	Enrollment / [redacted] 78	AST	76 (U/L)	13	30
	Enrollment / [redacted] 99	ALT	60 (U/L)	7	23
	Enrollment / [redacted] 99	AST	75 (U/L)	13	30
	Enrollment / [redacted] 73	ALB	4.8 (g/dL)	4.1	5.1
	Enrollment / [redacted] 73	ALP	262 (U/L)	36	113
	Enrollment / [redacted] 73	ALT	24 (U/L)	10	42
	Enrollment / [redacted] 73	AST	30 (U/L)	13	30
	Enrollment / [redacted] 73	BILD	0.1 (mg/dL)	0	0.4
	Enrollment / [redacted] 73	BILI	0.4 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 73	HCT	36.2 (%)	40.7	50.1
	Enrollment / [redacted] 73	HGB	12.3 (g/dL)	13.7	16.8

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 73	PLAT	39.9 (10E4/uL)	15	34.8
	Enrollment / [redacted] / 73	PROT	6.9 (g/dL)	6.6	8.1
	Enrollment / [redacted] / 73	RBC	4.5 (10E6/uL)	4.35	5.55
	Enrollment / [redacted] / 73	WBC	12.5 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] / 76	AST	51 (U/L)	13	30
	Enrollment / [redacted] / 77	ALT	51 (U/L)	10	42
	Enrollment / [redacted] / 77	AST	113 (U/L)	13	30
	Enrollment / [redacted] / 79	ALT	82 (U/L)	10	42
	Enrollment / [redacted] / 79	AST	83 (U/L)	13	30
	Enrollment / [redacted] / 100	AST	53 (U/L)	13	30
	Enrollment / [redacted] / 583	ALB	4 (g/dL)	4.1	5.1
	Enrollment / [redacted] / 583	ALP	222 (U/L)	38	113
	Enrollment / [redacted] / 583	ALT	27 (U/L)	10	42
	Enrollment / [redacted] / 583	AST	34 (U/L)	13	30
	Enrollment / [redacted] / 583	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / 583	HCT	38.6 (%)	40.7	50.1
	Enrollment / [redacted] / 583	HGB	12.8 (g/dL)	13.7	16.8
	Enrollment / [redacted] / 583	PLAT	334 (10E3/uL)	158	348
	Enrollment / [redacted] / 583	PROT	8.6 (g/dL)	6.6	8.1
	Enrollment / [redacted] / 583	RBC	4.65 (10E6/uL)	4.35	5.55
	Enrollment / [redacted] / 583	WBC	8.4 (10E3/uL)	3.3	8.6

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 7	ALB	3.7 (g/dL)	4.1	5.1
	Enrollment / [redacted] / 7	ALP	264 (U/L)	38	113
	Enrollment / [redacted] / 7	ALT	58 (IU/L)	7	23
	Enrollment / [redacted] / 7	AST	66 (IU/L)	13	30
	Enrollment / [redacted] / 7	BILI	0.87 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / 7	HCT	30.4 (%)	35.1	44.4
	Enrollment / [redacted] / 7	HGB	10.5 (g/dL)	11.6	14.8
	Enrollment / [redacted] / 7	PLAT	834 (10E3/uL)	158	348
	Enrollment / [redacted] / 7	PROT	5.5 (g/dL)	6.6	8.1
	Enrollment / [redacted] / 7	RBC	3.24 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] / 7	WBC	9.1 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] / 3	ALB	3.2 (g/dL)	3.8	5.2
	Enrollment / [redacted] / 3	ALP	187 (U/L)	-	-
	Enrollment / [redacted] / 3	ALT	17 (U/L)	13	56
	Enrollment / [redacted] / 3	AST	37 (U/L)	24	68
	Enrollment / [redacted] / 3	BILD	0.54 (mg/dL)	0	0.4
	Enrollment / [redacted] / 3	BILI	8.4 (mg/dL)	0.11	0.66
	Enrollment / [redacted] / 3	HCT	41 (%)	29.6	41.5
	Enrollment / [redacted] / 3	HGB	13.9 (g/dL)	9.8	14.1
	Enrollment / [redacted] / 3	PLAT	269 (10E3/uL)	230	780
	Enrollment / [redacted] / 3	PROT	4.9 (g/dL)	5.3	7.1
	Enrollment / [redacted] / 3	RBC	1.39 (10E6/uL)	3.71	5.19

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 3	WBC	6.6 (10E3/uL)	4.5	19
	Enrollment [redacted] / 0	ALB	4.5 (g/dL)	3.8	5.2
	Enrollment [redacted] / 0	ALP	145 (U/L)	138	451
	Enrollment [redacted] / 0	ALT	27 (U/L)	9	38
	Enrollment [redacted] / 0	AST	60 (U/L)	24	57
	Enrollment [redacted] / 0	BILD	0.1 (mg/dL)	0	0.4
	Enrollment [redacted] / 0	BILI	0.24 (mg/dL)	0.16	0.67
	Enrollment [redacted] / 0	HCT	32.8 (%)	31.7	42.4
	Enrollment [redacted] / 0	HGB	11.1 (g/dL)	10.7	14.1
	Enrollment [redacted] / 0	PLAT	428 (10E3/uL)	168	650
	Enrollment [redacted] / 0	PROT	6.5 (g/dL)	5.7	7.5
	Enrollment [redacted] / 0	RBC	4.2 (10E6/uL)	3.93	5.38
	Enrollment [redacted] / 0	WBC	9.2 (10E3/uL)	4.3	19.1
	Enrollment / [redacted] 23	ALT	49 (U/L)	9	38
	Enrollment / [redacted] 23	AST	96 (U/L)	24	57
	Enrollment / [redacted] 26	ALT	433 (U/L)	9	38
	Enrollment / [redacted] 26	AST	668 (U/L)	24	57
	Enrollment / [redacted] 27	ALT	463 (U/L)	9	38
	Enrollment / [redacted] 27	AST	617 (U/L)	24	57
	Enrollment / [redacted] 34	ALT	342 (U/L)	9	38
	Enrollment / [redacted] 34	AST	253 (U/L)	24	57
	Enrollment / [redacted] 41	ALT	385 (U/L)	9	38

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 41	AST	348 (U/L)	24	57
	Enrollment / [redacted] 43	ALT	489 (U/L)	9	38
	Enrollment / [redacted] 43	AST	415 (U/L)	24	57
	Enrollment / [redacted] 58	ALT	174 (U/L)	9	38
	Enrollment / [redacted] 58	AST	132 (U/L)	24	57
	Enrollment / [redacted] 84	ALT	48 (U/L)	9	38
	Enrollment / [redacted] 84	AST	76 (U/L)	24	57
	Enrollment / [redacted] 112	AST	67 (U/L)	24	57
	Enrollment / [redacted] 14	ALB	4.7 (g/dL)	4.1	5.1
	Enrollment / [redacted] 14	ALP	213 (U/L)	38	113
	Enrollment / [redacted] 14	ALT	17 (U/L)	7	23
	Enrollment / [redacted] 14	AST	33 (U/L)	13	30
	Enrollment / [redacted] 14	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 14	HCT	40.6 (%)	35.1	44.4
	Enrollment / [redacted] 14	HGB	12.7 (g/dL)	11.6	14.8
	Enrollment / [redacted] 14	PLAT	444 (10E3/uL)	158	348
	Enrollment / [redacted] 14	PROT	6.9 (g/dL)	6.6	8.1
	Enrollment / [redacted] 14	RBC	4.64 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] 14	WBC	5.8 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 18	AST	64 (U/L)	13	30
	Enrollment / [redacted] 21	ALT	25 (U/L)	7	23
	Enrollment / [redacted] 21	AST	101 (U/L)	13	30

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] -21	PLAT	25 (10E3/uL)	158	348
	Enrollment / [redacted] -23	PLAT	39 (10E3/uL)	158	348
	Enrollment / [redacted] -81	ALT	53 (U/L)	7	23
	Enrollment / [redacted] -14	ALB	4.3 (g/dL)	3.5	4.4
	Enrollment / [redacted] -14	ALP	732 (U/L)	433	1140
	Enrollment / [redacted] -14	ALT	38 (U/L)	11	55
	Enrollment / [redacted] -14	AST	37 (U/L)	21	70
	Enrollment / [redacted] -14	BILD	0 (mg/dL)	0	0.4
	Enrollment / [redacted] -14	BILI	0.5 (U/L)	0.2	1.2
	Enrollment / [redacted] -14	HCT	36.3 (%)	37.4	48.6
	Enrollment / [redacted] -14	HGB	12.4 (g/dL)	12.2	16.2
	Enrollment / [redacted] -14	PLAT	594 (10E3/uL)	138	309
	Enrollment / [redacted] -14	PROT	5.9 (g/dL)	5	6.5
	Enrollment / [redacted] -14	RBC	4.11 (10E6/uL)	3.5	4.7
	Enrollment / [redacted] -14	WBC	8.4 (10E3/uL)	5	19.5
	Enrollment [redacted] 1	ALB	4.1 (g/dL)	3.5	4.4
	Enrollment [redacted] 1	ALP	769 (U/L)	433	1140
	Enrollment [redacted] 1	ALT	38 (U/L)	11	55
	Enrollment [redacted] 1	AST	37 (U/L)	21	70
	Enrollment [redacted] 1	BILD	0 (mg/dL)	0	0.4
	Enrollment [redacted] 1	BILI	0.4 (mg/dL)	0.2	1.2
	Enrollment [redacted] 1	HCT	35.7 (%)	37.4	48.6

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] 1	HGB	11.9 (g/dL)	12.2	16.2
	Enrollment [redacted] 1	PLAT	613 (10E3/uL)	138	309
	Enrollment [redacted] 1	PROT	5.7 (g/dL)	5	6.5
	Enrollment [redacted] 1	RBC	4.14 (10E6/uL)	3.5	4.7
	Enrollment [redacted] 1	WBC	9.2 (10E3/uL)	5	19.5
	Enrollment / [redacted] 15	ALB	4.4 (g/dL)	3.6	4.6
	Enrollment / [redacted] 15	ALP	713 (U/L)	406	1100
	Enrollment / [redacted] 15	ALT	38 (U/L)	12	62
	Enrollment / [redacted] 15	AST	45 (U/L)	23	39
	Enrollment / [redacted] 15	BILD	0 (mg/dL)	0	0.4
	Enrollment / [redacted] 15	BILI	0.4 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 15	HCT	37.7 (%)	37.4	48.6
	Enrollment / [redacted] 15	HGB	12.3 (g/dL)	10.3	12.3
	Enrollment / [redacted] 15	PLAT	511 (10E3/uL)	138	309
	Enrollment / [redacted] 15	PROT	5.8 (g/dL)	5.2	6.7
	Enrollment / [redacted] 15	RBC	4.51 (10E6/uL)	3.35	4.05
	Enrollment / [redacted] 15	WBC	9.3 (10E3/uL)	5	19.5
	Enrollment / [redacted] 29	ALB	4.4 (g/dL)	3.6	4.6
	Enrollment / [redacted] 29	ALP	535 (U/L)	406	1100
	Enrollment / [redacted] 29	ALT	36 (U/L)	23	39
	Enrollment / [redacted] 29	AST	40 (U/L)	24	40
	Enrollment / [redacted] 29	BILD	0 (mg/dL)	0	0.4
	Enrollment / [redacted] 29	BILI	0.3 (mg/dL)	0.2	1.2

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 29	HCT	36.8 (%)	37.4	48.6
	Enrollment / [redacted] 29	HGB	11.9 (g/dL)	10.3	12.3
	Enrollment / [redacted] 29	PLAT	484 (10E3/uL)	138	309
	Enrollment / [redacted] 29	PROT	6 (g/dL)	5.2	6.7
	Enrollment / [redacted] 29	RBC	4.47 (10E6/uL)	3.5	4.7
	Enrollment / [redacted] 29	WBC	10.4 (10E3/uL)	5	19.5
	Enrollment / [redacted] 36	ALB	4.4 (g/dL)	3.8	4.7
	Enrollment / [redacted] 36	ALP	580 (U/L)	386	1070
	Enrollment / [redacted] 36	ALT	35 (U/L)	13	64
	Enrollment / [redacted] 36	AST	41 (U/L)	24	43
	Enrollment / [redacted] 36	BILD	0 (mg/dL)	0	0.4
	Enrollment / [redacted] 36	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 36	HCT	37.8 (%)	37.4	48.6
	Enrollment / [redacted] 36	HGB	12.4 (g/dL)	10.3	12.3
	Enrollment / [redacted] 36	PLAT	482 (10E3/uL)	138	309
	Enrollment / [redacted] 36	PROT	6 (g/dL)	5.4	6.9
	Enrollment / [redacted] 36	RBC	4.73 (10E6/uL)	3.35	4.05
	Enrollment / [redacted] 36	WBC	8.7 (10E3/uL)	5	19.5
	Enrollment / [redacted] 41	ALB	5.1 (g/dL)	3.8	4.7
	Enrollment / [redacted] 41	ALP	636 (U/L)	386	1070
	Enrollment / [redacted] 41	ALT	56 (U/L)	13	64
	Enrollment / [redacted] 41	AST	100 (U/L)	24	43
	Enrollment / [redacted] 41	BILD	0 (mg/dL)	0	0.4

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 41	BILI	0.2 (mg/dL)	0.2	1.2
	Enrollment / [redacted] / 41	HCT	38 (%)	37.4	48.6
	Enrollment / [redacted] / 41	HGB	12.4 (g/dL)	10.3	12.3
	Enrollment / [redacted] / 41	PLAT	214 (10E3/uL)	138	309
	Enrollment / [redacted] / 41	PROT	7.1 (g/dL)	5.4	6.9
	Enrollment / [redacted] / 41	RBC	4.71 (10E6/uL)	3.35	4.05
	Enrollment / [redacted] / 41	WBC	4.7 (10E3/uL)	5	19.5
	Enrollment / [redacted] / 44	ALB	4.5 (g/dL)	3.8	4.7
	Enrollment / [redacted] / 44	ALP	575 (U/L)	386	1070
	Enrollment / [redacted] / 44	ALT	42 (U/L)	13	64
	Enrollment / [redacted] / 44	AST	90 (U/L)	24	43
	Enrollment / [redacted] / 44	BILD	0 (mg/dL)	0	0.4
	Enrollment / [redacted] / 44	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / [redacted] / 44	HCT	40.2 (%)	37.4	48.6
	Enrollment / [redacted] / 44	HGB	13.3 (g/dL)	10.3	12.3
	Enrollment / [redacted] / 44	PLAT	143 (10E3/uL)	138	309
	Enrollment / [redacted] / 44	PROT	6.3 (g/dL)	5.4	6.9
	Enrollment / [redacted] / 44	RBC	4.96 (10E6/uL)	3.35	4.05
	Enrollment / [redacted] / 44	WBC	10.9 (10E3/uL)	5	19.5
	Enrollment / [redacted] / 51	ALB	4.2 (g/dL)	3.8	4.7
	Enrollment / [redacted] / 51	ALP	478 (U/L)	386	1070
	Enrollment / [redacted] / 51	ALT	26 (U/L)	13	64
	Enrollment / [redacted] / 51	AST	46 (U/L)	24	43

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 51	BILD	0.1 (mg/dL)	0	0.4
	Enrollment / [redacted] 51	BILI	0.2 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 51	HCT	38.3 (%)	37.4	48.6
	Enrollment / [redacted] 51	HGB	13 (g/dL)	10.3	12.3
	Enrollment / [redacted] 51	PLAT	349 (10E3/uL)	138	309
	Enrollment / [redacted] 51	PROT	5.9 (g/dL)	5.4	6.9
	Enrollment / [redacted] 51	RBC	4.83 (10E6/uL)	3.35	4.05
	Enrollment / [redacted] 51	WBC	7.2 (10E3/uL)	5	19.5
	Enrollment / [redacted] 58	ALB	4.6 (g/dL)	3.8	4.7
	Enrollment / [redacted] 58	ALP	478 (U/L)	386	1070
	Enrollment / [redacted] 58	ALT	28 (U/L)	13	64
	Enrollment / [redacted] 58	AST	49 (U/L)	24	43
	Enrollment / [redacted] 58	BILD	0.1 (mg/dL)	0	0.4
	Enrollment / [redacted] 58	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 58	HCT	43.2 (%)	37.4	48.6
	Enrollment / [redacted] 58	HGB	14.1 (g/dL)	10.3	12.3
	Enrollment / [redacted] 58	PLAT	373 (10E3/uL)	138	309
	Enrollment / [redacted] 58	PROT	6.6 (g/dL)	5.4	6.9
	Enrollment / [redacted] 58	RBC	5.41 (10E6/uL)	3.35	4.05
	Enrollment / [redacted] 58	WBC	9.5 (10E3/uL)	5	19.5
	Enrollment / [redacted] 65	ALB	4.8 (g/dL)	3.8	4.7
	Enrollment / [redacted] 65	ALP	474 (U/L)	386	1070
	Enrollment / [redacted] 65	ALT	29 (U/L)	13	64

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 65	AST	46 (U/L)	24	43
	Enrollment / [redacted] 65	BILD	0 (mg/dL)	0	0.4
	Enrollment / [redacted] 65	BILI	0.2 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 65	HCT	42.2 (%)	37.4	48.6
	Enrollment / [redacted] 65	HGB	13.8 (g/dL)	10.3	12.3
	Enrollment / [redacted] 65	PLAT	373 (10E3/uL)	138	309
	Enrollment / [redacted] 65	PROT	6.6 (g/dL)	5.4	6.9
	Enrollment / [redacted] 65	RBC	5.3 (10E6/uL)	3.35	4.05
	Enrollment / [redacted] 65	WBC	5.5 (10E3/uL)	5	19.5
	Enrollment / [redacted] 79	ALB	4.7 (g/dL)	3.8	4.7
	Enrollment / [redacted] 79	ALP	501 (U/L)	386	1070
	Enrollment / [redacted] 79	ALT	37 (U/L)	13	64
	Enrollment / [redacted] 79	AST	57 (U/L)	24	43
	Enrollment / [redacted] 79	BILD	0 (mg/dL)	0	0.4
	Enrollment / [redacted] 79	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 79	HCT	43.2 (%)	37.4	48.6
	Enrollment / [redacted] 79	HGB	14 (g/dL)	10.3	12.3
	Enrollment / [redacted] 79	PLAT	421 (10E3/uL)	138	309
	Enrollment / [redacted] 79	PROT	6.6 (g/dL)	5.4	6.9
	Enrollment / [redacted] 79	RBC	5.4 (10E6/uL)	3.35	4.05
	Enrollment / [redacted] 79	WBC	7.9 (10E3/uL)	5	19.5
	Enrollment / [redacted] 92	ALB	4.6 (g/dL)	3.8	4.7
	Enrollment / [redacted] 92	ALP	526 (U/L)	386	1070

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 92	ALT	35 (U/L)	13	64
	Enrollment / [redacted] 92	AST	59 (U/L)	24	43
	Enrollment / [redacted] 92	BILD	0 (mg/dL)	0	0.4
	Enrollment / [redacted] 92	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 92	HCT	41.5 (%)	37.4	48.6
	Enrollment / [redacted] 92	HGB	13.5 (g/dL)	10.3	12.3
	Enrollment / [redacted] 92	PLAT	375 (10E3/uL)	138	309
	Enrollment / [redacted] 92	PROT	6.3 (g/dL)	5.4	6.9
	Enrollment / [redacted] 92	RBC	5.27 (10E6/uL)	3.35	4.05
	Enrollment / [redacted] 92	WBC	6.1 (10E3/uL)	5	19.5
	Enrollment / [redacted] / -1	ALP	878 (IU/L)	106	322
	Enrollment / [redacted] / -1	ALT	14 (IU/L)	7	23
	Enrollment / [redacted] / -1	AST	24 (IU/L)	13	30
	Enrollment / [redacted] / -1	BILD	0.03 (mg/dL)	0.1	0.3
	Enrollment / [redacted] / -1	BILI	0.31 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / -1	HCT	36.9 (%)	11.6	14.8
	Enrollment / [redacted] / -1	HGB	12.4 (g/dL)	11.6	14.8
	Enrollment / [redacted] / -1	PLAT	423 (10E3/uL)	158	348
	Enrollment / [redacted] / -1	PROT	7.1 (g/dL)	6.6	8.1
	Enrollment / [redacted] / -1	RBC	4.59 (10E12/L)	3.86	4.92
	Enrollment / [redacted] / -1	WBC	11.2 (10E9/L)	3.3	8.6

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 267	ALB	4.9 (g/dL)	4.1	5.1
	Enrollment / [redacted] 267	ALP	629 (U/L)	-	-
	Enrollment / [redacted] 267	ALT	22 (U/L)	10	42
	Enrollment / [redacted] 267	AST	42 (U/L)	13	30
	Enrollment / [redacted] 267	BILD	0.1 (mg/dL)	0	0.2
	Enrollment / [redacted] 267	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 267	HCT	39.9 (%)	40.7	50.1
	Enrollment / [redacted] 267	HGB	12.7 (g/dL)	13.7	16.8
	Enrollment / [redacted] 267	PLAT	325 (10E3/uL)	158	348
	Enrollment / [redacted] 267	PROT	7.1 (g/dL)	6.6	8.1
	Enrollment / [redacted] 267	RBC	5.54 (10E6/uL)	4.35	5.55
	Enrollment / [redacted] 267	WBC	12 (10E3/uL)	3.3	8.6
[redacted]	Enrollment [redacted] / 0	ALT	21 (U/L)	7	23
	Enrollment [redacted] / 0	AST	32 (U/L)	13	30
	Enrollment [redacted] / 0	PLAT	396 (10E3/uL)	158	348
[redacted]	Enrollment / [redacted] 307	ALB	4.8 (g/dL)	-	-
	Enrollment / [redacted] 307	ALP	178 (U/L)	-	-
	Enrollment / [redacted] 307	ALT	23 (U/L)	-	-
	Enrollment / [redacted] 307	AST	43 (U/L)	-	-
	Enrollment / [redacted] 307	BILI	0.7 (mg/dL)	-	-
	Enrollment / [redacted] 307	HCT	36.4 (%)	-	-

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 307	HGB	12.1 (g/dL)	-	-
	Enrollment / [redacted] / 307	PLAT	327 (10E3/uL)	-	-
	Enrollment / [redacted] / 307	PROT	6.9 (g/dL)	-	-
	Enrollment / [redacted] / 307	RBC	4.32 (10E6/uL)	-	-
	Enrollment / [redacted] / 307	WBC	6.2 (10E3/uL)	-	-
	Enrollment / [redacted] / 311	ALT	956 (U/L)	-	-
	Enrollment / [redacted] / 311	AST	1370 (U/L)	-	-
	Enrollment / [redacted] / 311	PLAT	152 (10E3/uL)	-	-
	Enrollment / [redacted] / 482	ALT	137 (U/L)	10	42
	Enrollment / [redacted] / 482	AST	100 (U/L)	13	30
	Enrollment / [redacted] / 362	ALB	4 (g/dL)	4.1	5.1
	Enrollment / [redacted] / 362	ALP	472 (U/L)	106	322
	Enrollment / [redacted] / 362	ALT	80 (U/L)	7	23
	Enrollment / [redacted] / 362	AST	55 (U/L)	13	30
	Enrollment / [redacted] / 362	BILI	0.5 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / 362	HCT	33.9 (%)	35.1	44.4
	Enrollment / [redacted] / 362	HGB	10.9 (g/dL)	11.6	14.8
	Enrollment / [redacted] / 362	PLAT	336 (10E3/uL)	158	348
	Enrollment / [redacted] / 362	PROT	6.3 (g/dL)	6.6	8.1
	Enrollment / [redacted] / 362	RBC	4.07 (10E6/uL)	386	492
	Enrollment / [redacted] / 362	WBC	6.2 (10E3/uL)	3.3	8.6

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 134	ALB	4.1 (g/dL)	4.1	5.1
	Enrollment / [redacted] 134	ALP	528 (U/L)	106	322
	Enrollment / [redacted] 134	ALT	25 (U/L)	7	23
	Enrollment / [redacted] 134	AST	37 (U/L)	13	30
	Enrollment / [redacted] 134	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 134	HCT	35.5 (%)	35.1	44.4
	Enrollment / [redacted] 134	HGB	12 (g/dL)	11.6	14.8
	Enrollment / [redacted] 134	PLAT	301 (10E3/uL)	158	348
	Enrollment / [redacted] 134	PROT	6.2 (g/dL)	6.6	8.1
	Enrollment / [redacted] 134	RBC	4.2 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] 134	WBC	8 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] / 16	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment / [redacted] / 16	ALT	27 (U/L)	7	23
	Enrollment / [redacted] / 16	AST	30 (U/L)	13	30
	Enrollment / [redacted] / 16	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / 16	HCT	35.9 (%)	35.1	44.4
	Enrollment / [redacted] / 16	HGB	12 (g/dL)	11.6	14.8
	Enrollment / [redacted] / 16	PLAT	488 (10E3/uL)	158	348
	Enrollment / [redacted] / 16	PROT	6.5 (g/dL)	6.6	8.1
	Enrollment / [redacted] / 16	RBC	4.35 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] / 16	WBC	10.5 (10E3/uL)	3.3	8.6

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 212	ALT	12 (U/L)	7	23
	Enrollment / [redacted] 212	AST	29 (U/L)	13	30
	Enrollment / [redacted] 212	BILI	0.8 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 212	HGB	11.8 (g/dL)	10.9	14.2
	Enrollment / [redacted] 212	PLAT	176 (10E3/uL)	180	620
	Enrollment / [redacted] 212	RBC	4.39 (10E6/uL)	4.35	5.05
	Enrollment / [redacted] 212	WBC	10000 (cells/uL)	4200	18800
	Enrollment [redacted] / 0	ALB	3.8 (g/dL)	4.1	5.1
	Enrollment [redacted] / 0	ALT	35 (U/L)	10	42
	Enrollment [redacted] / 0	AST	35 (U/L)	13	30
	Enrollment [redacted] / 0	HCT	34.9 (%)	-	-
	Enrollment [redacted] / 0	HGB	11.7 (g/dL)	-	-
	Enrollment [redacted] / 0	PLAT	538 (10E3/uL)	-	-
	Enrollment [redacted] / 0	PROT	5.3 (g/dL)	6.6	8.1
	Enrollment [redacted] / 0	RBC	4.17 (10E6/uL)	-	-
	Enrollment [redacted] / 0	WBC	810 (10E3/uL)	-	-
	Enrollment [redacted] 20	ALB	4.1 (g/dL)	3.9	4.9
	Enrollment [redacted] 20	ALT	20 (U/L)	8	42
	Enrollment [redacted] 20	AST	26 (U/L)	13	33
	Enrollment [redacted] 20	BILI	0.38 (mg/dL)	0.3	1.2
	Enrollment [redacted] 20	HCT	38.3 (%)	40	50

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 20	HGB	12 (g/dL)	13.5	17
	Enrollment / [redacted] 20	PLAT	39.8 (10E4/uL)	15	35
	Enrollment / [redacted] 20	PROT	5.8 (g/dL)	6.7	8.3
	Enrollment / [redacted] 20	RBC	4.85 (10E6/uL)	4.3	5.7
	Enrollment / [redacted] 20	WBC	8.48 (10E3/uL)	3.5	8.5
	Enrollment / [redacted] 24	AST	66 (U/L)	13	30
	Enrollment / [redacted] 24	PLAT	19.1 (10E4/uL)	15	35
	Enrollment / [redacted] 25	ALT	46 (U/L)	8	42
	Enrollment / [redacted] 26	HGB	10.9 (g/dL)	13.5	17
	Enrollment / [redacted] 27	PLAT	3 (10E4/uL)	15	35
	Enrollment / [redacted] 28	ALB	4.19 (g/dL)	3.9	4.9
	Enrollment / [redacted] 28	ALP	166 (IU/L)	128	469
	Enrollment / [redacted] 28	ALT	14 (IU/L)	4	44
	Enrollment / [redacted] 28	AST	28 (IU/L)	8	38
	Enrollment / [redacted] 28	BILI	0.3 (mg/dL)	0.2	1.2
	Enrollment / [redacted] 28	HGB	12.1 (g/dL)	13.5	17.6
	Enrollment / [redacted] 28	PLAT	36.2 (10E4/uL)	13	36.2
	Enrollment / [redacted] 28	PROT	6.25 (g/dL)	6.7	8.3
	Enrollment / [redacted] 28	RBC	4.41 (10E6/uL)	4.1	5.3
	Enrollment / [redacted] 28	WBC	10160 (cells/uL)	3900	9800
	Enrollment / [redacted] 32	AST	54 (IU/L)	8	38
	Enrollment / [redacted] 35	ALT	72 (IU/L)	4	44

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 35	AST	126 (IU/L)	8	38
	Enrollment / [redacted] 35	PLAT	8.2 (10E4/uL)	13	36.2
	Enrollment / [redacted] 39	ALT	403 (IU/L)	4	44
	Enrollment / [redacted] 39	AST	633 (IU/L)	8	38
	Enrollment / [redacted] 39	HGB	9.5 (g/dL)	13.5	17.6
	Enrollment / [redacted] 39	PLAT	3 (10E4/uL)	13	36.2
	Enrollment / [redacted] 43	ALT	625 (IU/L)	4	44
	Enrollment / [redacted] 43	AST	505 (IU/L)	8	38
	Enrollment / [redacted] 43	HGB	8.9 (g/dL)	13.5	17.6
	Enrollment / [redacted] 43	PLAT	11.4 (10E4/uL)	13	36.2
	Enrollment / [redacted] 46	ALT	504 (IU/L)	4	44
	Enrollment / [redacted] 46	AST	251 (IU/L)	3	38
	Enrollment / [redacted] 49	ALT	258 (IU/L)	4	44
	Enrollment / [redacted] 49	AST	95 (IU/L)	8	38
	Enrollment / [redacted] 57	ALT	161 (IU/L)	4	44
	Enrollment / [redacted] 57	AST	151 (IU/L)	8	38
	Enrollment / [redacted] 71	ALT	1710 (IU/L)	4	44
	Enrollment / [redacted] 71	AST	2160 (IU/L)	8	38
	Enrollment / [redacted] 78	ALT	1206 (IU/L)	4	44
	Enrollment / [redacted] 78	AST	1112 (IU/L)	8	38
	Enrollment / [redacted] 87	ALT	379 (IU/L)	4	44
	Enrollment / [redacted] 87	AST	272 (IU/L)	8	38
	Enrollment / [redacted] 99	ALT	71 (IU/L)	4	44

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 99	AST	60 (IU/L)	8	38
	Enrollment / [redacted] 113	ALT	23 (IU/L)	4	44
	Enrollment / [redacted] 113	AST	35 (IU/L)	8	38
	Enrollment / [redacted] / -6	ALB	4.3 (g/dL)	4.1	5.1
	Enrollment / [redacted] / -6	ALP	193 (IU/L)	38	113
	Enrollment / [redacted] / -6	ALT	12 (IU/L)	7	23
	Enrollment / [redacted] / -6	AST	34 (IU/L)	13	30
	Enrollment / [redacted] / -6	BILD	0.1 (mg/dL)	0	0.3
	Enrollment / [redacted] / -6	BILI	0.3 (mg/dL)	0.4	1.5
	Enrollment / [redacted] / -6	HCT	36.7 (%)	35.1	44.4
	Enrollment / [redacted] / -6	HGB	11.6 (g/dL)	11.6	14.8
	Enrollment / [redacted] / -6	PLAT	425 (10E3/uL)	158	348
	Enrollment / [redacted] / -6	PROT	6.6 (g/dL)	6.6	8.1
	Enrollment / [redacted] / -6	RBC	4.51 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] / -6	WBC	8.3 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 294	ALB	4.6 (g/dL)	4.1	5.1
	Enrollment / [redacted] 294	ALT	50 (U/L)	7	23
	Enrollment / [redacted] 294	AST	42 (U/L)	13	30
	Enrollment / [redacted] 294	HCT	38.6 (%)	35.1	44.4
	Enrollment / [redacted] 294	HGB	13.8 (g/dL)	11.6	14.8
	Enrollment / [redacted] 294	PLAT	295 (10E3/uL)	300	450

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 294	PROT	7.1 (g/dL)	6.6	8.1
	Enrollment / [redacted] 294	RBC	4.55 (10E6/uL)	3.86	4.92
	Enrollment / [redacted] 294	WBC	10.4 (10E3/uL)	3.3	8.6
	Enrollment [redacted] / 9	ALB	4.6 (g/dL)	4.1	5.1
	Enrollment [redacted] / 9	ALP	156 (U/L)	38	113
	Enrollment [redacted] / 9	ALT	16 (U/L)	7	23
	Enrollment [redacted] / 9	AST	33 (U/L)	13	30
	Enrollment [redacted] / 9	BILD	0 (mg/dL)	0	0.4
	Enrollment [redacted] / 9	BILI	0.4 (mg/dL)	0.2	1.2
	Enrollment [redacted] / 9	HCT	35.1 (%)	35.1	44.4
	Enrollment [redacted] / 9	HGB	11.3 (g/dL)	11.6	14.8
	Enrollment [redacted] / 9	PLAT	320 (10E3/uL)	300	450
	Enrollment [redacted] / 9	PROT	6.8 (g/dL)	6.6	8.1
	Enrollment [redacted] / 9	RBC	4.35 (10E6/uL)	3.86	4.92
	Enrollment [redacted] / 9	WBC	16.3 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 15	AST	93 (U/L)	13	30
	Enrollment / [redacted] 15	PLAT	83 (10E3/uL)	300	450
	Enrollment / [redacted] 18	PLAT	63 (10E3/uL)	300	450
	Enrollment / [redacted] 21	AST	718 (U/L)	13	30
	Enrollment [redacted] / 0	ALT	29 (U/L)	10	42
	Enrollment [redacted] / 0	AST	44 (U/L)	13	30

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment [redacted] / 0	BILI	1 (mg/dL)	0.4	1.5
	Enrollment [redacted] / 0	HCT	35.1 (%)	40.7	50.1
	Enrollment [redacted] / 0	HGB	11.5 (g/dL)	13.7	16.8
	Enrollment [redacted] / 0	PLAT	355 (10E3/uL)	158	348
	Enrollment [redacted] / 0	RBC	4.13 (10E6/uL)	4.35	5.55
	Enrollment [redacted] / 0	WBC	12.8 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 101	ALB	4.4 (g/dL)	4	5
	Enrollment / [redacted] 101	ALP	163 (IU/L)	38	113
	Enrollment / [redacted] 101	ALT	14 (IU/L)	8	42
	Enrollment / [redacted] 101	AST	29 (IU/L)	13	33
	Enrollment / [redacted] 101	BILI	0.4 (mg/dL)	0.3	1.2
	Enrollment / [redacted] 101	HCT	35.9 (%)	39.7	51
	Enrollment / [redacted] 101	HGB	11.6 (g/dL)	13.5	17
	Enrollment / [redacted] 101	PLAT	438 (10E3/uL)	130	350
	Enrollment / [redacted] 101	PROT	6.4 (g/dL)	6.7	8.3
	Enrollment / [redacted] 101	RBC	4.51 (10E6/uL)	4.3	5.5
	Enrollment / [redacted] 101	WBC	11.17 (10E3/uL)	3.3	8.8
	Enrollment / [redacted] -20	ALB	4.6 (g/dL)	3.5	5.5
	Enrollment / [redacted] -20	ALP	427 (IU/L)	106	322
	Enrollment / [redacted] -20	ALT	22 (IU/L)	10	42
	Enrollment / [redacted] -20	AST	54 (IU/L)	13	30

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted]-20	BILI	0.4 (mg/dL)	0.1	1.2
	Enrollment / [redacted]-20	HCT	31.9 (%)	40	50
	Enrollment / [redacted]-20	HGB	10.9 (g/dL)	13	17
	Enrollment / [redacted]-20	PLAT	310 (10E3/uL)	150	350
	Enrollment / [redacted]-20	PROT	6.5 (g/dL)	6.5	8.2
	Enrollment / [redacted]-20	RBC	4.22 (10E6/uL)	4	5.4
	Enrollment / [redacted]-20	WBC	11.8 (10E3/uL)	4.7	8.7
	Enrollment / [redacted] 20	ALB	4 (g/dL)	3.5	5.5
	Enrollment / [redacted] 20	ALT	28 (IU/L)	10	42
	Enrollment / [redacted] 20	AST	46 (IU/L)	13	30
	Enrollment / [redacted] 20	BILD	1.5 (mg/dL)	0	0.3
	Enrollment / [redacted] 20	BILI	7.7 (mg/dL)	0.1	1.2
	Enrollment / [redacted] 20	HCT	47.4 (%)	40	50
	Enrollment / [redacted] 20	HGB	16.7 (g/dL)	13	17
	Enrollment / [redacted] 20	PLAT	39.9 (10E4/uL)	15	34.8
	Enrollment / [redacted] 20	PROT	5.5 (g/dL)	6.5	8.2
	Enrollment / [redacted] 20	RBC	5 (10E6/uL)	4.4	5.6
	Enrollment / [redacted] 20	WBC	12.3 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] 25	ALT	63 (IU/L)	10	42
	Enrollment / [redacted] 25	AST	248 (IU/L)	13	30
	Enrollment / [redacted] 25	PLAT	10 (10E4/uL)	15	34.8
	Enrollment / [redacted] 26	ALT	71 (IU/L)	10	42

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 26	PLAT	6.9 (10E4/uL)	15	34.8
	Enrollment / [redacted] -15	ALT	24 (U/L)	12	51
	Enrollment / [redacted] -15	AST	37 (U/L)	25	67
	Enrollment / [redacted] -15	BILI	0.48 (mg/dL)	0.12	0.59
	Enrollment / [redacted] -15	HCT	40.9 (%)	30	41
	Enrollment / [redacted] -15	HGB	12.8 (g/dL)	10	14
	Enrollment / [redacted] -15	PLAT	348 (10E3/uL)	200	740
	Enrollment / [redacted] 22	ALB	3.7 (g/dL)	3.1	3.6
	Enrollment / [redacted] 22	ALT	39 (U/L)	13	66
	Enrollment / [redacted] 22	AST	33 (U/L)	22	66
	Enrollment / [redacted] 22	BILD	0.08 (mg/dL)	-	-
	Enrollment / [redacted] 22	BILI	0.16 (mg/dL)	0.13	0.8
	Enrollment / [redacted] 22	HCT	22.5 (%)	28.5	41.1
	Enrollment / [redacted] 22	HGB	7.4 (g/dL)	9.5	13.7
	Enrollment / [redacted] 22	PLAT	75.2 (10E4/uL)	25	82
	Enrollment / [redacted] 22	PROT	5.8 (g/dL)	5.1	5.8
	Enrollment / [redacted] 22	RBC	2.64 (10E6/uL)	3.4	5
	Enrollment / [redacted] 22	WBC	7.83 (10E3/uL)	4.56	18.9
	Enrollment / [redacted] 51	ALT	54 (U/L)	13	56
	Enrollment / [redacted] 51	AST	73 (U/L)	23	67
	Enrollment / [redacted] 67	ALT	112 (U/L)	13	56

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 67	AST	117 (U/L)	23	67
	Enrollment / [redacted] -15	ALB	4 (g/dL)	3.7	5.5
	Enrollment / [redacted] -15	ALP	635 (U/L)	106	322
	Enrollment / [redacted] -15	ALT	22 (U/L)	5	45
	Enrollment / [redacted] -15	AST	40 (U/L)	10	40
	Enrollment / [redacted] -15	BILD	0.1 (mg/dL)	0	0.4
	Enrollment / [redacted] -15	BILI	0.5 (mg/dL)	0.3	1.2
	Enrollment / [redacted] -15	HCT	37.8 (%)	34.3	45.2
	Enrollment / [redacted] -15	HGB	11.5 (g/dL)	11.2	15.2
	Enrollment / [redacted] -15	PLAT	430 (10E3/uL)	140	380
	Enrollment / [redacted] -15	PROT	6.1 (g/dL)	6.5	8.2
	Enrollment / [redacted] -15	WBC	7.38 (10E3/uL)	3.5	9.7
	Enrollment [redacted] / 3	ALT	21 (U/L)	4	44
	Enrollment [redacted] / 3	AST	42 (U/L)	8	38
	Enrollment [redacted] / 3	PLAT	414 (10E3/uL)	140	340
	Enrollment / [redacted] 35	ALB	4.9 (g/dL)	4.1	5.11
	Enrollment / [redacted] 35	ALP	163 (IU/L)	38	113
	Enrollment / [redacted] 35	ALT	14 (IU/L)	7	23
	Enrollment / [redacted] 35	AST	42 (IU/L)	13	30
	Enrollment / [redacted] 35	BILD	0 (mg/dL)	0	0.3

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] 35	BILI	0.4 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 35	HCT	37.5 (%)	35.1	44.4
	Enrollment / [redacted] 35	HGB	12.4 (g/dL)	11.6	14.8
	Enrollment / [redacted] 35	PLAT	352 (10E3/uL)	158	348
	Enrollment / [redacted] 35	PROT	6.7 (g/dL)	6.6	8.1
	Enrollment / [redacted] 35	RBC	4.71 (10E12/L)	3.86	4.92
	Enrollment / [redacted] 35	WBC	9.1 (10E9/L)	3.3	8.6
	Enrollment / [redacted] 42	ALB	3.8 (g/dL)	3.1	4.6
	Enrollment / [redacted] 42	ALP	208 (U/L)	168	567
	Enrollment / [redacted] 42	ALT	26 (U/L)	13	56
	Enrollment / [redacted] 42	AST	38 (U/L)	22	66
	Enrollment / [redacted] 42	BILI	0.2 (mg/dL)	0.1	0.8
	Enrollment / [redacted] 42	HCT	36.6 (%)	30	41.6
	Enrollment / [redacted] 42	HGB	12.4 (g/dL)	10	14.2
	Enrollment / [redacted] 42	PLAT	560 (10E3/uL)	220	760
	Enrollment / [redacted] 42	PROT	5.5 (g/dL)	5.1	6.8
	Enrollment / [redacted] 42	RBC	4.34 (10E6/uL)	3.8	5.23
	Enrollment / [redacted] 42	WBC	11.1 (10E3/uL)	4.4	19.1
	Enrollment / [redacted] 48	ALT	385 (U/L)	9	38
	Enrollment / [redacted] 48	AST	661 (U/L)	23	57
	Enrollment / [redacted] 48	PLAT	129 (10E3/uL)	220	760

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] -50	ALP	186 (U/L)	38	113
	Enrollment [redacted] / -5	ALB	4.4 (g/dL)	4.1	5.1
	Enrollment [redacted] / -5	ALT	14 (U/L)	7	23
	Enrollment [redacted] / -5	AST	28 (U/L)	13	30
	Enrollment [redacted] / -5	BILI	0.4 (mg/dL)	0.4	1.5
	Enrollment [redacted] / -5	HCT	41.1 (%)	35.1	44.4
	Enrollment [redacted] / -5	HGB	12.4 (g/dL)	11.6	14.8
	Enrollment [redacted] / -5	PLAT	31.4 (10E4/uL)	15	34
	Enrollment [redacted] / -5	PROT	7 (g/dL)	6.6	8.1
	Enrollment [redacted] / -5	RBC	4.99 (10E6/L)	3.86	4.92
	Enrollment [redacted] / -5	WBC	8.8 (10E3/uL)	3.3	8.6
	Enrollment [redacted] / 8	ALT	36 (U/L)	7	23
	Enrollment [redacted] / 8	AST	101 (U/L)	13	30
	Enrollment [redacted] / 8	PLAT	6 (10E4/uL)	15	34
	Enrollment / [redacted] / 71	ALT	121 (U/L)	7	23
	Enrollment / [redacted] / 71	AST	130 (U/L)	13	30
[redacted]	Enrollment / [redacted] 151	ALB	4.7 (g/dL)	4.1	5.1
	Enrollment / [redacted] 151	ALP	324 (U/L)	38	113
	Enrollment / [redacted] 151	ALT	19 (U/L)	10	42
	Enrollment / [redacted] 151	AST	42 (U/L)	13	30
	Enrollment / [redacted] 151	BILI	0.2 (mg/dL)	0.4	1.5
	Enrollment / [redacted] 151	HCT	40.8 (%)	40.7	50.1

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Listing 16.4.1.jpn
Listing of Laboratory Values at Baseline
Japan OAV101 Treated Patients

Patient	Visit/ Date/ Study Day	Test	Result (units)	Clinically Significant Limits	
				Very Low	Very High
[redacted]	Enrollment / [redacted] / 151	HGB	13.8 (g/dL)	13.7	16.8
	Enrollment / [redacted] / 151	PLAT	444 (10E3/uL)	158	348
	Enrollment / [redacted] / 151	PROT	6.6 (g/dL)	6.6	8.1
	Enrollment / [redacted] / 151	RBC	5 (10E6/uL)	4.35	5.55
	Enrollment / [redacted] / 151	WBC	7 (10E3/uL)	3.3	8.6
	Enrollment / [redacted] / 155	AST	79 (U/L)	13	30
	Enrollment / [redacted] / 157	AST	100 (U/L)	13	30
	Enrollment / [redacted] / 203	ALB	2.7 (g/dL)	4.1	5.1
	Enrollment / [redacted] / 203	ALT	6128 (U/L)	10	42
	Enrollment / [redacted] / 203	AST	24297 (U/L)	13	30
	Enrollment / [redacted] / 203	HGB	11.8 (g/dL)	13.7	16.8
	Enrollment / [redacted] / 203	PLAT	142 (10E3/uL)	158	348
	Enrollment / [redacted] / 203	PROT	4.5 (g/dL)	6.6	8.1

Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Switch to OAV101	[redacted] / 371	ALT	342 (U/L)	3	49	Yes
RESTORE		Switch to OAV101	/ 394	ALT	90 (U/L)	3	49	
RESTORE		Switch to OAV101	/ 400	ALT	185 (U/L)	3	49	Yes
RESTORE		Switch to OAV101	/ 406	ALT	329 (U/L)	3	49	Yes
RESTORE		Switch to OAV101	/ 414	ALT	227 (U/L)	3	49	Yes
RESTORE		Switch to OAV101	/ 421	ALT	128 (U/L)	3	49	
RESTORE		Switch to OAV101	/ 429	ALT	69 (U/L)	3	49	
RESTORE		Switch to OAV101	/ 435	ALT	50 (U/L)	3	49	
RESTORE		Switch to OAV101	/ 554	ALT	210 (U/L)	3	49	Yes
RESTORE		Switch to OAV101	/ 568	ALT	71 (U/L)	3	49	
RESTORE		Switch to OAV101	/ 595	ALT	182 (U/L)	3	49	Yes
RESTORE		Switch to OAV101	/ 632	ALT	86 (U/L)	3	49	
RESTORE		Switch to OAV101	/ 371	AST	529 (U/L)	9	37	Yes
RESTORE		Switch to OAV101	/ 394	AST	97 (U/L)	9	37	
RESTORE		Switch to OAV101	/ 400	AST	158 (U/L)	9	37	Yes
RESTORE		Switch to OAV101	/ 406	AST	228 (U/L)	9	37	Yes
RESTORE		Switch to OAV101	/ 414	AST	132 (U/L)	9	37	Yes
RESTORE		Switch to OAV101	/ 421	AST	86 (U/L)	9	37	
RESTORE		Switch to OAV101	/ 429	AST	66 (U/L)	9	37	
RESTORE		Switch to OAV101	/ 435	AST	55 (U/L)	9	37	
RESTORE		Switch to OAV101	/ 554	AST	171 (U/L)	9	37	Yes
RESTORE		Switch to OAV101	/ 568	AST	71 (U/L)	9	37	
RESTORE		Switch to OAV101	/ 595	AST	139 (U/L)	9	37	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Switch to OAV101	[redacted] 632	AST	86 (U/L)	9	37	
RESTORE		OAV101 mono	/ 1	ALP	431 (U/L)	38	113	Yes
RESTORE		OAV101 mono	/ 7	AST	142 (U/L)	9	37	Yes
RESTORE		OAV101 mono	/ 9	AST	334 (U/L)	9	37	Yes
RESTORE		OAV101 mono	/ 15	AST	71 (U/L)	9	37	
RESTORE		OAV101 mono	/ 14	WBC	37.5 (10E3/uL)	3.5	9.1	Yes
RESTORE		OAV101 mono	/ 28	WBC	21.3 (10E3/uL)	3.5	9.1	
RESTORE		Switch to OAV101	542	ALT	145 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	896	ALT	17 (U/L)	7	23	
RESTORE		Switch to OAV101	541	AST	164 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	542	AST	287 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	896	AST	35 (U/L)	13	30	
RESTORE		Switch to OAV101	105	ALT	2871 (U/L)	10	42	Yes
RESTORE		Switch to OAV101	149	ALT	64 (U/L)	10	42	
RESTORE		Switch to OAV101	163	ALT	235 (U/L)	10	42	Yes
RESTORE		Switch to OAV101	/ 97	AST	123 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	105	AST	1856 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	149	AST	75 (U/L)	13	30	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Switch to OAV101	[redacted] / 163	AST	280 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 158	ALT	787 (U/L)	12	50.5	Yes
RESTORE		Bridge to OAV101	/ 159	ALT	924 (U/L)	12	50.5	Yes
RESTORE		Bridge to OAV101	/ 158	AST	1527 (U/L)	24.5	66.5	Yes
RESTORE		Bridge to OAV101	/ 49	ALT	617 (U/L)	9.4	38.4	Yes
RESTORE		Bridge to OAV101	/ 19	AST	585 (U/L)	23	56.5	Yes
RESTORE		Switch to OAV101	/ 494	ALT	97 (U/L)	6	27	Yes
RESTORE		Switch to OAV101	/ 498	ALT	239 (U/L)	6	27	Yes
RESTORE		Switch to OAV101	/ 550	ALT	333 (U/L)	6	27	Yes
RESTORE		Switch to OAV101	/ 678	ALT	65 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 706	ALT	66 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 736	ALT	41 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 739	ALT	35 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 741	ALT	32 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 776	ALT	33 (U/L)	6	27	
RESTORE		Switch to OAV101	/ 494	AST	270 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	/ 498	AST	423 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	/ 550	AST	181 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	/ 678	AST	62 (U/L)	13	30	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Switch to OAV101	[redacted] 706	AST	65 (U/L)	13	30	
RESTORE		Switch to OAV101	736	AST	36 (U/L)	13	30	
RESTORE		Switch to OAV101	739	AST	39 (U/L)	13	30	
RESTORE		Switch to OAV101	741	AST	36 (U/L)	13	30	
RESTORE		Switch to OAV101	776	AST	41 (U/L)	13	30	
RESTORE		Switch to OAV101	377	ALT	123 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	378	ALT	106 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	379	ALT	82 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	426	ALT	67 (U/L)	7	23	
RESTORE		Switch to OAV101	442	ALT	55 (U/L)	7	23	
RESTORE		Switch to OAV101	461	ALT	52 (U/L)	7	23	
RESTORE		Switch to OAV101	377	AST	218 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	378	AST	186 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	379	AST	139 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	426	AST	61 (U/L)	13	30	
RESTORE		Switch to OAV101	442	AST	53 (U/L)	13	30	
RESTORE		Switch to OAV101	461	AST	51 (U/L)	13	30	
RESTORE		OAV101 mono	/ 6	ALT	473 (U/L)	10	42	Yes
RESTORE		OAV101 mono	/ 7	ALT	314 (U/L)	10	42	Yes
RESTORE		OAV101 mono	/ 8	ALT	232 (U/L)	10	42	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	OAV101 mono	[redacted] 11	ALT	121 (U/L)	10	42	
RESTORE		OAV101 mono	/ 6	AST	524 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 7	AST	286 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 8	AST	184 (U/L)	13	30	Yes
RESTORE		OAV101 mono	11	AST	71 (U/L)	13	30	
RESTORE		Switch to OAV101	242	ALT	78 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	279	ALT	768 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	242	AST	152 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	275	AST	514 (U/L)	13	30	Yes
RESTORE		OAV101 mono	22	ALT	175 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	30	ALT	317 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	44	ALT	459 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	58	ALT	317 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	72	ALT	118 (IU/L)	5	25	Yes
RESTORE		OAV101 mono	86	ALT	50 (IU/L)	5	25	
RESTORE		OAV101 mono	100	ALT	41 (IU/L)	5	25	
RESTORE		OAV101 mono	30	AST	214 (IU/L)	15	50	Yes
RESTORE		OAV101 mono	44	AST	294 (IU/L)	15	50	Yes
RESTORE		OAV101 mono	58	AST	175 (IU/L)	15	50	Yes
RESTORE		OAV101 mono	72	AST	86 (IU/L)	15	50	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	OAV101 mono	[redacted] 86	AST	55 (IU/L)	15	50	
RESTORE		OAV101 mono	100	AST	57 (IU/L)	15	50	
RESTORE		Bridge to OAV101	112	ALT	247 (IU/L)	5	65	Yes
RESTORE		Bridge to OAV101	113	ALT	180 (IU/L)	5	65	
RESTORE		Bridge to OAV101	112	AST	427 (IU/L)	15	80	Yes
RESTORE		Bridge to OAV101	113	AST	242 (IU/L)	15	80	Yes
RESTORE		OAV101 mono	/ 6	ALT	1759 (IU/L)	9	38	Yes
RESTORE		OAV101 mono	/ 7	ALT	1218 (IU/L)	9	38	Yes
RESTORE		OAV101 mono	43	ALT	107 (IU/L)	9	38	
RESTORE		OAV101 mono	57	ALT	256 (IU/L)	9	38	Yes
RESTORE		OAV101 mono	/ 6	AST	2959 (IU/L)	23	57	Yes
RESTORE		OAV101 mono	/ 7	AST	1336 (IU/L)	23	57	Yes
RESTORE		OAV101 mono	43	AST	112 (IU/L)	23	57	
RESTORE		OAV101 mono	57	AST	207 (IU/L)	23	57	Yes
RESTORE		Bridge to OAV101	28	ALT	952 (IU/L)	0	40	Yes
RESTORE		Bridge to OAV101	29	ALT	960 (IU/L)	0	40	Yes
RESTORE		Bridge to OAV101	189	ALT	55 (IU/L)	0	40	
RESTORE		Bridge to OAV101	28	AST	1676 (IU/L)	0	37	Yes
RESTORE		Bridge to OAV101	161	AST	43 (IU/L)	0	37	

Note: Source specifies patients that were originally from US MAP, RESTORE.
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Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Bridge to OAV101	[redacted] 217	AST	52 (IU/L)	0	37	
RESTORE		OAV101 mono	/ 7	ALT	123 (IU/L)	0	40	Yes
RESTORE		OAV101 mono	19	ALT	64 (IU/L)	0	40	
RESTORE		OAV101 mono	28	ALT	78 (IU/L)	0	40	
RESTORE		OAV101 mono	/ 7	AST	190 (IU/L)	0	37	Yes
RESTORE		OAV101 mono	19	AST	75 (IU/L)	0	37	
RESTORE		Switch to OAV101	272	ALT	206 (IU/L)	7	23	Yes
RESTORE		Switch to OAV101	273	ALT	273 (IU/L)	7	23	Yes
RESTORE		Switch to OAV101	272	AST	455 (IU/L)	13	30	Yes
RESTORE		Switch to OAV101	273	AST	565 (IU/L)	13	30	Yes
RESTORE		Switch to OAV101	527	AST	62 (IU/L)	13	30	
RESTORE		Switch to OAV101	730	ALT	220 (U/L)	10	42	Yes
RESTORE		Switch to OAV101	679	AST	181 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	702	AST	61 (U/L)	13	30	
RESTORE		Switch to OAV101	730	AST	190 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	980	AST	65 (IU/L)	13	30	
RESTORE		OAV101 mono	/ 6	ALT	156 (U/L)	10	38	Yes
RESTORE		OAV101 mono	/ 6	AST	331 (U/L)	13	57	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Switch to OAV101	[redacted] 230	ALT	229 (IU/L)	9	38	Yes
RESTORE		Switch to OAV101	238	ALT	45 (IU/L)	9	38	
RESTORE		Switch to OAV101	396	ALT	31 (IU/L)	9	38	
RESTORE		Switch to OAV101	424	ALT	26 (IU/L)	9	38	
RESTORE		Switch to OAV101	1505	ALT	81 (IU/L)	9	38	
RESTORE		Switch to OAV101	230	AST	444 (IU/L)	24	57	Yes
RESTORE		Switch to OAV101	238	AST	53 (IU/L)	24	57	
RESTORE		Switch to OAV101	396	AST	44 (IU/L)	24	57	
RESTORE		Switch to OAV101	424	AST	41 (IU/L)	24	57	
RESTORE		Switch to OAV101	1505	AST	46 (IU/L)	24	57	
RESTORE		Combo w/OAV101	/ 19	ALT	414 (IU/L)	7	23	Yes
RESTORE		Combo w/OAV101	/ 21	ALT	284 (IU/L)	7	23	Yes
RESTORE		Combo w/OAV101	/ 26	ALT	65 (IU/L)	7	23	
RESTORE		Combo w/OAV101	624	ALT	70 (IU/L)	7	23	Yes
RESTORE		Combo w/OAV101	/ 19	AST	743 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101	/ 21	AST	279 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101	/ 26	AST	46 (IU/L)	13	30	
RESTORE		Combo w/OAV101	624	AST	50 (IU/L)	13	30	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Bridge to OAV101	[redacted] / 9	BILI	13.21 (mg/dL)	0.4	1.5	Yes
RESTORE		Switch to OAV101	546	ALT	117 (IU/L)	5	31	Yes
RESTORE		Switch to OAV101	499	RBC	437 (10E6/uL)	15	35	Yes
RESTORE		OAV101 mono	/ 6	ALT	513 (U/L)	10	30	Yes
RESTORE		OAV101 mono	/ 3	AST	111 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 6	AST	1085 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	254	ALT	274 (U/L)	10	30	Yes
RESTORE		Bridge to OAV101	268	ALT	379 (U/L)	10	30	Yes
RESTORE		Bridge to OAV101	282	ALT	165 (U/L)	10	30	Yes
RESTORE		Bridge to OAV101	268	AST	354 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	282	AST	142 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	224	ALT	132 (U/L)	10	30	Yes
RESTORE		Switch to OAV101	229	ALT	132 (U/L)	10	30	Yes
RESTORE		Switch to OAV101	256	ALT	886 (U/L)	10	30	Yes
RESTORE		Switch to OAV101	224	AST	232 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	229	AST	248 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	256	AST	1401 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Transient add-on	[redacted] / 4	AST	111 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	119	ALT	149 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	119	AST	243 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	141	AST	97 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	144	AST	123 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	231	ALT	81 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	268	ALT	32 (U/L)	7	23	
RESTORE		Bridge to OAV101	275	ALT	53 (U/L)	7	23	
RESTORE		Bridge to OAV101	231	AST	134 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	268	AST	51 (U/L)	13	30	
RESTORE		Bridge to OAV101	114	ALT	243 (IU/L)	4	43	Yes
RESTORE		Bridge to OAV101	114	AST	239 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 56	ALT	119 (U/L)	4	43	
RESTORE		Bridge to OAV101	/ 56	ALT	266 (U/L)	4	43	Yes
RESTORE		Bridge to OAV101	/ 59	ALT	259 (U/L)	4	43	Yes
RESTORE		Bridge to OAV101	/ 64	ALT	70 (U/L)	4	43	
RESTORE		Bridge to OAV101	/ 91	ALT	49 (U/L)	4	43	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Bridge to OAV101	[redacted] 56	AST	298 (U/L)	8	38	Yes
RESTORE		Bridge to OAV101	56	AST	700 (U/L)	8	38	Yes
RESTORE		Bridge to OAV101	59	AST	397 (U/L)	8	38	Yes
RESTORE		Bridge to OAV101	64	AST	61 (U/L)	8	38	
RESTORE		Bridge to OAV101	77	AST	50 (U/L)	8	38	
RESTORE		Bridge to OAV101	91	AST	93 (U/L)	8	38	
RESTORE		Combo w/OAV101	422	ALT	205 (U/L)	4	44	Yes
RESTORE		OAV101 mono	/ 1	ALP	389 (U/L)	38	113	Yes
RESTORE		OAV101 mono	/ 5	AST	111 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 6	AST	124 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 8	AST	71 (U/L)	13	30	
RESTORE		OAV101 mono	12	AST	51 (U/L)	13	30	
RESTORE		OAV101 mono	61	AST	44 (U/L)	13	30	
RESTORE		OAV101 mono	75	AST	63 (U/L)	13	30	
RESTORE		OAV101 mono	89	AST	57 (U/L)	13	30	
RESTORE		OAV101 mono	103	AST	65 (U/L)	13	30	
RESTORE		OAV101 mono	138	AST	64 (U/L)	13	30	
RESTORE		OAV101 mono	166	AST	72 (U/L)	13	30	
RESTORE		OAV101 mono	196	AST	51 (U/L)	13	30	
RESTORE		OAV101 mono	252	AST	54 (U/L)	13	30	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	OAV101 mono	[redacted] 326	AST	53 (U/L)	13	30	
RESTORE		OAV101 mono	390	AST	53 (U/L)	13	30	
RESTORE		OAV101 mono	446	AST	48 (U/L)	13	30	
RESTORE		OAV101 mono	509	AST	47 (U/L)	13	30	
RESTORE		OAV101 mono	580	AST	49 (U/L)	13	30	
RESTORE		OAV101 mono	642	AST	47 (U/L)	13	30	
RESTORE		OAV101 mono	691	AST	54 (U/L)	13	30	
RESTORE		Bridge to OAV101	187	ALT	130 (IU/L)	10	30	Yes
RESTORE		Bridge to OAV101	189	ALT	179 (IU/L)	10	30	Yes
RESTORE		Bridge to OAV101	187	AST	279 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	189	AST	295 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 5	ALT	491 (U/L)	7	23	Yes
RESTORE		OAV101 mono	/ 6	ALT	1067 (U/L)	7	23	Yes
RESTORE		OAV101 mono	27	ALT	115 (U/L)	7	23	Yes
RESTORE		OAV101 mono	34	ALT	480 (U/L)	7	23	Yes
RESTORE		OAV101 mono	/ 5	AST	781 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 6	AST	1678 (U/L)	13	30	Yes
RESTORE		OAV101 mono	27	AST	124 (U/L)	13	30	Yes
RESTORE		OAV101 mono	34	AST	257 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	OAV101 mono	[redacted] / 20	ALT	454 (IU/L)	9	53	Yes
RESTORE		OAV101 mono	/ 7	ALT	314 (IU/L)	7	53	Yes
RESTORE		OAV101 mono	/ 20	ALT	550 (IU/L)	7	53	Yes
RESTORE		OAV101 mono	132	ALT	68 (U/L)	7	53	
RESTORE		OAV101 mono	/ 7	AST	326 (IU/L)	17	70	Yes
RESTORE		OAV101 mono	/ 20	AST	298 (IU/L)	17	70	Yes
RESTORE		OAV101 mono	132	AST	70 (U/L)	17	70	
RESTORE		OAV101 mono	/ 14	ALT	94 (U/L)	7	23	Yes
RESTORE		OAV101 mono	/ 20	ALT	365 (U/L)	7	23	Yes
RESTORE		OAV101 mono	/ 26	ALT	464 (U/L)	7	23	Yes
RESTORE		OAV101 mono	/ 32	ALT	342 (U/L)	7	23	Yes
RESTORE		OAV101 mono	/ 39	ALT	167 (U/L)	7	23	Yes
RESTORE		OAV101 mono	/ 53	ALT	98 (U/L)	7	23	Yes
RESTORE		OAV101 mono	/ 69	ALT	45 (U/L)	7	23	
RESTORE		OAV101 mono	/ 83	ALT	29 (U/L)	7	23	
RESTORE		OAV101 mono	102	ALT	32 (U/L)	7	23	
RESTORE		OAV101 mono	123	ALT	49 (U/L)	7	23	
RESTORE		OAV101 mono	151	ALT	73 (U/L)	7	23	Yes
RESTORE		OAV101 mono	193	ALT	66 (U/L)	7	23	
RESTORE		OAV101 mono	/ 4	AST	100 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	OAV101 mono	[redacted] / 6	AST	157 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 9	AST	91 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 14	AST	152 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 20	AST	382 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 26	AST	398 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 32	AST	239 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 39	AST	107 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 53	AST	105 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 69	AST	59 (U/L)	13	30	
RESTORE		OAV101 mono	/ 83	AST	60 (U/L)	13	30	
RESTORE		OAV101 mono	102	AST	63 (U/L)	13	30	
RESTORE		OAV101 mono	123	AST	95 (U/L)	13	30	Yes
RESTORE		OAV101 mono	151	AST	101 (U/L)	13	30	Yes
RESTORE		OAV101 mono	193	AST	75 (U/L)	13	30	
RESTORE		Bridge to OAV101	/ 25	ALT	351 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	/ 29	ALT	110 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	/ 32	ALT	63 (U/L)	7	23	
RESTORE		Bridge to OAV101	/ 58	ALT	51 (U/L)	7	23	
RESTORE		Bridge to OAV101	/ 78	ALT	60 (U/L)	7	23	
RESTORE		Bridge to OAV101	/ 99	ALT	60 (U/L)	7	23	
RESTORE		Bridge to OAV101	134	ALT	100 (U/L)	7	23	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Bridge to OAV101	[redacted] 162	ALT	73 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	195	ALT	56 (U/L)	7	23	
RESTORE		Bridge to OAV101	/ 25	AST	282 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 29	AST	153 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 58	AST	67 (U/L)	13	30	
RESTORE		Bridge to OAV101	/ 78	AST	76 (U/L)	13	30	
RESTORE		Bridge to OAV101	/ 99	AST	75 (U/L)	13	30	
RESTORE		Bridge to OAV101	134	AST	133 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	162	AST	90 (U/L)	13	30	
RESTORE		Bridge to OAV101	195	AST	71 (U/L)	13	30	
RESTORE		Bridge to OAV101	/ 77	AST	113 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 79	AST	83 (U/L)	13	30	
RESTORE		Bridge to OAV101	100	AST	53 (U/L)	13	30	
RESTORE		Bridge to OAV101	107	AST	77 (U/L)	13	30	
RESTORE		Bridge to OAV101	114	AST	71 (U/L)	13	30	
RESTORE		Bridge to OAV101	121	AST	71 (U/L)	13	30	
RESTORE		Bridge to OAV101	163	AST	51 (U/L)	13	30	
RESTORE		Bridge to OAV101	184	AST	50 (U/L)	13	30	
RESTORE		Switch to OAV101	591	ALT	157 (U/L)	10	42	Yes
RESTORE		Switch to OAV101	589	AST	133 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Switch to OAV101	[redacted] / 591	AST	309 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 13	AST	136 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 15	AST	101 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 18	AST	81 (IU/L)	13	30	
RESTORE		Bridge to OAV101	/ 55	AST	119 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 76	AST	88 (IU/L)	13	30	
RESTORE		Combo w/OAV101	/ 3	BILI	8.4 (mg/dL)	0.11	0.66	Yes
RESTORE		OAV101 mono	/ 26	ALT	433 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 27	ALT	463 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 34	ALT	342 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 41	ALT	385 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 43	ALT	489 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 58	ALT	174 (U/L)	9	38	Yes
RESTORE		OAV101 mono	/ 84	ALT	48 (U/L)	9	38	
RESTORE		OAV101 mono	/ 26	AST	668 (U/L)	24	57	Yes
RESTORE		OAV101 mono	/ 27	AST	617 (U/L)	24	57	Yes
RESTORE		OAV101 mono	/ 34	AST	253 (U/L)	24	57	Yes
RESTORE		OAV101 mono	/ 41	AST	348 (U/L)	24	57	Yes
RESTORE		OAV101 mono	/ 43	AST	415 (U/L)	24	57	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	OAV101 mono	[redacted] 58	AST	132 (U/L)	24	57	
RESTORE		OAV101 mono	84	AST	76 (U/L)	24	57	
RESTORE		OAV101 mono	112	AST	67 (U/L)	24	57	
RESTORE		Bridge to OAV101	21	AST	101 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 7	ALT	134 (IU/L)	7	23	Yes
RESTORE		OAV101 mono	36	ALT	1016 (IU/L)	7	23	Yes
RESTORE		OAV101 mono	120	ALT	19 (IU/L)	7	23	
RESTORE		OAV101 mono	/ 7	AST	176 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	36	AST	584 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	120	AST	28 (IU/L)	13	30	
RESTORE		Switch to OAV101	275	AST	112 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	282	AST	57 (U/L)	13	30	
RESTORE		Switch to OAV101	289	AST	41 (U/L)	13	30	
RESTORE		Switch to OAV101	362	ALT	80 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	413	ALT	247 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	415	ALT	287 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	687	ALT	159 (U/L)	7	23	Yes
RESTORE		Switch to OAV101	413	AST	211 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Switch to OAV101	[redacted] 415	AST	228 (U/L)	13	30	Yes
RESTORE		Switch to OAV101	687	AST	138 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	140	ALT	458 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	140	AST	632 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	29	ALT	1057 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	88	ALT	65 (IU/L)	7	23	
RESTORE		Bridge to OAV101	29	AST	1377 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	88	AST	67 (IU/L)	13	30	
RESTORE		Bridge to OAV101	216	ALT	77 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	217	ALT	103 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	268	ALT	88 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	282	ALT	94 (U/L)	7	23	Yes
RESTORE		Bridge to OAV101	216	AST	184 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	217	AST	209 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	268	AST	82 (U/L)	13	30	
RESTORE		Bridge to OAV101	282	AST	109 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 6	AST	107 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 8	AST	57 (U/L)	13	30	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Bridge to OAV101	[redacted] / 46	ALT	158 (U/L)	8	42	Yes
RESTORE		Bridge to OAV101	/ 46	AST	140 (U/L)	13	33	Yes
RESTORE		Bridge to OAV101	224	AST	69 (U/L)	13	33	
RESTORE		Bridge to OAV101	410	AST	37 (U/L)	13	33	
RESTORE		Bridge to OAV101	/ 39	ALT	403 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 43	ALT	625 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 46	ALT	504 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 49	ALT	258 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 57	ALT	161 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 71	ALT	1710 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 78	ALT	1206 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 87	ALT	379 (IU/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 99	ALT	71 (IU/L)	4	44	
RESTORE		Bridge to OAV101	113	ALT	23 (IU/L)	4	44	
RESTORE		Bridge to OAV101	/ 35	AST	126 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 39	AST	633 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 43	AST	505 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 46	AST	251 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 49	AST	95 (IU/L)	8	38	
RESTORE		Bridge to OAV101	/ 57	AST	151 (IU/L)	8	38	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Bridge to OAV101	[redacted] / 71	AST	2160 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 78	AST	1112 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 87	AST	272 (IU/L)	8	38	Yes
RESTORE		Bridge to OAV101	/ 99	AST	60 (IU/L)	8	38	
RESTORE		Bridge to OAV101	113	AST	35 (IU/L)	8	38	
RESTORE		OAV101 mono	/ 6	ALT	140 (IU/L)	7	23	Yes
RESTORE		OAV101 mono	/ 6	AST	230 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101	348	AST	117 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101	363	AST	32 (IU/L)	13	30	
RESTORE		Bridge to OAV101	/ 15	AST	93 (U/L)	13	30	Yes
RESTORE		Bridge to OAV101	/ 21	AST	718 (U/L)	13	30	Yes
RESTORE		Add-on	/ 7	AST	152 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101	113	ALT	232 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101	116	ALT	570 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101	118	ALT	455 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101	120	ALT	266 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101	124	ALT	99 (IU/L)	8	42	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Combo w/OAV101	[redacted] / 145	ALT	117 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 159	ALT	130 (IU/L)	8	42	Yes
RESTORE		Combo w/OAV101	/ 173	ALT	54 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 189	ALT	43 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 208	ALT	57 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 224	ALT	104 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 238	ALT	119 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 252	ALT	94 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 280	ALT	54 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 308	ALT	51 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 336	ALT	48 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 364	ALT	49 (IU/L)	8	42	
RESTORE		Combo w/OAV101	/ 106	AST	185 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101	/ 113	AST	316 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101	/ 116	AST	545 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101	/ 118	AST	238 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101	/ 120	AST	96 (IU/L)	13	33	
RESTORE		Combo w/OAV101	/ 124	AST	40 (IU/L)	13	33	
RESTORE		Combo w/OAV101	/ 131	AST	38 (IU/L)	13	33	
RESTORE		Combo w/OAV101	/ 145	AST	122 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101	/ 159	AST	72 (IU/L)	13	33	
RESTORE		Combo w/OAV101	/ 173	AST	46 (IU/L)	13	33	
RESTORE		Combo w/OAV101	/ 189	AST	46 (IU/L)	13	33	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Combo w/OAV101	[redacted] 208	AST	66 (IU/L)	13	33	
RESTORE		Combo w/OAV101	224	AST	113 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101	238	AST	100 (IU/L)	13	33	Yes
RESTORE		Combo w/OAV101	252	AST	79 (IU/L)	13	33	
RESTORE		Combo w/OAV101	280	AST	49 (IU/L)	13	33	
RESTORE		Combo w/OAV101	308	AST	57 (IU/L)	13	33	
RESTORE		Combo w/OAV101	336	AST	51 (IU/L)	13	33	
RESTORE		Combo w/OAV101	364	AST	50 (IU/L)	13	33	
RESTORE		OAV101 mono	/ 6	ALT	521 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	/ 9	ALT	261 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	13	ALT	106 (IU/L)	10	42	
RESTORE		OAV101 mono	15	ALT	68 (IU/L)	10	42	
RESTORE		OAV101 mono	44	ALT	386 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	51	ALT	727 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	53	ALT	708 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	57	ALT	600 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	63	ALT	376 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	79	ALT	134 (IU/L)	10	42	Yes
RESTORE		OAV101 mono	93	ALT	89 (IU/L)	10	42	
RESTORE		OAV101 mono	121	ALT	56 (IU/L)	10	42	
RESTORE		OAV101 mono	132	ALT	50 (IU/L)	10	42	

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	OAV101 mono	[redacted] / 6	AST	890 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	/ 9	AST	351 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	13	AST	86 (IU/L)	13	30	
RESTORE		OAV101 mono	15	AST	60 (IU/L)	13	30	
RESTORE		OAV101 mono	20	AST	46 (IU/L)	13	30	
RESTORE		OAV101 mono	30	AST	52 (IU/L)	13	30	
RESTORE		OAV101 mono	44	AST	389 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	51	AST	681 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	53	AST	558 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	57	AST	478 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	63	AST	290 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	79	AST	126 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	93	AST	108 (IU/L)	13	30	Yes
RESTORE		OAV101 mono	121	AST	70 (IU/L)	13	30	
RESTORE		OAV101 mono	132	AST	74 (IU/L)	13	30	
RESTORE		Bridge to OAV101	25	AST	248 (IU/L)	13	30	Yes
RESTORE		Bridge to OAV101	20	BILD	1.5 (mg/dL)	0	0.3	Yes
RESTORE		Bridge to OAV101	20	BILI	7.7 (mg/dL)	0.1	1.2	Yes
RESTORE		OAV101 mono	48	ALT	1153 (IU/L)	12	51	Yes
RESTORE		OAV101 mono	48	AST	969 (IU/L)	25	67	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	OAV101 mono	[redacted] / 22	AST	151 (IU/L)	10	40	Yes
RESTORE		OAV101 mono	/ 79	AST	55 (IU/L)	10	40	
RESTORE		OAV101 mono	1123	WBC	13640 (10E3/uL)	3.5	9.7	Yes
RESTORE		Bridge to OAV101	/ 28	ALT	224 (U/L)	4	44	Yes
RESTORE		Bridge to OAV101	/ 28	AST	465 (U/L)	8	38	Yes
RESTORE		Bridge to OAV101	329	AST	63 (U/L)	8	38	
RESTORE		Bridge to OAV101	453	AST	58 (U/L)	8	38	
RESTORE		Bridge to OAV101	/ 44	ALT	240 (IU/L)	7	23	Yes
RESTORE		Bridge to OAV101	/ 58	ALT	335 (IU/L)	7	23	Yes
RESTORE		Bridge to OAV101	/ 44	AST	229 (IU/L)	13	30	Yes
RESTORE		Combo w/OAV101	/ 48	ALT	385 (U/L)	13	56	Yes
RESTORE		Combo w/OAV101	/ 48	AST	661 (U/L)	22	66	Yes
RESTORE		OAV101 mono	/ 71	ALT	121 (U/L)	7	23	Yes
RESTORE		OAV101 mono	/ 8	AST	101 (U/L)	13	30	Yes
RESTORE		OAV101 mono	/ 71	AST	130 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.2.jpn
Listing of Clinically Significant Laboratory Values
Japan OAV101 Treated Patients

Source	Patient	Therapy Group	Date/ Study Day	Test	Result (units)	Test Normal Low	Test Normal High	Result >3 x ULN
RESTORE	[redacted]	Switch to OAV101	[redacted] / 203	ALT	6128 (U/L)	10	42	Yes
RESTORE	[redacted]	Switch to OAV101	[redacted] / 157	AST	100 (U/L)	13	30	Yes
RESTORE	[redacted]	Switch to OAV101	[redacted] / 203	AST	24297 (U/L)	13	30	Yes

Note: Source specifies patients that were originally from US MAP, RESTORE.
Note: Study day is calculated relative to the date of the first treatment for SMA. Baseline is the last assessment within 45 days prior to the infusion of OAV101 or, if not available, the first assessment within the first 15 days after the OAV101 infusion.

Listing 16.4.jpn
Listing of Lack of Drug Effect
Japan OAV101 Treated Patients that Switched Treatment due to Lack of Drug Effect

Patient	Therapy Group	Primary Reason for Switching/Adding New Therapy	Lack of Drug Effect Specify
[redacted]	Add-on	Perceived lack of drug effect	Motor function
[redacted]	Combo w/OAV101	Perceived lack of drug effect	Swallowing or feeding ability for age
[redacted]	Combo w/OAV101	Perceived lack of drug effect	Motor function
[redacted]	Combo w/OAV101	Perceived lack of drug effect	
[redacted]	Combo w/OAV101	Perceived lack of drug effect	Respiratory function
[redacted]	Switch to OAV101	Perceived lack of drug effect	Swallowing or feeding ability for age
[redacted]	Transient add-on	Perceived lack of drug effect	

Listing 16.10.jpn
Listing of Lost to Follow-Up Patients
Japan OAV101 Treated Patients

Source	Patient	Age at time of consent /assent (months)	Therapy Group	SMN2 Gene Copy Number	SMA Type	Symptom Status	Patient Disposition	Primary Reason for Registry Discontinuation	Days/Age(months) at Last Data Point Captured
RESTORE	[redacted]	12	OAV101 mono	3 Copies	I	Symptomatic	Discontinued	Lost to Follow-up	89 / 14.9
RESTORE		30	Switch to OAV101	2 Copies	I	Symptomatic	Discontinued	Lost to Follow-up	1558 / 52.2
RESTORE		17	Switch to OAV101	2 Copies	I	Symptomatic	Discontinued	Lost to Follow-up	913 / 31.0
RESTORE		21	Switch to OAV101	2 Copies	I	Symptomatic	Discontinued	Lost to Follow-up	1446 / 52.5

Listing 16.11.jpn
Listing of Patients Excluded from the Japan Safety Analysis Set
All Japan Enrolled Patients

Patient	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
No patients qualify for this listing.					

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
[redacted]	Yes	Japanese	42.0	1	Yes	Add-on	[redacted]	[redacted]	[redacted]	[redacted]
	Yes	Japanese	55.5	1	Yes	Bridge to OAV101				
	Yes	Japanese	54.3	1	Yes	Bridge to OAV101				
	No	Japanese	45.1	5	Yes	Bridge to OAV101				
	Yes	Japanese	22.6	1	Yes	Bridge to OAV101				
	No	Japanese	50.2	5		Bridge to OAV101				
	Yes	Japanese	51.8	1	Yes	Bridge to OAV101				
	No	Taiwanese	26.7	1	Yes	Bridge to OAV101				
	No	Japanese	18.1	5		Bridge to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
[redacted]	No	Japanese	64.7	2	Yes	Bridge to OAV101	[redacted]			
	Yes	Japanese	35.5	1	Yes	Bridge to OAV101				
	No	Japanese	26.3	5	Yes	Bridge to OAV101				
	No	Japanese	23.5	5		Bridge to OAV101				
	No	Japanese	64.6	2	Yes	Bridge to OAV101				
	No	Japanese	28.8	5		Bridge to OAV101				
	Yes	Japanese	26.7	1	Yes	Bridge to OAV101				
	Yes	Japanese	51.6	1	Yes	Bridge to OAV101				
	Yes	Japanese	29.5	1	Yes	Bridge to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
[redacted]	Yes	Japanese	32.5	1	Yes	Bridge to OAV101	[redacted]			
	Yes	Japanese	28.3	1	Yes	Bridge to OAV101				
	No	Japanese	39.9	2	Yes	Bridge to OAV101				
	Yes	Japanese	51.7	1	Yes	Bridge to OAV101				
	Yes	Japanese	42.7	1	Yes	Bridge to OAV101				
	No	Japanese	64.4	2	Yes	Bridge to OAV101				
	Yes	Japanese	29.9	1	Yes	Bridge to OAV101				
	Yes	Japanese	41.5	1	Yes	Bridge to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
[redacted]	No	Japanese	43.7	2	Yes	Bridge to OAV101	[redacted]			
	No	Japanese	17.3	5		Bridge to OAV101				
	No	Japanese	20.7	5	Yes	Bridge to OAV101				
	No	Japanese	29.2	2	Yes	Bridge to OAV101				
	No	Japanese	50.1	2	Yes	Bridge to OAV101				
	Yes	Japanese	33.2	1	Yes	Combo w/OAV101				
	No	Japanese	18.8	5	Yes	Combo w/OAV101				
	Yes	Japanese	35.0	1	Yes	Combo w/OAV101				
	Yes	Japanese	49.4	1	Yes	Combo w/OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
[redacted]	Yes	Japanese	60.5	1	Yes	Combo w/OAV101	[redacted]			
	Yes	Japanese	45.5	1	Yes	Combo w/OAV101				
	No	Japanese	29.2	5	Yes	Combo w/OAV101				
	No	Japanese	44.4	2	Yes	OAV101 mono				
	No	Japanese	52.3	2	Yes	OAV101 mono				
	Yes	Japanese	65.2	1	Yes	OAV101 mono				
	Yes	Japanese	53.5	1	Yes	OAV101 mono				
	No	Vietnamese	22.3	5		OAV101 mono				
	No	Japanese	17.9	5		OAV101 mono				
	No	Japanese	18.5	5		OAV101 mono				
	Yes	Japanese	64.4	1	Yes	OAV101 mono				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
[redacted]	No	Indonesian	40.3	1	Yes	OAV101 mono	[redacted]			
	Yes	Japanese	52.0	1	Yes	OAV101 mono				
	No	Japanese	37.1	5		OAV101 mono				
	No	Japanese	57.8	2	Yes	OAV101 mono				
	No	Japanese	26.9	2	Yes	OAV101 mono				
	Yes	Japanese	50.6	1	Yes	OAV101 mono				
	Yes	Japanese	41.5	1	Yes	OAV101 mono				
	No	Japanese	50.8	2	Yes	OAV101 mono				
	Yes	Japanese	25.1	1	Yes	OAV101 mono				
	Yes	Japanese	59.4	1	Yes	OAV101 mono				
	No	Japanese	46.1	5	Yes	OAV101 mono				
	Yes	Japanese	32.7	1	Yes	OAV101 mono				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
[redacted]	No	Japanese	68.6	2	Yes	OAV101 mono	[redacted]			
	No	Japanese	52.5	2	Yes	OAV101 mono				
	Yes	Japanese	52.6	1	Yes	OAV101 mono				
	Yes	Japanese	44.8	1	Yes	OAV101 mono				
	No	Japanese	45.7	2	Yes	OAV101 mono				
	Yes	Japanese	62.9	1	Yes	Switch to OAV101				
	Yes	Japanese	68.4	1	Yes	Switch to OAV101				
	No	Chinese	47.5	1	Yes	Switch to OAV101				
	Yes	Japanese	69.9	1	Yes	Switch to OAV101				
	Yes	Japanese	71.4	1	Yes	Switch to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
[redacted]	No	Japanese	24.5	5		Switch to OAV101	[redacted]			
	Yes	Japanese	64.3	1	Yes	Switch to OAV101				
	Yes	Japanese	70.7	1	Yes	Switch to OAV101				
	Yes	Japanese	61.3	1	Yes	Switch to OAV101				
	Yes	Japanese	69.5	1	Yes	Switch to OAV101				
	Yes	Japanese	58.5	1	Yes	Switch to OAV101				
	Yes	Japanese	65.1	1	Yes	Switch to OAV101				
	Yes		56.6	1	Yes	Switch to OAV101				

Listing 16.13.jpn
Listing of Japan OAV101 Treated Patients
Japan OAV101 Treated Patients

Patient	HTA	Race	Age at Diagnosis (months)	SMA Type	Symptomatic at Diagnosis	Therapy Group	Start Date of OAV101	Start Date of NUSI	Start Date RISD	Date of Consent
[redacted]	No	Chinese	41.2	1	Yes	Switch to OAV101	[redacted]	[redacted]	[redacted]	[redacted]
	Yes	Japanese	58.8	1	Yes	Switch to OAV101				
	Yes	Japanese	22.8	1	Yes	Switch to OAV101				
	Yes	Japanese	48.3	1	Yes	Transient add-on				

Table 14.1.0.jpn
Analysis Datasets
All Japan Enrolled Patients

	Total (N=80)
Patients Enrolled at Japan Centers	80 (100)
OAV101 Treated Patients Excluded Patients	0
OAV101 Treated Patients	80 (100)
Safety Analysis Set Excluded Patients	0
Safety Analysis Set	80 (100)

Table 14.1.1.age3.jpn
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
All enrolled patients (Effectiveness Analysis Set)	14 (100)	9 (100)	21 (100)	32 (100)	4 (100)	80 (100)
Disposition						
Completed MAP	0	0	0	0	0	0
Completed Registry	0	0	0	0	0	0
Still enrolled	14 (100)	9 (100)	19 (90.5)	29 (90.6)	4 (100)	75 (93.8)
Early Termination	0	0	2 (9.5)	3 (9.4)	0	5 (6.3)
Death	0	0	1 (4.8)	0	0	1 (1.3)
Discontinued due to Adverse Event	0	0	0	0	0	0
Lost to Follow-up	0	0	1 (4.8)	3 (9.4)	0	4 (5.0)
Withdrawal of consent	0	0	0	0	0	0
Physician decision	0	0	0	0	0	0
Site is terminated by sponsor	0	0	0	0	0	0
Registry is terminated by sponsor	0	0	0	0	0	0

Percentages are based on all patients with known therapy course.
[a] Patient may have more than one response.

Table 14.1.1.age3.jpn
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
If death, Age at death (months) by Number of Copies of the SMN2 Gene mean (SD) [min, max]						
2 Copies	0	0	22.76 (-) [22.8, 22.8]	0	0	22.76 (-) [22.8, 22.8]
Treatment Courses						
OAV101 mono	6 (42.9)	1 (11.1)	4 (19.0)	13 (40.6)	1 (25.0)	25 (31.3)
Add-on	1 (7.1)	0	0	0	0	1 (1.3)
Transient add-on	1 (7.1)	0	0	0	0	1 (1.3)
Combo w/OAV101	2 (14.3)	3 (33.3)	1 (4.8)	1 (3.1)	0	7 (8.8)
Bridge to OAV101	4 (28.6)	5 (55.6)	13 (61.9)	7 (21.9)	1 (25.0)	30 (37.5)
Switch to OAV101	0	0	3 (14.3)	11 (34.4)	2 (50.0)	16 (20.0)
Nusi/risd combo	0	0	0	0	0	0
Risd mono	0	0	0	0	0	0
Nusi Mono	0	0	0	0	0	0
BSC	0	0	0	0	0	0
Treatment not yet established	0	0	0	0	0	0

Percentages are based on all patients with known therapy course.
[a] Patient may have more than one response.

Table 14.1.1.age3.jpn
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Ever exposed to OAV101	14 (100)	9 (100)	21 (100)	32 (100)	4 (100)	80 (100)
Ever exposed to NUSI	6 (42.9)	6 (66.7)	15 (71.4)	17 (53.1)	3 (75.0)	47 (58.8)
Ever exposed to RISD	4 (28.6)	4 (44.4)	3 (14.3)	3 (9.4)	0	14 (17.5)

Percentages are based on all patients with known therapy course.
[a] Patient may have more than one response.

Table 14.1.1.age3.jpn
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Did the patient receive SMA Treatment on or before the Informed Consent Date?						
Yes	14 (100)	9 (100)	21 (100)	29 (90.6)	4 (100)	77 (96.3)
No	0	0	0	3 (9.4)	0	3 (3.8)
Reason for no SMA treatment on or before the Informed Consent Date?[a]						
Waiting for insurance approval (US only)	0	0	0	0	0	0
Waiting for a new treatment option to be approved	0	0	0	0	0	0
Physician choice	0	0	0	1 (3.1)	0	1 (1.3)
Parent/Patient choice	0	0	0	1 (3.1)	0	1 (1.3)
Other	0	0	0	1 (3.1)	0	1 (1.3)

Percentages are based on all patients with known therapy course.
[a] Patient may have more than one response.

Table 14.1.1.age3.jpn
Patient Enrollment and Disposition by Age at OAV101 Infusion
Japan Analysis Set

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Patients with follow-up visit	14 (100)	9 (100)	21 (100)	32 (100)	4 (100)	80 (100)
Patients have been enrolled less than 1 year	2 (14.3)	0	2 (9.5)	0	0	4 (5.0)
Patients have been enrolled less than 1 year with Follow-up visit	2 (100)	0	2 (100)	0	0	4 (100)
Patients have been enrolled between 1-2 years	4 (28.6)	4 (44.4)	9 (42.9)	7 (21.9)	0	24 (30.0)
Patients have been enrolled between 1-2 years with Follow-up visit	4 (100)	4 (100)	9 (100)	7 (100)	0	24 (100)
Patients have been enrolled more than 2 years	8 (57.1)	5 (55.6)	10 (47.6)	25 (78.1)	4 (100)	52 (65.0)
Patients have been enrolled more than 2 years with Follow-up visit	8 (100)	5 (100)	10 (100)	25 (100)	4 (100)	52 (100)

Percentages are based on all patients with known therapy course.
[a] Patient may have more than one response.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Age at time of data cut (months)						
n	14	9	21	32	4	80
Mean	29.42	37.17	37.46	54.91	68.80	44.57
Std Dev	11.036	11.885	12.882	9.681	3.074	15.710
SE	2.950	3.962	2.811	1.711	1.537	1.756
Median	28.88	33.24	32.45	55.62	69.64	45.63
(Min, Max)	(17.3, 48.3)	(20.7, 51.8)	(22.6, 56.6)	(39.9, 69.9)	(64.6, 71.4)	(17.3, 71.4)
95% CI of the mean	(23.64, 35.20)	(29.40, 44.93)	(31.96, 42.97)	(51.55, 58.26)	(65.78, 71.81)	(41.12, 48.01)
0-6 months	0	0	0	0	0	0
>6-24 months	6 (42.9)	1 (11.1)	3 (14.3)	0	0	10 (12.5)
>24 months	8 (57.1)	8 (88.9)	18 (85.7)	32 (100)	4 (100)	70 (87.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a]Responses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.
[b]n restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Age at consent/assent into the registry (months)						
n	14	9	21	32	4	80
Mean	6.6	8.9	12.9	23.2	32.0	16.4
Std Dev	3.84	4.01	4.60	5.48	6.22	8.97
SE	1.03	1.34	1.00	0.97	3.11	1.00
Median	6.0	8.0	12.0	23.0	30.0	15.0
(Min, Max)	(2, 16)	(4, 17)	(7, 23)	(12, 37)	(27, 41)	(2, 41)
95% CI of the mean	(4.6, 8.7)	(6.3, 11.5)	(10.9, 14.8)	(21.3, 25.1)	(25.9, 38.1)	(14.5, 18.4)
0-6 months	7 (50.0)	3 (33.3)	0	0	0	10 (12.5)
>6-24 months	7 (50.0)	6 (66.7)	21 (100)	19 (59.4)	0	53 (66.3)
>24 months	0	0	0	13 (40.6)	4 (100)	17 (21.3)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a]Responses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.
[b]n restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Gestational age at birth (weeks) n (%)						
n	14	9	21	31	4	79
>35 weeks	14 (100)	9 (100)	21 (100)	29 (93.5)	4 (100)	77 (97.5)
33-35 weeks	0	0	0	1 (3.2)	0	1 (1.3)
30-32 weeks	0	0	0	0	0	0
28-29 weeks	0	0	0	0	0	0
<= 27 weeks	0	0	0	1 (3.2)	0	1 (1.3)
Gender n (%)						
n	14	9	21	32	4	80
Female	4 (28.6)	4 (44.4)	8 (38.1)	19 (59.4)	3 (75.0)	38 (47.5)
Male	10 (71.4)	5 (55.6)	13 (61.9)	13 (40.6)	1 (25.0)	42 (52.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a]Responses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.
[b]n restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Race n (%)						
n	14	9	21	32	4	80
American Indian or Alaska Native	0	0	0	0	0	0
Asian	14 (100)	9 (100)	19 (90.5)	32 (100)	4 (100)	78 (97.5)
Black or African American	0	0	0	0	0	0
Native Hawaiian or other Pacific Islander	0	0	0	0	0	0
White	0	0	0	0	0	0
Multiple	0	0	1 (4.8)	0	0	1 (1.3)
Not Reported[a]	0	0	1 (4.8)	0	0	1 (1.3)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a]Responses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.
[b]n restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Ethnicity n (%)						
n	14	9	21	32	4	80
Hispanic or Latino	0	0	0	0	0	0
Not Hispanic or Latino	14 (100)	9 (100)	20 (95.2)	32 (100)	4 (100)	79 (98.8)
Not Reported[a]	0	0	1 (4.8)	0	0	1 (1.3)
Primary caregiver at home n (%)						
n	1	2	9	8	2	22
Mother	0	2 (100)	7 (77.8)	6 (75.0)	1 (50.0)	16 (72.7)
Father	0	0	0	0	0	0
Mother and Father	1 (100)	0	2 (22.2)	2 (25.0)	1 (50.0)	6 (27.3)
Grandparent(s)	0	0	0	0	0	0
Other	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a]Responses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.
[b]n restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
[redacted]						

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a]Responses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.
[b]n restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
[redacted]						

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a]Responses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.
[b]n restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Age at start of Treatment n (%)						
n	14	9	21	32	4	80
< 6 months	14 (100)	9 (100)	10 (47.6)	5 (15.6)	1 (25.0)	39 (48.8)
=> 6 and < 12 months	0	0	11 (52.4)	6 (18.8)	1 (25.0)	18 (22.5)
=> 12 and < 24 months	0	0	0	21 (65.6)	1 (25.0)	22 (27.5)
=> 24 months	0	0	0	0	1 (25.0)	1 (1.3)
Age at start of Treatment (months)						
n	14	9	21	32	4	80
Mean	1.36	2.44	5.33	12.59	14.75	7.69
Std Dev	0.842	1.424	3.454	5.701	10.243	6.638
SE	0.225	0.475	0.754	1.008	5.121	0.742
Median	2.00	3.00	6.00	14.00	16.50	6.00
(Min, Max)	(0.0, 2.0)	(0.0, 5.0)	(1.0, 11.0)	(1.0, 22.0)	(2.0, 24.0)	(0.0, 24.0)
95% CI of the mean	(0.92, 1.80)	(1.51, 3.37)	(3.86, 6.81)	(10.62, 14.57)	(4.71, 24.79)	(6.23, 9.14)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a]Responses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.
[b]n restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.2.age3.jpn
Patient Demographics by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Weight at start of OAV101 Treatment n (%)						
n	13	8	21	30	3	75
< 8.5 kg	13 (100)	8 (100)	16 (76.2)	17 (56.7)	1 (33.3)	55 (73.3)
>= 8.5-13.5 kg	0	0	5 (23.8)	13 (43.3)	2 (66.7)	20 (26.7)
>= 13.5-21 kg	0	0	0	0	0	0
>= 21 kg	0	0	0	0	0	0
Weight at start of OAV101 Treatment (kg)						
n	13	8	21	30	3	75
Mean	4.35	5.11	7.78	8.38	8.73	7.18
Std Dev	1.110	1.169	1.034	1.465	1.250	2.037
SE	0.308	0.413	0.226	0.267	0.722	0.235
Median	4.10	5.30	7.50	8.20	9.30	7.40
(Min, Max)	(2.6, 6.4)	(3.1, 6.6)	(5.6, 10.1)	(5.5, 11.2)	(7.3, 9.6)	(2.6, 11.2)
95% CI of the mean	(3.75, 4.96)	(4.30, 5.92)	(7.34, 8.22)	(7.85, 8.90)	(7.32, 10.15)	(6.72, 7.64)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a]Responses of "Not Reported" may include cases where information was either never requested from the patient or requested but not received, or if the patient marked 'prefer not to answer', etc.
[b]n restricted to number with insurance and is used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Was the patient screened for SMA as a new-born n (%)						
n	14	9	21	32	4	80
Yes	5 (35.7)	3 (33.3)	2 (9.5)	0	0	10 (12.5)
No	9 (64.3)	6 (66.7)	19 (90.5)	32 (100)	4 (100)	70 (87.5)
Did the patient display symptoms at the time of diagnosis n (%)						
n	14	9	21	32	4	80
Yes	8 (57.1)	7 (77.8)	19 (90.5)	32 (100)	4 (100)	70 (87.5)
No	6 (42.9)	2 (22.2)	2 (9.5)	0	0	10 (12.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
SMA symptoms at diagnosis. Did the patient display symptoms at the time of diagnosis[a] n (%)						
n[b]	8	7	19	32	4	70
Hypotonia	8 (100)	7 (100)	18 (94.7)	32 (100)	4 (100)	69 (98.6)
Limb weakness	8 (100)	5 (71.4)	19 (100)	28 (87.5)	4 (100)	64 (91.4)
Pneumonia or respiratory symptoms	4 (50.0)	4 (57.1)	5 (26.3)	6 (18.8)	1 (25.0)	20 (28.6)
Tongue fasciculations	3 (37.5)	5 (71.4)	14 (73.7)	16 (50.0)	0	38 (54.3)
Developmental delay	2 (25.0)	2 (28.6)	11 (57.9)	22 (68.8)	2 (50.0)	39 (55.7)
Constipation	1 (12.5)	1 (14.3)	2 (10.5)	5 (15.6)	0	9 (12.9)
Swallowing or feeding difficulties	4 (50.0)	3 (42.9)	6 (31.6)	7 (21.9)	1 (25.0)	21 (30.0)
Other	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Age at first symptoms onset (months)[b]						
n	9	7	19	32	4	71
Mean	0.2	0.7	3.6	5.9	7.8	4.2
Std Dev	0.44	0.76	2.85	4.16	7.59	4.21
SE	0.15	0.29	0.65	0.74	3.79	0.50
Median	0.0	1.0	4.0	5.5	6.5	3.0
(Min, Max)	(0, 1)	(0, 2)	(0, 9)	(0, 15)	(0, 18)	(0, 18)
95% CI of the mean	(-0.1, 0.5)	(0.2, 1.3)	(2.3, 4.9)	(4.5, 7.3)	(0.3, 15.2)	(3.2, 5.1)
< 6 months	9 (100)	7 (100)	13 (68.4)	16 (50.0)	2 (50.0)	47 (66.2)
=> 6 and < 12 months	0	0	6 (31.6)	13 (40.6)	1 (25.0)	20 (28.2)
=> 12 and < 24 months	0	0	0	3 (9.4)	1 (25.0)	4 (5.6)
=> 24 months	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Genetically confirmed SMA diagnosis prior to birth n (%)						
n	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Age at genetically confirmed SMA diagnosis (post-partum months)						
n	14	9	21	31	4	79
Mean	0.9	2.3	5.3	11.5	14.3	7.1
Std Dev	0.73	1.41	3.44	5.44	10.37	6.30
SE	0.20	0.47	0.75	0.98	5.19	0.71
Median	1.0	2.0	6.0	13.0	16.5	6.0
(Min, Max)	(0, 2)	(0, 5)	(0, 10)	(1, 24)	(1, 23)	(0, 24)
95% CI of the mean	(0.5, 1.3)	(1.4, 3.3)	(3.8, 6.8)	(9.6, 13.5)	(4.1, 24.4)	(5.7, 8.5)
< 6 months	14 (100)	9 (100)	9 (42.9)	6 (19.4)	1 (25.0)	39 (49.4)
=> 6 and < 12 months	0	0	12 (57.1)	6 (19.4)	1 (25.0)	19 (24.1)
=> 12 and < 24 months	0	0	0	18 (58.1)	2 (50.0)	20 (25.3)
=> 24 months	0	0	0	1 (3.2)	0	1 (1.3)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Has the Number of Copies of the SMN2 Gene been determined n (%)						
n	14	9	21	32	4	80
Yes	14 (100)	9 (100)	21 (100)	32 (100)	4 (100)	80 (100)
No	0	0	0	0	0	0
Number of Copies of the SMN2 Gene n (%)						
n	14	9	21	32	4	80
1 Copy	0	0	0	0	0	0
2 Copies	10 (71.4)	6 (66.7)	11 (52.4)	12 (37.5)	1 (25.0)	40 (50.0)
3 Copies	4 (28.6)	3 (33.3)	10 (47.6)	20 (62.5)	3 (75.0)	40 (50.0)
4 Copies	0	0	0	0	0	0
>4 Copies	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Genetic testing for SMA n (%)						
n	14	9	21	32	4	80
SMN1 homozygous deletion of exon 7 (or 7&8)	14 (100)	8 (88.9)	21 (100)	30 (93.8)	4 (100)	77 (96.3)
SMN1 heterozygous deletion of exon 7/8 and subtle mutation (intragenic deletion or duplication or point mutation)	0	1 (11.1)	0	2 (6.3)	0	3 (3.8)
Other SMN1 result	0	0	0	0	0	0
Patient has the c.859G>C SMN2 variant n (%)						
n	14	9	21	32	4	80
Yes	0	0	0	0	0	0
No	0	3 (33.3)	5 (23.8)	4 (12.5)	0	12 (15.0)
Not Tested	14 (100)	6 (66.7)	16 (76.2)	28 (87.5)	4 (100)	68 (85.0)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
SMA Type n (%)						
n[b]	6	4	19	31	4	64
0	0	0	0	0	0	0
I	6 (100)	4 (100)	16 (84.2)	21 (67.7)	2 (50.0)	49 (76.6)
II	0	0	3 (15.8)	10 (32.3)	2 (50.0)	15 (23.4)
III	0	0	0	0	0	0
IV	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
SMA function status by age at first dose n (%)						
n	9	6	20	31	4	70
Non-sitter	9 (100)	6 (100)	20 (100)	22 (71.0)	2 (50.0)	59 (84.3)
< 6 months[d]	9 (100)	6 (100)	9 (45.0)	5 (22.7)	1 (50.0)	30 (50.8)
=> 6 and < 12 months[d]	0	0	11 (55.0)	6 (27.3)	1 (50.0)	18 (30.5)
=> 12 and < 24 months[d]	0	0	0	11 (50.0)	0	11 (18.6)
=> 24 months[d]	0	0	0	0	0	0
Sitter	0	0	0	9 (29.0)	1 (25.0)	10 (14.3)
< 6 months[d]	0	0	0	0	0	0
=> 6 and < 12 months[d]	0	0	0	0	0	0
=> 12 and < 24 months[d]	0	0	0	9 (100)	1 (100)	10 (100)
=> 24 months[d]	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Standing	0	0	0	0	1 (25.0)	1 (1.4)
< 6 months[d]	0	0	0	0	0	0
=> 6 and < 12 months[d]	0	0	0	0	0	0
=> 12 and < 24 months[d]	0	0	0	0	0	0
=> 24 months[d]	0	0	0	0	1 (100)	1 (100)
Walker	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Does the patient have an immediate familial history of SMA? n (%)						
n	13	9	21	32	4	79
Yes	5 (38.5)	1 (11.1)	3 (14.3)	2 (6.3)	1 (25.0)	12 (15.2)
No	8 (61.5)	8 (88.9)	18 (85.7)	30 (93.8)	3 (75.0)	67 (84.8)
Relative(s) with history of SMA[a] n (%)						
n[c]	5	1	3	2	1	12
Biological Mother is a known carrier	2 (40.0)	0	0	0	0	2 (16.7)
Biological Father is a known carrier	2 (40.0)	0	0	0	0	2 (16.7)
Biological Sibling(s)	4 (80.0)	1 (100)	2 (66.7)	0	0	7 (58.3)
Other	1 (20.0)	0	1 (33.3)	2 (100)	1 (100)	5 (41.7)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Weight at diagnosis of SMA (kg)						
n	13	8	21	27	4	73
Mean	3.96	4.04	6.34	7.52	7.55	6.17
Std Dev	0.974	1.003	2.095	1.553	2.511	2.190
SE	0.270	0.355	0.457	0.299	1.255	0.256
Median	3.60	3.95	7.10	7.80	8.30	6.00
(Min, Max)	(2.7, 6.0)	(2.7, 5.4)	(3.1, 9.7)	(4.6, 10.0)	(4.0, 9.6)	(2.7, 10.0)
95% CI of the mean	(3.43, 4.49)	(3.34, 4.73)	(5.45, 7.24)	(6.94, 8.11)	(5.09, 10.01)	(5.67, 6.67)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.3.age3.jpn
SMA Medical History by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Length/Height at diagnosis of SMA (cm)						
n	13	8	21	27	4	73
Mean	53.22	54.61	63.47	69.17	71.00	63.19
Std Dev	4.039	5.450	7.359	6.740	10.116	9.181
SE	1.120	1.927	1.606	1.297	5.058	1.075
Median	53.30	55.60	65.30	69.00	74.35	62.50
(Min, Max)	(47.3, 58.5)	(47.0, 61.2)	(51.4, 76.4)	(56.7, 80.0)	(56.8, 78.5)	(47.0, 80.0)
95% CI of the mean	(51.02, 55.41)	(50.84, 58.39)	(60.32, 66.61)	(66.63, 71.72)	(61.09, 80.91)	(61.09, 65.30)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] patient may have more than one response.
[b] includes only patients with symptoms.
[c] includes only patients with an immediate familial history of SMA.
[d] Number in functional status used as the denominator for calculating percentages.

Table 14.1.6.age3.jpn
OAV101 Treatment by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Time of therapy relative to SMA diagnosis (months)						
n	14	9	21	32	4	80
Mean	0.57	1.59	3.42	6.41	9.82	4.23
Std Dev	0.159	1.275	2.823	6.406	10.061	5.384
SE	0.042	0.425	0.616	1.132	5.031	0.602
Median	0.54	1.25	2.56	1.84	7.67	1.38
(Min, Max)	(0.4, 0.9)	(0.4, 3.8)	(0.3, 9.0)	(0.6, 19.3)	(1.1, 22.8)	(0.3, 22.8)
95% CI of the mean	(0.49, 0.65)	(0.76, 2.42)	(2.21, 4.62)	(4.19, 8.63)	(-0.04, 19.68)	(3.05, 5.41)
Time from diagnosis to treatment n(%)						
n	14	9	21	32	4	80
0-6 months	14 (100)	9 (100)	17 (81.0)	18 (56.3)	2 (50.0)	60 (75.0)
> 6-24 months	0	0	4 (19.0)	14 (43.8)	2 (50.0)	20 (25.0)
> 24 months	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.age3.jpn
OAV101 Treatment by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Age at OAV101 infusion (months)						
n	14	9	21	32	4	80
Mean	1.6	3.8	8.4	17.6	24.0	11.1
Std Dev	0.65	0.83	1.43	3.45	0.00	7.42
SE	0.17	0.28	0.31	0.61	0.00	0.83
Median	2.0	4.0	8.0	17.0	24.0	10.0
(Min, Max)	(0, 2)	(3, 5)	(6, 11)	(12, 23)	(24, 24)	(0, 24)
95% CI of the mean	(1.2, 1.9)	(3.2, 4.3)	(7.8, 9.0)	(16.4, 18.8)	(24.0, 24.0)	(9.5, 12.8)
Age at OAV101 infusion (categorical)						
< 6 months	14 (100)	9 (100)	0	0	0	23 (28.8)
≥ 6 and < 12 months	0	0	21 (100)	0	0	21 (26.3)
≥ 12 and < 24 months	0	0	0	32 (100)	0	32 (40.0)
≥ 24 months	0	0	0	0	4 (100)	4 (5.0)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.age3.jpn
OAV101 Treatment by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Primary reason for switching to OAV101? n(%)						
n	6	8	17	18	3	52
Perceived lack of drug effect	1 (16.7)	0	1 (5.9)	0	0	2 (3.8)
Motor function	1 (16.7)	0	0	0	0	1 (1.9)
Respiratory function	0	0	0	0	0	0
Swallowing or feeding ability for age	0	0	1 (5.9)	0	0	1 (1.9)
Electrophysiological/another biomarker response	0	0	0	0	0	0
No additional response chosen	0	0	0	0	0	0
Adverse Events	0	0	0	0	0	0
Parent/Caregiver/Patient decision	4 (66.7)	6 (75.0)	13 (76.5)	14 (77.8)	2 (66.7)	39 (75.0)
Alternative Treatment Available and Reimbursed	1 (16.7)	2 (25.0)	3 (17.6)	4 (22.2)	1 (33.3)	11 (21.2)
Other	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.age3.jpn
OAV101 Treatment by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Duration since OAV101 infusion (months)						
n	14	9	21	32	4	80
Mean	27.94	33.29	27.94	36.37	45.08	32.77
Std Dev	10.820	11.696	12.531	9.761	3.306	11.575
SE	2.892	3.899	2.734	1.725	1.653	1.294
Median	26.91	30.62	23.26	38.32	45.98	34.68
(Min, Max)	(16.3, 46.5)	(17.5, 47.6)	(13.6, 47.4)	(12.6, 47.5)	(40.5, 47.9)	(12.6, 47.9)
95% CI of the mean	(22.27, 33.60)	(25.65, 40.93)	(22.58, 33.30)	(32.99, 39.76)	(41.84, 48.32)	(30.23, 35.31)
OAV101 Exposure (person-year)	32.6	25.0	48.9	97.0	15.0	218.5

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.g5tx.jpn
OAV101 Treatment by Therapy at OAV101 Infusion
Japan OAV101 Treated Patients

	OAV101 mono (N=25)	Add-on (N=1)	Transient add-on (N=1)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=30)	Switch to OAV101 (N=16)	Total OAV101 (N=80)
Time of therapy relative to SMA diagnosis (months)							
n	25	1	1	7	30	16	80
Mean	0.91	0.53	0.49	2.84	3.02	12.77	4.23
Std Dev	0.387	-	-	3.919	3.045	5.154	5.384
SE	0.077	-	-	1.481	0.556	1.288	0.602
Median	0.79	0.53	0.49	1.48	1.63	12.47	1.38
(Min, Max)	(0.3, 1.9)	(0.5, 0.5)	(0.5, 0.5)	(0.4, 11.4)	(0.4, 12.7)	(5.0, 22.8)	(0.3, 22.8)
95% CI of the mean	(0.76, 1.06)	(-, -)	(-, -)	(-0.06, 5.74)	(1.93, 4.11)	(10.24, 15.29)	(3.05, 5.41)
Time from diagnosis to treatment n(%)							
n	25	1	1	7	30	16	80
0-6 months	25 (100)	1 (100)	1 (100)	6 (85.7)	25 (83.3)	2 (12.5)	60 (75.0)
> 6-24 months	0	0	0	1 (14.3)	5 (16.7)	14 (87.5)	20 (25.0)
> 24 months	0	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.g5tx.jpn
OAV101 Treatment by Therapy at OAV101 Infusion
Japan OAV101 Treated Patients

	OAV101 mono (N=25)	Add-on (N=1)	Transient add-on (N=1)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=30)	Switch to OAV101 (N=16)	Total OAV101 (N=80)
Age at OAV101 infusion (months)							
n	25	1	1	7	30	16	80
Mean	11.3	2.0	2.0	5.6	9.4	17.8	11.1
Std Dev	6.88	-	-	5.06	6.90	5.67	7.42
SE	1.38	-	-	1.91	1.26	1.42	0.83
Median	13.0	2.0	2.0	4.0	7.0	18.0	10.0
(Min, Max)	(1, 24)	(2, 2)	(2, 2)	(1, 15)	(0, 24)	(7, 24)	(0, 24)
95% CI of the mean	(8.6, 14.0)	(-, -)	(-, -)	(1.8, 9.3)	(6.9, 11.8)	(15.0, 20.6)	(9.5, 12.8)
Age at OAV101 infusion (categorical)							
< 6 months	7 (28.0)	1 (100)	1 (100)	5 (71.4)	9 (30.0)	0	23 (28.8)
≥ 6 and < 12 months	4 (16.0)	0	0	1 (14.3)	13 (43.3)	3 (18.8)	21 (26.3)
≥ 12 and < 24 months	13 (52.0)	0	0	1 (14.3)	7 (23.3)	11 (68.8)	32 (40.0)
≥ 24 months	1 (4.0)	0	0	0	1 (3.3)	2 (12.5)	4 (5.0)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.g5tx.jpn
OAV101 Treatment by Therapy at OAV101 Infusion
Japan OAV101 Treated Patients

	OAV101 mono (N=25)	Add-on (N=1)	Transient add-on (N=1)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=30)	Switch to OAV101 (N=16)	Total OAV101 (N=80)
Primary reason for switching to OAV101? n(%)							
n	0	0	0	7	29	16	52
Perceived lack of drug effect				1 (14.3)	0	1 (6.3)	2 (3.8)
Motor function				1 (14.3)	0	0	1 (1.9)
Respiratory function				0	0	0	0
Swallowing or feeding ability for age				0	0	1 (6.3)	1 (1.9)
Electrophysiological/another biomarker response				0	0	0	0
No additional response chosen				0	0	0	0
Adverse Events				0	0	0	0
Parent/Caregiver/Patient decision				4 (57.1)	23 (79.3)	12 (75.0)	39 (75.0)
Alternative Treatment Available and Reimbursed				2 (28.6)	6 (20.7)	3 (18.8)	11 (21.2)
Other				0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.6.g5tx.jpn
OAV101 Treatment by Therapy at OAV101 Infusion
Japan OAV101 Treated Patients

	OAV101 mono (N=25)	Add-on (N=1)	Transient add-on (N=1)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=30)	Switch to OAV101 (N=16)	Total OAV101 (N=80)
Duration since OAV101 infusion (months)							
n	25	1	1	7	30	16	80
Mean	33.05	39.82	46.52	33.18	29.51	36.96	32.77
Std Dev	11.166	-	-	10.248	11.614	12.137	11.575
SE	2.233	-	-	3.873	2.120	3.034	1.294
Median	36.34	39.82	46.52	34.27	26.60	44.90	34.68
(Min, Max)	(12.6, 47.5)	(39.8, 39.8)	(46.5, 46.5)	(16.8, 45.1)	(13.6, 47.6)	(15.8, 47.9)	(12.6, 47.9)
95% CI of the mean	(28.68, 37.43)	(-, -)	(-, -)	(25.59, 40.77)	(25.35, 33.67)	(31.02, 42.91)	(30.23, 35.31)
OAV101 Exposure (person-year)	68.9	3.3	3.9	19.4	73.8	49.3	218.5

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.7.cpy.jpn
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Time of therapy relative to SMA diagnosis (months)						
n	0	28	19	0	0	47
Mean		0.72	1.64			1.09
Std Dev		2.604	3.794			3.133
SE		0.492	0.870			0.457
Median		0.26	0.33			0.26
(Min, Max)		(-2.4, 13.6)	(-0.3, 12.5)			(-2.4, 13.6)
95% CI of the mean		(-0.24, 1.69)	(-0.07, 3.34)			(0.20, 1.99)
Time from diagnosis to treatment						
0-6 months n (%)	0	26 (92.9)	16 (84.2)	0	0	42 (89.4)
>6-24 months n (%)	0	1 (3.6)	2 (10.5)	0	0	3 (6.4)
>24 months n (%)	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.7.cpy.jpn
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Age at first dose (months)						
n	0	28	19	0	0	47
Mean		3.9	9.8			6.3
Std Dev		3.67	6.93			5.95
SE		0.69	1.59			0.87
Median		3.0	9.0			5.0
(Min, Max)		(0, 15)	(0, 22)			(0, 22)
95% CI of the mean		(2.5, 5.2)	(6.7, 12.9)			(4.6, 8.0)
Age at first dose (categorical)	0	28	19	0	0	47
< 6 months n (%)	0	21 (75.0)	5 (26.3)	0	0	26 (55.3)
=> 6 and < 12 months n (%)	0	5 (17.9)	8 (42.1)	0	0	13 (27.7)
=> 12 and < 24 months n (%)	0	2 (7.1)	6 (31.6)	0	0	8 (17.0)
=> 24 months n (%)	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.7.cpy.jpn
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Duration of therapy (months)						
n	0	28	19	0	0	47
Mean		6.18	4.48			5.49
Std Dev		7.305	6.558			6.990
SE		1.380	1.505			1.020
Median		2.14	2.07			2.14
(Min, Max)		(0.4, 30.1)	(0.4, 26.7)			(0.4, 30.1)
95% CI of the mean		(3.47, 8.88)	(1.53, 7.43)			(3.49, 7.49)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.7.cpy.jpn
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Has the patient received OAV101, Risdiplam, or other SMA treatment and then added/switched to Nusinersen? n (%)						
n	0	28	17	0	0	45
Yes	0	1 (3.6)	3 (17.6)	0	0	4 (8.9)
No	0	27 (96.4)	14 (82.4)	0	0	41 (91.1)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.7.cpy.jpn
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Primary reason for adding/switching to Nusinersen and reason for discontinuing SMA Treatment (if applicable)[a]? n (%)						
n	0	1	3	0	0	4
Perceived lack of drug effect	0	1 (100)	0	0	0	1 (25.0)
Motor function	0	1 (100)	0	0	0	1 (25.0)
Respiratory function	0	0	0	0	0	0
Swallowing or feeding ability for age	0	0	0	0	0	0
Electrophysiological/another biomarker response	0	0	0	0	0	0
No additional response chosen	0	0	0	0	0	0
Adverse event	0	0	0	0	0	0
Parent/Caregiver/Patient decision	0	0	2 (66.7)	0	0	2 (50.0)
Alternative Treatment Available and Reimbursed	0	0	1 (33.3)	0	0	1 (25.0)
Irregular compliance	0	0	0	0	0	0
Other	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.7.cpy.jpn
Nusinersen Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=28)	3 Copies (N=19)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=47)
Nusinersen Exposure (person-year)	-	14.4	7.1	-	-	21.5

Note: 'n' specified for each variable is used as the denominator for calculating percentages.

Table 14.1.7.txn.jpn
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=6)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=15)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Time of therapy relative to SMA diagnosis (months)								
n	0	1	6	25	15	0	0	47
Mean		13.63	0.24	0.74	1.19			1.09
Std Dev		-	0.183	2.405	3.245			3.133
SE		-	0.075	0.481	0.838			0.457
Median		13.63	0.21	0.16	0.33			0.26
(Min, Max)		(13.6, 13.6)	(0.1, 0.5)	(-0.3, 12.2)	(-2.4, 12.5)			(-2.4, 13.6)
95% CI of the mean		(-, -)	(0.09, 0.38)	(-0.21, 1.68)	(-0.45, 2.83)			(0.20, 1.99)
Time from diagnosis to treatment								
0-6 months n (%)	0	0	6 (100)	23 (92.0)	13 (86.7)	0	0	42 (89.4)
>6-24 months n (%)	0	1 (100)	0	1 (4.0)	1 (6.7)	0	0	3 (6.4)
>24 months n (%)	0	0	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.txn.jpn
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=6)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=15)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Age at first dose (months)								
n	0	1	6	25	15	0	0	47
Mean		15.0	3.0	6.7	6.3			6.3
Std Dev		-	2.37	6.84	4.88			5.95
SE		-	0.97	1.37	1.26			0.87
Median		15.0	2.5	3.0	6.0			5.0
(Min, Max)		(15, 15)	(0, 7)	(0, 22)	(1, 20)			(0, 22)
95% CI of the mean		(-, -)	(1.1, 4.9)	(4.0, 9.4)	(3.8, 8.7)			(4.6, 8.0)
Age at first dose (categorical)	0	1	6	25	15	0	0	47
< 6 months n (%)	0	0	5 (83.3)	14 (56.0)	7 (46.7)	0	0	26 (55.3)
=> 6 and < 12 months n (%)	0	0	1 (16.7)	5 (20.0)	7 (46.7)	0	0	13 (27.7)
=> 12 and < 24 months n (%)	0	1 (100)	0	6 (24.0)	1 (6.7)	0	0	8 (17.0)
=> 24 months n (%)	0	0	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.txn.jpn
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=6)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=15)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Duration of therapy (months)								
n	0	1	6	25	15	0	0	47
Mean		30.09	2.37	2.82	9.55			5.49
Std Dev		-	3.736	5.666	4.771			6.990
SE		-	1.525	1.133	1.232			1.020
Median		30.09	0.69	0.92	6.21			2.14
(Min, Max)		(30.1, 30.1)	(0.4, 9.9)	(0.4, 26.7)	(5.3, 19.2)			(0.4, 30.1)
95% CI of the mean		(-, -)	(-0.62, 5.36)	(0.60, 5.04)	(7.13, 11.96)			(3.49, 7.49)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.txn.jpn
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=6)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=15)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Has the patient received OAV101, Risdiplam, or other SMA treatment and then added/switched to Nusinersen? n (%)								
n	0	1	6	24	14	0	0	45
Yes	0	1 (100)	0	3 (12.5)	0	0	0	4 (8.9)
No	0	0	6 (100)	21 (87.5)	14 (100)	0	0	41 (91.1)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.txn.jpn
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=6)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=15)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Primary reason for adding/switching to Nusinersen and reason for discontinuing SMA Treatment (if applicable)[a]? n (%)								
n	0	1	0	3	0	0	0	4
Perceived lack of drug effect	0	1 (100)	0	0	0	0	0	1 (25.0)
Motor function	0	1 (100)	0	0	0	0	0	1 (25.0)
Respiratory function	0	0	0	0	0	0	0	0
Swallowing or feeding ability for age	0	0	0	0	0	0	0	0
Electrophysiological/another biomarker response	0	0	0	0	0	0	0	0
No additional response chosen	0	0	0	0	0	0	0	0
Adverse event	0	0	0	0	0	0	0	0
Parent/Caregiver/Patient decision	0	0	0	2 (66.7)	0	0	0	2 (50.0)
Alternative Treatment Available and Reimbursed	0	0	0	1 (33.3)	0	0	0	1 (25.0)
Irregular compliance	0	0	0	0	0	0	0	0
Other	0	0	0	0	0	0	0	0

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.7.txn.jpn
Nusinersen Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=0)	Transient add-on (N=1)	Combo w/OAV101 (N=6)	Bridge to OAV101 (N=25)	Switch to OAV101 (N=15)	Nusi/risd combo (N=0)	Nusi Mono (N=0)	Total (N=47)
Nusinersen Exposure (person-year)	-	2.5	1.2	5.9	11.9	-	-	21.5

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percent.

Table 14.1.8.age3.jpn
AAV9 Antibody Testing Results by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Patients with ≥ 1 AAV9 tests meeting eligibility criteria[a] n(%)						
n	14	9	21	32	4	80
Patient with only 1 test reported	14 (100)	8 (88.9)	20 (95.2)	32 (100)	4 (100)	78 (97.5)
Patient with 2 or more tests reported[b]	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
Time between tests (days)[c]						
n	0	1	1	0	0	2
Mean		21.00	14.00			17.50
Std Dev		-	-			4.950
SE		-	-			3.500
Median		21.00	14.00			17.50
(Min, Max)		(21.0, 21.0)	(14.0, 14.0)			(14.0, 21.0)
95% CI of the mean		(-, -)	(-, -)			(10.64, 24.36)

[a] Eligibility criteria is defined as an AAV9 result of ≤ 1:50.
[b] AAV9 retesting may be performed if an AAV9 result is ≥ 1:50.
[c] Patients who reported 2 or more AAV9 test results.

Table 14.1.9.cpy.jpn
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=11)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=14)
Time of therapy relative to SMA diagnosis (months)						
n	0	11	3	0	0	14
Mean		11.89	9.48			11.38
Std Dev		14.244	15.945			14.009
SE		4.295	9.206			3.744
Median		6.47	0.53			3.55
(Min, Max)		(0.0, 39.7)	(0.0, 27.9)			(0.0, 39.7)
95% CI of the mean		(3.48, 20.31)	(-8.56, 27.53)			(4.04, 18.72)
Time from diagnosis to treatment						
0-6 months n (%)	0	5 (45.5)	2 (66.7)	0	0	7 (50.0)
>6-24 months n (%)	0	3 (27.3)	0	0	0	3 (21.4)
>24 months n (%)	0	3 (27.3)	1 (33.3)	0	0	4 (28.6)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.cpy.jpn
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=11)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=14)
Age at first dose (months)						
n	0	11	3	0	0	14
Mean		15.9	16.7			16.1
Std Dev		13.59	16.17			13.50
SE		4.10	9.33			3.61
Median		8.0	14.0			11.0
(Min, Max)		(2, 42)	(2, 34)			(2, 42)
95% CI of the mean		(7.9, 23.9)	(-1.6, 35.0)			(9.0, 23.1)
< 6 months n (%)	0	3 (27.3)	1 (33.3)	0	0	4 (28.6)
=> 6 and < 12 months n (%)	0	3 (27.3)	0	0	0	3 (21.4)
=> 12 and < 24 months n (%)	0	1 (9.1)	1 (33.3)	0	0	2 (14.3)
=> 24 months n (%)	0	4 (36.4)	1 (33.3)	0	0	5 (35.7)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.cpy.jpn
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=11)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=14)
Duration of therapy (months)						
n	0	11	3	0	0	14
Mean		11.07	4.24			9.61
Std Dev		10.460	5.919			9.901
SE		3.154	3.417			2.646
Median		8.18	0.92			7.47
(Min, Max)		(0.6, 33.2)	(0.7, 11.1)			(0.6, 33.2)
95% CI of the mean		(4.89, 17.26)	(-2.46, 10.94)			(4.42, 14.80)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.cpy.jpn
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=11)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=14)
Has the patient received OAV101 Nusinersen or other SMA treatment and added Risdiplam? n (%)						
n	0	11	3	0	0	14
Yes	0	7 (63.6)	1 (33.3)	0	0	8 (57.1)
No	0	4 (36.4)	2 (66.7)	0	0	6 (42.9)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.cpy.jpn
Risdiplam Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=11)	3 Copies (N=3)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=14)
Primary reason for adding Risdiplam[a]? n (%)						
n	0	7	1	0	0	8
Perceived lack of drug effect	0	3 (42.9)	1 (100)	0	0	4 (50.0)
Motor function	0	1 (14.3)	0	0	0	1 (12.5)
Respiratory function	0	0	1 (100)	0	0	1 (12.5)
Swallowing or feeding ability for age	0	1 (14.3)	0	0	0	1 (12.5)
Electrophysiological/another biomarker response	0	0	0	0	0	0
No additional response chosen	0	1 (14.3)	0	0	0	1 (12.5)
Adverse Events	0	1 (14.3)	0	0	0	1 (12.5)
Parent/Caregiver/Patient decision	0	1 (14.3)	0	0	0	1 (12.5)
Alternative Treatment Available and Reimbursed	0	1 (14.3)	0	0	0	1 (12.5)
Other	0	1 (14.3)	0	0	0	1 (12.5)
Risdiplam Exposure (person-year)	-	10.2	1.1	-	-	11.2

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jpn
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=1)	Transient add-on (N=0)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=5)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=14)
Time of therapy relative to SMA diagnosis (months)								
n	1	0	7	5	1	0	0	14
Mean	25.95		18.79	0.33	0.13			11.38
Std Dev	-		14.240	0.243	-			14.009
SE	-		5.382	0.108	-			3.744
Median	25.95		23.26	0.26	0.13			3.55
(Min, Max)	(26.0, 26.0)		(0.0, 39.7)	(0.0, 0.6)	(0.1, 0.1)			(0.0, 39.7)
95% CI of the mean	(-, -)		(8.24, 29.34)	(0.12, 0.54)	(-, -)			(4.04, 18.72)
Time from diagnosis to treatment								
0-6 months n (%)	0	0	1 (14.3)	5 (100)	1 (100)	0	0	7 (50.0)
>6-24 months n (%)	0	0	3 (42.9)	0	0	0	0	3 (21.4)
>24 months n (%)	1 (100)	0	3 (42.9)	0	0	0	0	4 (28.6)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jpn
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=1)	Transient add-on (N=0)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=5)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=14)
Age at first dose (months)								
n	1	0	7	5	1	0	0	14
Mean	28.0		21.4	9.0	2.0			16.1
Std Dev	-		15.03	7.48	-			13.50
SE	-		5.68	3.35	-			3.61
Median	28.0		27.0	8.0	2.0			11.0
(Min, Max)	(28, 28)		(3, 42)	(2, 19)	(2, 2)			(2, 42)
95% CI of the mean	(-, -)		(10.3, 32.6)	(2.4, 15.6)	(-, -)			(9.0, 23.1)
< 6 months n (%)	0	0	1 (14.3)	2 (40.0)	1 (100)	0	0	4 (28.6)
=> 6 and < 12 months n (%)	0	0	2 (28.6)	1 (20.0)	0	0	0	3 (21.4)
=> 12 and < 24 months n (%)	0	0	0	2 (40.0)	0	0	0	2 (14.3)
=> 24 months n (%)	1 (100)	0	4 (57.1)	0	0	0	0	5 (35.7)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jpn
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=1)	Transient add-on (N=0)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=5)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=14)
Duration of therapy (months)								
n	1	0	7	5	1	0	0	14
Mean	14.39		15.18	1.14	8.18			9.61
Std Dev	-		10.582	0.712	-			9.901
SE	-		4.000	0.318	-			2.646
Median	14.39		11.07	0.92	8.18			7.47
(Min, Max)	(14.4, 14.4)		(5.1, 33.2)	(0.6, 2.4)	(8.2, 8.2)			(0.6, 33.2)
95% CI of the mean	(-, -)		(7.34, 23.02)	(0.52, 1.77)	(-, -)			(4.42, 14.80)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jpn
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=1)	Transient add-on (N=0)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=5)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=14)
Has the patient received OAV101 Nusinersen or other SMA treatment and added Risdiplam? n (%)								
n	1	0	7	5	1	0	0	14
Yes	1 (100)	0	6 (85.7)	1 (20.0)	0	0	0	8 (57.1)
No	0	0	1 (14.3)	4 (80.0)	1 (100)	0	0	6 (42.9)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jpn
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=1)	Transient add-on (N=0)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=5)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=14)
Primary reason for adding Risdiplam[a]? n (%)								
n	1	0	6	1	0	0	0	8
Perceived lack of drug effect	1 (100)	0	3 (50.0)	0	0	0	0	4 (50.0)
Motor function	1 (100)	0	0	0	0	0	0	1 (12.5)
Respiratory function	0	0	1 (16.7)	0	0	0	0	1 (12.5)
Swallowing or feeding ability for age	0	0	1 (16.7)	0	0	0	0	1 (12.5)
Electrophysiological/another biomarker response	0	0	0	0	0	0	0	0
No additional response chosen	0	0	1 (16.7)	0	0	0	0	1 (12.5)
Adverse Events	0	0	1 (16.7)	0	0	0	0	1 (12.5)
Parent/Caregiver/Patient decision	0	0	0	1 (100)	0	0	0	1 (12.5)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.9.txr.jpn
Risdiplam Treatment by Therapy
Japan OAV101 Treated Patients

	Add-on (N=1)	Transient add-on (N=0)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=5)	Switch to OAV101 (N=1)	Nusi/risd combo (N=0)	Risd mono (N=0)	Total (N=14)
Alternative Treatment Available and Reimbursed	0	0	1 (16.7)	0	0	0	0	1 (12.5)
Other	0	0	1 (16.7)	0	0	0	0	1 (12.5)
Risdiplam Exposure (person-year)	1.2	-	8.9	0.5	0.7	-	-	11.2

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
[a] Number receiving OAV101 or other SMA treatment when added is used as the denominator for calculating percentages.

Table 14.1.10.cpy.jpn
Glucocorticosteroid Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=40)	3 Copies (N=40)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=80)
Glucocorticosteroid medication name n (%)						
n	0	39	38	0	0	77
Prednisolone	0	39 (100)	38 (100)	0	0	77 (100)
Prednisone	0	0	0	0	0	0
Other	0	1 (2.6)	3 (7.9)	0	0	4 (5.2)
Prednisolone Dose (mg/kg)						
n	0	38	38	0	0	76
Mean		0.76	0.81			0.78
Std Dev		0.162	0.221			0.194
SE		0.026	0.036			0.022
Median		0.71	0.77			0.74
(Min, Max)		(0.6, 1.5)	(0.5, 1.5)			(0.5, 1.5)
95% CI of the mean		(0.70, 0.81)	(0.74, 0.88)			(0.74, 0.82)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
Patients may have more than one type of glucocorticosteroid.

Table 14.1.10.cpy.jpn
Glucocorticosteroid Treatment by Number of Copies of the SMN2 Gene
Japan OAV101 Treated Patients

	1 Copy (N=0)	2 Copies (N=40)	3 Copies (N=40)	4 Copies (N=0)	>4 Copies (N=0)	Total (N=80)
Duration of Prednisolone (months)						
n	0	38	38	0	0	76
Mean		2.59	3.32			2.95
Std Dev		0.789	1.404			1.189
SE		0.128	0.228			0.136
Median		2.35	3.25			2.71
(Min, Max)		(1.7, 4.6)	(1.0, 7.5)			(1.0, 7.5)
95% CI of the mean		(2.34, 2.84)	(2.87, 3.76)			(2.69, 3.22)

Note: 'n' specified for each variable is used as the denominator for calculating percentages.
Patients may have more than one type of glucocorticosteroid.

Table 14.3.1.0.oav.age3.jpn
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Treatment Emergent Adverse Events						
≥1 TEAE (any grade)	14 (100)	9 (100)	21 (100)	32 (100)	4 (100)	80 (100)
Grade ≥3 AE	7 (50.0)	8 (88.9)	17 (81.0)	27 (84.4)	4 (100)	63 (78.8)
Any Serious AE	8 (57.1)	8 (88.9)	13 (61.9)	21 (65.6)	1 (25.0)	51 (63.8)
Related AE	13 (92.9)	9 (100)	21 (100)	32 (100)	4 (100)	79 (98.8)
OAV101	13 (92.9)	9 (100)	21 (100)	32 (100)	4 (100)	79 (98.8)
NUSI	0	0	0	0	0	0
RISD	0	0	0	0	0	0
Serious Related AE	1 (7.1)	2 (22.2)	5 (23.8)	10 (31.3)	0	18 (22.5)
OAV101	1 (7.1)	2 (22.2)	5 (23.8)	10 (31.3)	0	18 (22.5)
NUSI	0	0	0	0	0	0
RISD	0	0	0	0	0	0

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Table 14.3.1.0.oav.age3.jpn
Summary of OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan OAV101 Treated Patients

	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Any AEs of Special Interest						
Hepatotoxicity	11 (78.6)	8 (88.9)	18 (85.7)	29 (90.6)	4 (100)	70 (87.5)
Transient Thrombocytopenia	3 (21.4)	6 (66.7)	11 (52.4)	26 (81.3)	4 (100)	50 (62.5)
Thrombotic microangiopathy	0	0	0	4 (12.5)	0	4 (5.0)
Cardiac Adverse Events	5 (35.7)	4 (44.4)	6 (28.6)	8 (25.0)	3 (75.0)	26 (32.5)
Sensory Abnormalities Suggestive of Ganglionopathy	0	0	0	0	0	0
New malignancies	0	0	0	0	0	0
New incidence of neurological disorders	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
New incidence of autoimmune disorders	0	0	0	1 (3.1)	0	1 (1.3)
New incidence of hematological disorders	0	1 (11.1)	1 (4.8)	10 (31.3)	1 (25.0)	13 (16.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Any Adverse Event	77 (96.3)	19 (23.8)	22 (27.5)	17 (21.3)	22 (27.8)	20 (27.8)	12 (25.0)	80 (100)
Blood and lymphatic system disorders	13 (16.3)	0	0	0	0	1 (1.4)	1 (2.1)	13 (16.3)
Anaemia	0	0	0	0	0	1 (1.4)	0	1 (1.3)
Eosinophilia	0	0	0	0	0	0	1 (2.1)	1 (1.3)
Haemolytic anaemia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Thrombocytopenia	9 (11.3)	0	0	0	0	0	0	9 (11.3)
Thrombotic microangiopathy	4 (5.0)	0	0	0	0	0	0	4 (5.0)
Cardiac disorders	1 (1.3)	2 (2.5)	0	0	1 (1.3)	0	0	4 (5.0)
Arrhythmia	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Bradycardia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Cardiac failure congestive	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Cardio-respiratory arrest	0	0	0	0	1 (1.3)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Congenital, familial and genetic disorders	0	0	0	0	3 (3.8)	0	1 (2.1)	4 (5.0)
Cryptorchism	0	0	0	0	3 (3.8)	0	1 (2.1)	4 (5.0)
Endocrine disorders	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Cushingoid	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Gastrointestinal disorders	33 (41.3)	2 (2.5)	2 (2.5)	1 (1.3)	1 (1.3)	3 (4.2)	2 (4.2)	39 (48.8)
Constipation	0	0	1 (1.3)	0	0	0	1 (2.1)	2 (2.5)
Diarrhoea	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Dysphagia	0	1 (1.3)	0	0	1 (1.3)	1 (1.4)	0	3 (3.8)
Gastric fistula	0	0	0	1 (1.3)	0	2 (2.8)	1 (2.1)	4 (5.0)
Gastric haemorrhage	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Nausea	5 (6.3)	0	0	0	0	0	0	5 (6.3)
Vomiting	30 (37.5)	1 (1.3)	0	0	0	0	1 (2.1)	32 (40.0)

Adverse Events were coded using MedDRA, version 27.0.
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Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
General disorders and administration site conditions	64 (80.0)	1 (1.3)	2 (2.5)	1 (1.3)	0	0	0	65 (81.3)
Asthenia	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Drug withdrawal syndrome	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Generalised oedema	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Inflammation	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Malaise	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Medical device site haemorrhage	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Pyrexia	64 (80.0)	0	1 (1.3)	0	0	0	0	65 (81.3)
Hepatobiliary disorders	8 (10.0)	0	3 (3.8)	0	0	1 (1.4)	0	11 (13.8)
Acute hepatic failure	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Cholelithiasis	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Hepatic function abnormal	8 (10.0)	0	2 (2.5)	0	0	1 (1.4)	0	11 (13.8)
Hepatomegaly	1 (1.3)	0	0	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Immune system disorders	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Hypersensitivity	1 (1.3)	0	0	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Infections and infestations	3 (3.8)	3 (3.8)	10 (12.5)	12 (15.0)	15 (19.0)	15 (20.8)	10 (20.8)	42 (52.5)
Bronchiolitis	0	0	0	0	0	1 (1.4)	0	1 (1.3)
Bronchitis	0	1 (1.3)	0	2 (2.5)	1 (1.3)	4 (5.6)	0	8 (10.0)
Bronchitis viral	0	0	1 (1.3)	0	0	0	1 (2.1)	2 (2.5)
COVID-19	0	0	1 (1.3)	0	3 (3.8)	1 (1.4)	3 (6.3)	8 (10.0)
Epstein-Barr virus infection	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Gastroenteritis	0	0	0	0	0	1 (1.4)	1 (2.1)	2 (2.5)
Gastroenteritis norovirus	0	0	0	0	0	1 (1.4)	0	1 (1.3)
Lower respiratory tract infection	0	0	0	0	0	0	1 (2.1)	1 (1.3)
Metapneumovirus bronchiolitis	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Metapneumovirus infection	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Metapneumovirus pneumonia	0	0	0	0	0	1 (1.4)	0	1 (1.3)
Nasopharyngitis	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Norovirus infection	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Parainfluenzae virus infection	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Pharyngitis	0	0	0	0	0	0	1 (2.1)	1 (1.3)
Pneumonia	0	0	0	0	2 (2.5)	5 (6.9)	4 (8.3)	11 (13.8)

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Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Pneumonia aspiration	2 (2.5)	1 (1.3)	0	3 (3.8)	0	4 (5.6)	2 (4.2)	12 (15.0)
Pneumonia influenzal	0	0	0	0	1 (1.3)	0	1 (2.1)	2 (2.5)
Pneumonia parainfluenzae viral	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Pneumonia respiratory syncytial viral	0	0	2 (2.5)	2 (2.5)	2 (2.5)	0	0	6 (7.5)
Pneumonia viral	0	0	0	0	0	0	1 (2.1)	1 (1.3)
Respiratory syncytial virus bronchiolitis	0	0	0	0	1 (1.3)	2 (2.8)	0	3 (3.8)
Respiratory syncytial virus infection	0	0	1 (1.3)	0	2 (2.5)	2 (2.8)	0	5 (6.3)
Respiratory tract infection	1 (1.3)	0	0	0	0	0	1 (2.1)	2 (2.5)
Rhinovirus infection	0	0	1 (1.3)	1 (1.3)	0	0	0	2 (2.5)
Serratia infection	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Tonsillitis	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Upper respiratory tract infection	0	0	2 (2.5)	3 (3.8)	3 (3.8)	1 (1.4)	1 (2.1)	10 (12.5)
Viral infection	0	0	0	0	0	1 (1.4)	0	1 (1.3)
Viral upper respiratory tract infection	0	0	0	0	2 (2.5)	0	0	2 (2.5)

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Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Injury, poisoning and procedural complications	0	0	0	1 (1.3)	0	3 (4.2)	0	4 (5.0)
Femur fracture	0	0	0	1 (1.3)	0	1 (1.4)	0	2 (2.5)
Joint dislocation	0	0	0	0	0	2 (2.8)	0	2 (2.5)

Adverse Events were coded using MedDRA, version 27.0.
Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Investigations	72 (90.0)	10 (12.5)	6 (7.5)	2 (2.5)	2 (2.5)	0	0	72 (90.0)
Alanine aminotransferase increased	46 (57.5)	2 (2.5)	4 (5.0)	0	0	0	0	52 (65.0)
Amylase increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Aspartate aminotransferase increased	51 (63.8)	1 (1.3)	1 (1.3)	0	0	0	0	53 (66.3)
Blood creatine phosphokinase MB increased	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Blood creatine phosphokinase increased	6 (7.5)	0	1 (1.3)	1 (1.3)	0	0	0	8 (10.0)
Blood creatinine increased	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Blood lactate dehydrogenase increased	21 (26.3)	0	0	0	0	0	0	21 (26.3)
Blood pressure increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Blood urea increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
C-reactive protein increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Gamma-glutamyltransferase increased	0	3 (3.8)	0	0	0	0	0	3 (3.8)
Haemoglobin decreased	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Haptoglobin decreased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Hepatic enzyme increased	6 (7.5)	0	0	1 (1.3)	0	0	0	7 (8.8)

Adverse Events were coded using MedDRA, version 27.0.
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Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Intraocular pressure increased	0	1 (1.3)	0	0	0	0	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Neutrophil count decreased	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Platelet count decreased	40 (50.0)	0	0	0	0	0	0	40 (50.0)
Serum ferritin increased	15 (18.8)	0	0	0	0	0	0	15 (18.8)
Troponin I increased	11 (13.8)	2 (2.5)	0	0	0	0	0	13 (16.3)
Troponin T increased	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Urine output decreased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Weight decreased	2 (2.5)	1 (1.3)	0	0	2 (2.5)	0	0	5 (6.3)
Weight increased	1 (1.3)	0	0	0	0	0	0	1 (1.3)
White blood cell count decreased	7 (8.8)	0	0	0	0	0	0	7 (8.8)

Adverse Events were coded using MedDRA, version 27.0.
Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Metabolism and nutrition disorders	18 (22.5)	0	1 (1.3)	1 (1.3)	1 (1.3)	1 (1.4)	0	21 (26.3)
Decreased appetite	14 (17.5)	0	0	0	0	0	0	14 (17.5)
Feeding disorder	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Hypercholesterolaemia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Hypertriglyceridaemia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Hypoglycaemia	0	0	0	0	0	1 (1.4)	0	1 (1.3)
Hypophagia	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Metabolic acidosis	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Underweight	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Weight gain poor	2 (2.5)	0	0	1 (1.3)	0	0	0	3 (3.8)

Adverse Events were coded using MedDRA, version 27.0.
Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Musculoskeletal and connective tissue disorders	0	0	1 (1.3)	1 (1.3)	3 (3.8)	1 (1.4)	0	5 (6.3)
Joint range of motion decreased	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Kyphosis	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Muscle atrophy	0	0	0	1 (1.3)	0	0	0	1 (1.3)
Scoliosis	0	0	1 (1.3)	0	2 (2.5)	1 (1.4)	0	4 (5.0)
Nervous system disorders	0	1 (1.3)	0	0	1 (1.3)	0	0	2 (2.5)
Cerebral atrophy	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Seizure	0	0	0	0	1 (1.3)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Renal and urinary disorders	3 (3.8)	0	1 (1.3)	0	0	0	0	4 (5.0)
Acute kidney injury	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Chronic kidney disease	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Glycosuria	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Haematuria	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Proteinuria	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Renal impairment	1 (1.3)	0	0	0	0	0	0	1 (1.3)

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Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Respiratory, thoracic and mediastinal disorders	0	2 (2.5)	3 (3.8)	2 (2.5)	2 (2.5)	4 (5.6)	2 (4.2)	12 (15.0)
Acute respiratory failure	0	0	0	0	1 (1.3)	3 (4.2)	0	4 (5.0)
Apnoea	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Atelectasis	0	0	0	2 (2.5)	0	1 (1.4)	1 (2.1)	4 (5.0)
Hypoxia	0	0	0	0	0	1 (1.4)	0	1 (1.3)
Nocturnal dyspnoea	0	0	0	0	0	1 (1.4)	0	1 (1.3)
Pleural effusion	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Respiration abnormal	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Respiratory disorder	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Respiratory failure	0	0	0	0	1 (1.3)	0	1 (2.1)	2 (2.5)
Sleep apnoea syndrome	0	1 (1.3)	0	0	0	0	0	1 (1.3)
Upper respiratory tract inflammation	0	0	1 (1.3)	0	0	0	0	1 (1.3)
Skin and subcutaneous tissue disorders	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Urticaria	1 (1.3)	0	0	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.2.oav.aeptin.jpn
OAV101 Treatment Emergent Adverse Events Incidence of First Event
Japan OAV101 Treated Patients

SOC Preferred Term	≤ 2 wks (N=80)	> 2 and ≤ 4 wks (N=80)	> 4 wks and ≤ 3 months (N=80)	> 3 months and ≤ 6 months (N=80)	> 6 months and ≤ 12 months (N=79)	> 12 months and ≤ 24 months (N=72)	> 24 months (N=48)	Total (N=80)
Vascular disorders	2 (2.5)	0	0	0	1 (1.3)	0	3 (6.3)	6 (7.5)
Hypertension	2 (2.5)	0	0	0	0	0	0	2 (2.5)
Kawasaki's disease	0	0	0	0	1 (1.3)	0	3 (6.3)	4 (5.0)
Not Coded	2 (2.5)	0	0	0	1 (1.3)	0	1 (2.1)	4 (5.0)
Acute liver disorder(Grade 4)	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Liver dysfunction(AST/ALT)	1 (1.3)	0	0	0	0	0	0	1 (1.3)
Motor developmental delay due to inadequate effect of Zolgensma	0	0	0	0	1 (1.3)	0	0	1 (1.3)
Respiratory tract infections with human metapneumovirus	0	0	0	0	0	0	1 (2.1)	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Percentages are based on the number of patients who had at least one Adverse Event Form completed and with exposure indicated in column header.
Patients are only counted once at each level of summarization.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Any Adverse Event	14 (100)	9 (100)	21 (100)	32 (100)	4 (100)	80 (100)
Blood and lymphatic system disorders	0	1 (11.1)	1 (4.8)	10 (31.3)	1 (25.0)	13 (16.3)
Thrombocytopenia	0	1 (11.1)	1 (4.8)	6 (18.8)	1 (25.0)	9 (11.3)
Thrombotic microangiopathy	0	0	0	4 (12.5)	0	4 (5.0)
Anaemia	0	1 (11.1)	0	0	0	1 (1.3)
Eosinophilia	0	0	0	0	1 (25.0)	1 (1.3)
Haemolytic anaemia	0	0	0	1 (3.1)	0	1 (1.3)
Cardiac disorders	0	0	2 (9.5)	2 (6.3)	0	4 (5.0)
Arrhythmia	0	0	0	1 (3.1)	0	1 (1.3)
Bradycardia	0	0	1 (4.8)	0	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.1)	0	1 (1.3)
Cardio-respiratory arrest	0	0	1 (4.8)	0	0	1 (1.3)
Congenital, familial and genetic disorders	2 (14.3)	0	1 (4.8)	1 (3.1)	0	4 (5.0)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Cryptorchism	2 (14.3)	0	1 (4.8)	1 (3.1)	0	4 (5.0)
Endocrine disorders	0	0	1 (4.8)	0	0	1 (1.3)
Cushingoid	0	0	1 (4.8)	0	0	1 (1.3)
Gastrointestinal disorders	4 (28.6)	6 (66.7)	13 (61.9)	13 (40.6)	3 (75.0)	39 (48.8)
Vomiting	4 (28.6)	5 (55.6)	10 (47.6)	11 (34.4)	2 (50.0)	32 (40.0)
Nausea	0	0	1 (4.8)	3 (9.4)	1 (25.0)	5 (6.3)
Gastric fistula	1 (7.1)	0	3 (14.3)	0	0	4 (5.0)
Dysphagia	1 (7.1)	1 (11.1)	1 (4.8)	0	0	3 (3.8)
Constipation	1 (7.1)	0	1 (4.8)	0	0	2 (2.5)
Diarrhoea	1 (7.1)	0	1 (4.8)	0	0	2 (2.5)
Gastric haemorrhage	0	0	0	0	1 (25.0)	1 (1.3)
General disorders and administration site conditions	9 (64.3)	7 (77.8)	19 (90.5)	26 (81.3)	4 (100)	65 (81.3)
Pyrexia	9 (64.3)	7 (77.8)	19 (90.5)	26 (81.3)	4 (100)	65 (81.3)
Asthenia	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Malaise	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Drug withdrawal syndrome	0	0	0	1 (3.1)	0	1 (1.3)
Generalised oedema	0	0	0	1 (3.1)	0	1 (1.3)
Inflammation	0	0	0	1 (3.1)	0	1 (1.3)
Medical device site haemorrhage	0	0	0	1 (3.1)	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (4.8)	0	0	1 (1.3)
Hepatobiliary disorders	3 (21.4)	2 (22.2)	4 (19.0)	1 (3.1)	1 (25.0)	11 (13.8)
Hepatic function abnormal	3 (21.4)	2 (22.2)	4 (19.0)	1 (3.1)	1 (25.0)	11 (13.8)
Acute hepatic failure	0	0	1 (4.8)	0	0	1 (1.3)
Cholelithiasis	0	0	0	1 (3.1)	0	1 (1.3)
Hepatomegaly	0	0	0	0	1 (25.0)	1 (1.3)
Immune system disorders	0	0	0	1 (3.1)	0	1 (1.3)
Hypersensitivity	0	0	0	1 (3.1)	0	1 (1.3)
Infections and infestations	5 (35.7)	7 (77.8)	11 (52.4)	19 (59.4)	0	42 (52.5)
Pneumonia aspiration	2 (14.3)	2 (22.2)	2 (9.5)	6 (18.8)	0	12 (15.0)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Pneumonia	2 (14.3)	3 (33.3)	1 (4.8)	5 (15.6)	0	11 (13.8)
Upper respiratory tract infection	2 (14.3)	1 (11.1)	3 (14.3)	4 (12.5)	0	10 (12.5)
Bronchitis	0	1 (11.1)	4 (19.0)	3 (9.4)	0	8 (10.0)
COVID-19	2 (14.3)	2 (22.2)	3 (14.3)	1 (3.1)	0	8 (10.0)
Pneumonia respiratory syncytial viral	1 (7.1)	1 (11.1)	1 (4.8)	3 (9.4)	0	6 (7.5)
Respiratory syncytial virus infection	1 (7.1)	1 (11.1)	0	3 (9.4)	0	5 (6.3)
Respiratory syncytial virus bronchiolitis	0	0	2 (9.5)	1 (3.1)	0	3 (3.8)
Bronchitis viral	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Gastroenteritis	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Pneumonia influenzal	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Respiratory tract infection	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Rhinovirus infection	0	0	2 (9.5)	0	0	2 (2.5)
Viral upper respiratory tract infection	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Bronchiolitis	0	0	1 (4.8)	0	0	1 (1.3)
Epstein-Barr virus infection	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Gastroenteritis norovirus	0	0	1 (4.8)	0	0	1 (1.3)
Lower respiratory tract infection	0	0	0	1 (3.1)	0	1 (1.3)
Metapneumovirus bronchiolitis	0	0	0	1 (3.1)	0	1 (1.3)
Metapneumovirus infection	0	0	1 (4.8)	0	0	1 (1.3)
Metapneumovirus pneumonia	0	1 (11.1)	0	0	0	1 (1.3)
Nasopharyngitis	0	1 (11.1)	0	0	0	1 (1.3)
Norovirus infection	0	0	1 (4.8)	0	0	1 (1.3)
Parainfluenzae virus infection	0	0	1 (4.8)	0	0	1 (1.3)
Pharyngitis	0	1 (11.1)	0	0	0	1 (1.3)
Pneumonia parainfluenzae viral	1 (7.1)	0	0	0	0	1 (1.3)
Pneumonia viral	0	0	1 (4.8)	0	0	1 (1.3)
Serratia infection	0	0	0	1 (3.1)	0	1 (1.3)
Tonsillitis	0	0	1 (4.8)	0	0	1 (1.3)
Viral infection	0	1 (11.1)	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Injury, poisoning and procedural complications	2 (14.3)	0	0	2 (6.3)	0	4 (5.0)
Femur fracture	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Joint dislocation	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Investigations	12 (85.7)	8 (88.9)	19 (90.5)	29 (90.6)	4 (100)	72 (90.0)
Aspartate aminotransferase increased	7 (50.0)	5 (55.6)	12 (57.1)	26 (81.3)	3 (75.0)	53 (66.3)
Alanine aminotransferase increased	6 (42.9)	5 (55.6)	12 (57.1)	26 (81.3)	3 (75.0)	52 (65.0)
Platelet count decreased	3 (21.4)	5 (55.6)	10 (47.6)	19 (59.4)	3 (75.0)	40 (50.0)
Blood lactate dehydrogenase increased	0	4 (44.4)	6 (28.6)	10 (31.3)	1 (25.0)	21 (26.3)
Serum ferritin increased	1 (7.1)	3 (33.3)	4 (19.0)	5 (15.6)	2 (50.0)	15 (18.8)
Troponin I increased	5 (35.7)	3 (33.3)	2 (9.5)	2 (6.3)	1 (25.0)	13 (16.3)
Blood creatine phosphokinase increased	1 (7.1)	1 (11.1)	1 (4.8)	4 (12.5)	1 (25.0)	8 (10.0)
Hepatic enzyme increased	1 (7.1)	2 (22.2)	2 (9.5)	2 (6.3)	0	7 (8.8)
White blood cell count decreased	2 (14.3)	2 (22.2)	3 (14.3)	0	0	7 (8.8)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Weight decreased	0	2 (22.2)	0	3 (9.4)	0	5 (6.3)
Gamma-glutamyltransferase increased	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Blood creatinine increased	0	0	0	2 (6.3)	0	2 (2.5)
Haemoglobin decreased	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Neutrophil count decreased	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Troponin T increased	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Amylase increased	0	0	1 (4.8)	0	0	1 (1.3)
Blood creatine phosphokinase MB increased	0	0	1 (4.8)	0	0	1 (1.3)
Blood pressure increased	0	0	0	1 (3.1)	0	1 (1.3)
Blood urea increased	0	0	0	1 (3.1)	0	1 (1.3)
C-reactive protein increased	0	0	0	1 (3.1)	0	1 (1.3)
Haptoglobin decreased	0	0	0	1 (3.1)	0	1 (1.3)
Intraocular pressure increased	0	0	1 (4.8)	0	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	0	0	1 (4.8)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Urine output decreased	0	0	1 (4.8)	0	0	1 (1.3)
Weight increased	0	0	0	1 (3.1)	0	1 (1.3)
Metabolism and nutrition disorders	2 (14.3)	3 (33.3)	6 (28.6)	9 (28.1)	1 (25.0)	21 (26.3)
Decreased appetite	0	1 (11.1)	5 (23.8)	7 (21.9)	1 (25.0)	14 (17.5)
Weight gain poor	0	1 (11.1)	2 (9.5)	0	0	3 (3.8)
Feeding disorder	1 (7.1)	0	0	0	0	1 (1.3)
Hypercholesterolaemia	0	1 (11.1)	0	0	0	1 (1.3)
Hypertriglyceridaemia	0	1 (11.1)	0	0	0	1 (1.3)
Hypoglycaemia	0	0	0	1 (3.1)	0	1 (1.3)
Hypophagia	0	0	0	1 (3.1)	0	1 (1.3)
Metabolic acidosis	0	0	0	1 (3.1)	0	1 (1.3)
Underweight	1 (7.1)	0	0	0	0	1 (1.3)
Musculoskeletal and connective tissue disorders	0	2 (22.2)	2 (9.5)	1 (3.1)	0	5 (6.3)
Scoliosis	0	2 (22.2)	1 (4.8)	1 (3.1)	0	4 (5.0)
Joint range of motion decreased	0	0	1 (4.8)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Kyphosis	0	1 (11.1)	0	0	0	1 (1.3)
Muscle atrophy	0	1 (11.1)	0	0	0	1 (1.3)
Nervous system disorders	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Cerebral atrophy	0	0	0	1 (3.1)	0	1 (1.3)
Seizure	0	0	1 (4.8)	0	0	1 (1.3)
Renal and urinary disorders	0	0	0	4 (12.5)	0	4 (5.0)
Haematuria	0	0	0	2 (6.3)	0	2 (2.5)
Proteinuria	0	0	0	2 (6.3)	0	2 (2.5)
Acute kidney injury	0	0	0	1 (3.1)	0	1 (1.3)
Chronic kidney disease	0	0	0	1 (3.1)	0	1 (1.3)
Glycosuria	0	0	0	1 (3.1)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.1)	0	1 (1.3)
Respiratory, thoracic and mediastinal disorders	5 (35.7)	2 (22.2)	1 (4.8)	4 (12.5)	0	12 (15.0)
Acute respiratory failure	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Atelectasis	0	1 (11.1)	0	3 (9.4)	0	4 (5.0)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Respiratory failure	2 (14.3)	0	0	0	0	2 (2.5)
Apnoea	1 (7.1)	0	0	0	0	1 (1.3)
Hypoxia	1 (7.1)	0	0	0	0	1 (1.3)
Nocturnal dyspnoea	1 (7.1)	0	0	0	0	1 (1.3)
Pleural effusion	0	0	1 (4.8)	0	0	1 (1.3)
Respiration abnormal	0	1 (11.1)	0	0	0	1 (1.3)
Respiratory disorder	0	0	1 (4.8)	0	0	1 (1.3)
Sleep apnoea syndrome	0	1 (11.1)	0	0	0	1 (1.3)
Upper respiratory tract inflammation	1 (7.1)	0	0	0	0	1 (1.3)
Skin and subcutaneous tissue disorders	0	0	0	0	1 (25.0)	1 (1.3)
Urticaria	0	0	0	0	1 (25.0)	1 (1.3)
Vascular disorders	1 (7.1)	1 (11.1)	0	4 (12.5)	0	6 (7.5)
Kawasaki's disease	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Hypertension	0	0	0	2 (6.3)	0	2 (2.5)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Not Coded	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Acute liver disorder(Grade 4)	0	0	0	1 (3.1)	0	1 (1.3)
Liver dysfunction(AST/ALT)	0	0	0	1 (3.1)	0	1 (1.3)
Motor developmental delay due to inadequate effect of Zolgensma	1 (7.1)	0	0	0	0	1 (1.3)
Respiratory tract infections with human metapneumovirus	0	1 (11.1)	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Any Adverse Event	14 (100)	9 (100)	21 (100)	32 (100)	4 (100)	80 (100)
Blood and lymphatic system disorders	0	1 (11.1)	1 (4.8)	10 (31.3)	1 (25.0)	13 (16.3)
Grade 1	0	0	0	5 (15.6)	0	5 (6.3)
Grade 3	0	1 (11.1)	1 (4.8)	3 (9.4)	1 (25.0)	6 (7.5)
Grade 4	0	0	0	2 (6.3)	0	2 (2.5)
Thrombocytopenia	0	1 (11.1)	1 (4.8)	6 (18.8)	1 (25.0)	9 (11.3)
Grade 1	0	0	0	5 (15.6)	1 (25.0)	6 (7.5)
Grade 3	0	1 (11.1)	1 (4.8)	1 (3.1)	0	3 (3.8)
Thrombotic microangiopathy	0	0	0	4 (12.5)	0	4 (5.0)
Grade 3	0	0	0	2 (6.3)	0	2 (2.5)
Grade 4	0	0	0	2 (6.3)	0	2 (2.5)
Anaemia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Eosinophilia	0	0	0	0	1 (25.0)	1 (1.3)
Grade 3	0	0	0	0	1 (25.0)	1 (1.3)
Haemolytic anaemia	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Cardiac disorders	0	0	2 (9.5)	2 (6.3)	0	4 (5.0)
Grade 1	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Grade 5	0	0	1 (4.8)	0	0	1 (1.3)
Arrhythmia	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Bradycardia	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Cardio-respiratory arrest	0	0	1 (4.8)	0	0	1 (1.3)
Grade 5	0	0	1 (4.8)	0	0	1 (1.3)
Congenital, familial and genetic disorders	2 (14.3)	0	1 (4.8)	1 (3.1)	0	4 (5.0)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 3	2 (14.3)	0	1 (4.8)	0	0	3 (3.8)
Cryptorchism	2 (14.3)	0	1 (4.8)	1 (3.1)	0	4 (5.0)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	2 (14.3)	0	1 (4.8)	0	0	3 (3.8)
Endocrine disorders	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Cushingoid	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Gastrointestinal disorders	4 (28.6)	6 (66.7)	13 (61.9)	13 (40.6)	3 (75.0)	39 (48.8)
Grade 1	2 (14.3)	4 (44.4)	6 (28.6)	8 (25.0)	2 (50.0)	22 (27.5)
Grade 2	0	1 (11.1)	3 (14.3)	5 (15.6)	1 (25.0)	10 (12.5)
Grade 3	1 (7.1)	1 (11.1)	4 (19.0)	0	0	6 (7.5)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Vomiting	4 (28.6)	5 (55.6)	10 (47.6)	11 (34.4)	2 (50.0)	32 (40.0)
Grade 1	3 (21.4)	4 (44.4)	7 (33.3)	7 (21.9)	1 (25.0)	22 (27.5)
Grade 2	0	1 (11.1)	3 (14.3)	4 (12.5)	1 (25.0)	9 (11.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
Nausea	0	0	1 (4.8)	3 (9.4)	1 (25.0)	5 (6.3)
Grade 1	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Grade 2	0	0	0	1 (3.1)	1 (25.0)	2 (2.5)
Gastric fistula	1 (7.1)	0	3 (14.3)	0	0	4 (5.0)
Grade 3	1 (7.1)	0	3 (14.3)	0	0	4 (5.0)
Dysphagia	1 (7.1)	1 (11.1)	1 (4.8)	0	0	3 (3.8)
Grade 3	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Constipation	1 (7.1)	0	1 (4.8)	0	0	2 (2.5)
Grade 2	1 (7.1)	0	1 (4.8)	0	0	2 (2.5)
Diarrhoea	1 (7.1)	0	1 (4.8)	0	0	2 (2.5)
Grade 1	1 (7.1)	0	1 (4.8)	0	0	2 (2.5)
Gastric haemorrhage	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
General disorders and administration site conditions	9 (64.3)	7 (77.8)	19 (90.5)	26 (81.3)	4 (100)	65 (81.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 1	9 (64.3)	5 (55.6)	12 (57.1)	17 (53.1)	4 (100)	47 (58.8)
Grade 2	0	2 (22.2)	5 (23.8)	7 (21.9)	0	14 (17.5)
Grade 3	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Grade 4	0	0	1 (4.8)	0	0	1 (1.3)
Pyrexia	9 (64.3)	7 (77.8)	19 (90.5)	26 (81.3)	4 (100)	65 (81.3)
Grade 1	9 (64.3)	5 (55.6)	13 (61.9)	18 (56.3)	4 (100)	49 (61.3)
Grade 2	0	2 (22.2)	5 (23.8)	8 (25.0)	0	15 (18.8)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Asthenia	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Malaise	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Drug withdrawal syndrome	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Generalised oedema	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)

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Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Inflammation	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Medical device site haemorrhage	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (4.8)	0	0	1 (1.3)
Grade 4	0	0	1 (4.8)	0	0	1 (1.3)
Hepatobiliary disorders	3 (21.4)	2 (22.2)	4 (19.0)	1 (3.1)	1 (25.0)	11 (13.8)
Grade 1	1 (7.1)	0	1 (4.8)	0	0	2 (2.5)
Grade 2	2 (14.3)	2 (22.2)	0	1 (3.1)	0	5 (6.3)
Grade 3	0	0	1 (4.8)	0	1 (25.0)	2 (2.5)
Grade 4	0	0	2 (9.5)	0	0	2 (2.5)
Hepatic function abnormal	3 (21.4)	2 (22.2)	4 (19.0)	1 (3.1)	1 (25.0)	11 (13.8)
Grade 1	1 (7.1)	0	2 (9.5)	0	0	3 (3.8)
Grade 2	2 (14.3)	2 (22.2)	0	1 (3.1)	0	5 (6.3)
Grade 3	0	0	1 (4.8)	0	1 (25.0)	2 (2.5)
Grade 4	0	0	1 (4.8)	0	0	1 (1.3)
Acute hepatic failure	0	0	1 (4.8)	0	0	1 (1.3)

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 4	0	0	1 (4.8)	0	0	1 (1.3)
Cholelithiasis	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Hepatomegaly	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Immune system disorders	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Hypersensitivity	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Infections and infestations	5 (35.7)	7 (77.8)	11 (52.4)	19 (59.4)	0	42 (52.5)
Grade 1	0	0	2 (9.5)	1 (3.1)	0	3 (3.8)
Grade 2	0	0	2 (9.5)	5 (15.6)	0	7 (8.8)
Grade 3	4 (28.6)	3 (33.3)	5 (23.8)	11 (34.4)	0	23 (28.8)
Grade 4	1 (7.1)	4 (44.4)	2 (9.5)	2 (6.3)	0	9 (11.3)
Pneumonia aspiration	2 (14.3)	2 (22.2)	2 (9.5)	6 (18.8)	0	12 (15.0)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)

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Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 2	0	0	0	2 (6.3)	0	2 (2.5)
Grade 3	1 (7.1)	2 (22.2)	1 (4.8)	3 (9.4)	0	7 (8.8)
Grade 4	1 (7.1)	0	1 (4.8)	0	0	2 (2.5)
Pneumonia	2 (14.3)	3 (33.3)	1 (4.8)	5 (15.6)	0	11 (13.8)
Grade 3	1 (7.1)	2 (22.2)	1 (4.8)	5 (15.6)	0	9 (11.3)
Grade 4	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Upper respiratory tract infection	2 (14.3)	1 (11.1)	3 (14.3)	4 (12.5)	0	10 (12.5)
Grade 1	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 2	1 (7.1)	0	0	2 (6.3)	0	3 (3.8)
Grade 3	1 (7.1)	1 (11.1)	2 (9.5)	1 (3.1)	0	5 (6.3)
Bronchitis	0	1 (11.1)	4 (19.0)	3 (9.4)	0	8 (10.0)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	1 (11.1)	3 (14.3)	3 (9.4)	0	7 (8.8)
COVID-19	2 (14.3)	2 (22.2)	3 (14.3)	1 (3.1)	0	8 (10.0)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	2 (14.3)	2 (22.2)	2 (9.5)	0	0	6 (7.5)
Pneumonia respiratory syncytial viral	1 (7.1)	1 (11.1)	1 (4.8)	3 (9.4)	0	6 (7.5)

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Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	1 (7.1)	0	0	2 (6.3)	0	3 (3.8)
Grade 4	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
Respiratory syncytial virus infection	1 (7.1)	1 (11.1)	0	3 (9.4)	0	5 (6.3)
Grade 3	1 (7.1)	0	0	2 (6.3)	0	3 (3.8)
Grade 4	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Respiratory syncytial virus bronchiolitis	0	0	2 (9.5)	1 (3.1)	0	3 (3.8)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	2 (9.5)	0	0	2 (2.5)
Bronchitis viral	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 3	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Gastroenteritis	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Grade 3	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Pneumonia influenzal	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Respiratory tract infection	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)

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Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Rhinovirus infection	0	0	2 (9.5)	0	0	2 (2.5)
Grade 3	0	0	2 (9.5)	0	0	2 (2.5)
Viral upper respiratory tract infection	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Grade 3	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Bronchiolitis	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Epstein-Barr virus infection	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Gastroenteritis norovirus	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Lower respiratory tract infection	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Metapneumovirus bronchiolitis	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Metapneumovirus infection	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)

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Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Metapneumovirus pneumonia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Nasopharyngitis	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Norovirus infection	0	0	1 (4.8)	0	0	1 (1.3)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
Parainfluenzae virus infection	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Pharyngitis	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Pneumonia parainfluenzae viral	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
Pneumonia viral	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Serratia infection	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Tonsillitis	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)

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Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Viral infection	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	0	1 (11.1)	0	0	0	1 (1.3)
Injury, poisoning and procedural complications	2 (14.3)	0	0	2 (6.3)	0	4 (5.0)
Grade 2	2 (14.3)	0	0	1 (3.1)	0	3 (3.8)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Femur fracture	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Grade 2	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Joint dislocation	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Investigations	12 (85.7)	8 (88.9)	19 (90.5)	29 (90.6)	4 (100)	72 (90.0)
Grade 1	7 (50.0)	3 (33.3)	3 (14.3)	3 (9.4)	1 (25.0)	17 (21.3)
Grade 2	3 (21.4)	1 (11.1)	4 (19.0)	4 (12.5)	0	12 (15.0)
Grade 3	1 (7.1)	3 (33.3)	9 (42.9)	15 (46.9)	3 (75.0)	31 (38.8)
Grade 4	1 (7.1)	1 (11.1)	3 (14.3)	7 (21.9)	0	12 (15.0)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Aspartate aminotransferase increased	7 (50.0)	5 (55.6)	12 (57.1)	26 (81.3)	3 (75.0)	53 (66.3)
Grade 1	3 (21.4)	1 (11.1)	1 (4.8)	3 (9.4)	0	8 (10.0)
Grade 2	3 (21.4)	1 (11.1)	3 (14.3)	4 (12.5)	0	11 (13.8)
Grade 3	1 (7.1)	2 (22.2)	6 (28.6)	16 (50.0)	3 (75.0)	28 (35.0)
Grade 4	0	1 (11.1)	2 (9.5)	3 (9.4)	0	6 (7.5)
Alanine aminotransferase increased	6 (42.9)	5 (55.6)	12 (57.1)	26 (81.3)	3 (75.0)	52 (65.0)
Grade 1	5 (35.7)	2 (22.2)	1 (4.8)	3 (9.4)	0	11 (13.8)
Grade 2	0	1 (11.1)	2 (9.5)	5 (15.6)	1 (25.0)	9 (11.3)
Grade 3	1 (7.1)	1 (11.1)	7 (33.3)	13 (40.6)	2 (50.0)	24 (30.0)
Grade 4	0	1 (11.1)	2 (9.5)	5 (15.6)	0	8 (10.0)
Platelet count decreased	3 (21.4)	5 (55.6)	10 (47.6)	19 (59.4)	3 (75.0)	40 (50.0)
Grade 1	2 (14.3)	5 (55.6)	8 (38.1)	7 (21.9)	1 (25.0)	23 (28.8)
Grade 2	1 (7.1)	0	0	8 (25.0)	1 (25.0)	10 (12.5)
Grade 3	0	0	2 (9.5)	2 (6.3)	1 (25.0)	5 (6.3)
Grade 4	0	0	0	2 (6.3)	0	2 (2.5)
Blood lactate dehydrogenase increased	0	4 (44.4)	6 (28.6)	10 (31.3)	1 (25.0)	21 (26.3)
Grade 1	0	4 (44.4)	5 (23.8)	10 (31.3)	1 (25.0)	20 (25.0)

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Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
Serum ferritin increased	1 (7.1)	3 (33.3)	4 (19.0)	5 (15.6)	2 (50.0)	15 (18.8)
Grade 1	1 (7.1)	3 (33.3)	3 (14.3)	4 (12.5)	2 (50.0)	13 (16.3)
Grade 2	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Troponin I increased	5 (35.7)	3 (33.3)	2 (9.5)	2 (6.3)	1 (25.0)	13 (16.3)
Grade 1	5 (35.7)	3 (33.3)	2 (9.5)	2 (6.3)	1 (25.0)	13 (16.3)
Blood creatine phosphokinase increased	1 (7.1)	1 (11.1)	1 (4.8)	4 (12.5)	1 (25.0)	8 (10.0)
Grade 1	1 (7.1)	1 (11.1)	1 (4.8)	3 (9.4)	1 (25.0)	7 (8.8)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Hepatic enzyme increased	1 (7.1)	2 (22.2)	2 (9.5)	2 (6.3)	0	7 (8.8)
Grade 1	1 (7.1)	1 (11.1)	1 (4.8)	1 (3.1)	0	4 (5.0)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
White blood cell count decreased	2 (14.3)	2 (22.2)	3 (14.3)	0	0	7 (8.8)
Grade 1	1 (7.1)	1 (11.1)	2 (9.5)	0	0	4 (5.0)
Grade 2	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Weight decreased	0	2 (22.2)	0	3 (9.4)	0	5 (6.3)
Grade 1	0	1 (11.1)	0	2 (6.3)	0	3 (3.8)
Grade 2	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Gamma-glutamyltransferase increased	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Grade 1	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Blood creatinine increased	0	0	0	2 (6.3)	0	2 (2.5)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Haemoglobin decreased	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 1	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Neutrophil count decreased	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Troponin T increased	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Grade 1	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Amylase increased	0	0	1 (4.8)	0	0	1 (1.3)

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
Blood creatine phosphokinase MB increased	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Blood pressure increased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Blood urea increased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
C-reactive protein increased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Haptoglobin decreased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Intraocular pressure increased	0	0	1 (4.8)	0	0	1 (1.3)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Urine output decreased	0	0	1 (4.8)	0	0	1 (1.3)

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Weight increased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Metabolism and nutrition disorders	2 (14.3)	3 (33.3)	6 (28.6)	9 (28.1)	1 (25.0)	21 (26.3)
Grade 1	2 (14.3)	2 (22.2)	4 (19.0)	4 (12.5)	1 (25.0)	13 (16.3)
Grade 2	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Grade 3	0	1 (11.1)	1 (4.8)	3 (9.4)	0	5 (6.3)
Decreased appetite	0	1 (11.1)	5 (23.8)	7 (21.9)	1 (25.0)	14 (17.5)
Grade 1	0	1 (11.1)	4 (19.0)	4 (12.5)	1 (25.0)	10 (12.5)
Grade 2	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 3	0	0	0	2 (6.3)	0	2 (2.5)
Weight gain poor	0	1 (11.1)	2 (9.5)	0	0	3 (3.8)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
Feeding disorder	1 (7.1)	0	0	0	0	1 (1.3)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)
Hypercholesterolaemia	0	1 (11.1)	0	0	0	1 (1.3)

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Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Hypertriglyceridaemia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Hypoglycaemia	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Hypophagia	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Metabolic acidosis	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Underweight	1 (7.1)	0	0	0	0	1 (1.3)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)
Musculoskeletal and connective tissue disorders	0	2 (22.2)	2 (9.5)	1 (3.1)	0	5 (6.3)
Grade 1	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
Grade 2	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Scoliosis	0	2 (22.2)	1 (4.8)	1 (3.1)	0	4 (5.0)

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Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 1	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Joint range of motion decreased	0	0	1 (4.8)	0	0	1 (1.3)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
Kyphosis	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Muscle atrophy	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Nervous system disorders	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Cerebral atrophy	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Seizure	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)

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Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Renal and urinary disorders	0	0	0	4 (12.5)	0	4 (5.0)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	2 (6.3)	0	2 (2.5)
Haematuria	0	0	0	2 (6.3)	0	2 (2.5)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Proteinuria	0	0	0	2 (6.3)	0	2 (2.5)
Grade 3	0	0	0	2 (6.3)	0	2 (2.5)
Acute kidney injury	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Chronic kidney disease	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Glycosuria	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)

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Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Respiratory, thoracic and mediastinal disorders	5 (35.7)	2 (22.2)	1 (4.8)	4 (12.5)	0	12 (15.0)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)
Grade 3	1 (7.1)	1 (11.1)	1 (4.8)	2 (6.3)	0	5 (6.3)
Grade 4	3 (21.4)	1 (11.1)	0	2 (6.3)	0	6 (7.5)
Acute respiratory failure	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	2 (6.3)	0	3 (3.8)
Atelectasis	0	1 (11.1)	0	3 (9.4)	0	4 (5.0)
Grade 3	0	1 (11.1)	0	3 (9.4)	0	4 (5.0)
Respiratory failure	2 (14.3)	0	0	0	0	2 (2.5)
Grade 3	1 (7.1)	0	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Apnoea	1 (7.1)	0	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Hypoxia	1 (7.1)	0	0	0	0	1 (1.3)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)
Nocturnal dyspnoea	1 (7.1)	0	0	0	0	1 (1.3)

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Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)
Pleural effusion	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Respiration abnormal	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	0	1 (11.1)	0	0	0	1 (1.3)
Respiratory disorder	0	0	1 (4.8)	0	0	1 (1.3)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Sleep apnoea syndrome	0	1 (11.1)	0	0	0	1 (1.3)
Grade 2	0	1 (11.1)	0	0	0	1 (1.3)
Upper respiratory tract inflammation	1 (7.1)	0	0	0	0	1 (1.3)
Grade 1	1 (7.1)	0	0	0	0	1 (1.3)
Skin and subcutaneous tissue disorders	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Urticaria	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Vascular disorders	1 (7.1)	1 (11.1)	0	4 (12.5)	0	6 (7.5)

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Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	1 (7.1)	1 (11.1)	0	3 (9.4)	0	5 (6.3)
Kawasaki's disease	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Grade 3	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Hypertension	0	0	0	2 (6.3)	0	2 (2.5)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Not Coded	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Grade 2	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Acute liver disorder(Grade 4)	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Liver dysfunction(AST/ALT)	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Motor developmental delay due to inadequate effect of Zolgensma	1 (7.1)	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.1.oav.age3.jpn
OAV101 Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)
Respiratory tract infections with human metapneumovirus	0	1 (11.1)	0	0	0	1 (1.3)
Grade 3	0	1 (11.1)	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Any Adverse Event	13 (92.9)	9 (100)	21 (100)	32 (100)	4 (100)	79 (98.8)
Blood and lymphatic system disorders	0	1 (11.1)	1 (4.8)	10 (31.3)	1 (25.0)	13 (16.3)
Grade 1	0	0	0	5 (15.6)	1 (25.0)	6 (7.5)
Grade 3	0	1 (11.1)	1 (4.8)	3 (9.4)	0	5 (6.3)
Grade 4	0	0	0	2 (6.3)	0	2 (2.5)
Thrombocytopenia	0	1 (11.1)	1 (4.8)	6 (18.8)	1 (25.0)	9 (11.3)
Grade 1	0	0	0	5 (15.6)	1 (25.0)	6 (7.5)
Grade 3	0	1 (11.1)	1 (4.8)	1 (3.1)	0	3 (3.8)
Thrombotic microangiopathy	0	0	0	4 (12.5)	0	4 (5.0)
Grade 3	0	0	0	2 (6.3)	0	2 (2.5)
Grade 4	0	0	0	2 (6.3)	0	2 (2.5)
Haemolytic anaemia	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Cardiac disorders	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Grade 1	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Arrhythmia	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Bradycardia	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Gastrointestinal disorders	3 (21.4)	3 (33.3)	10 (47.6)	12 (37.5)	2 (50.0)	30 (37.5)
Grade 1	3 (21.4)	2 (22.2)	7 (33.3)	8 (25.0)	1 (25.0)	21 (26.3)
Grade 2	0	1 (11.1)	3 (14.3)	4 (12.5)	1 (25.0)	9 (11.3)
Vomiting	3 (21.4)	3 (33.3)	10 (47.6)	10 (31.3)	2 (50.0)	28 (35.0)
Grade 1	3 (21.4)	2 (22.2)	7 (33.3)	7 (21.9)	1 (25.0)	20 (25.0)
Grade 2	0	1 (11.1)	3 (14.3)	3 (9.4)	1 (25.0)	8 (10.0)
Nausea	0	0	1 (4.8)	3 (9.4)	1 (25.0)	5 (6.3)
Grade 1	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Grade 2	0	0	0	1 (3.1)	1 (25.0)	2 (2.5)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
General disorders and administration site conditions	9 (64.3)	6 (66.7)	19 (90.5)	26 (81.3)	4 (100)	64 (80.0)
Grade 1	9 (64.3)	4 (44.4)	12 (57.1)	17 (53.1)	4 (100)	46 (57.5)
Grade 2	0	2 (22.2)	5 (23.8)	8 (25.0)	0	15 (18.8)
Grade 3	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 4	0	0	1 (4.8)	0	0	1 (1.3)
Pyrexia	9 (64.3)	6 (66.7)	19 (90.5)	26 (81.3)	4 (100)	64 (80.0)
Grade 1	9 (64.3)	4 (44.4)	13 (61.9)	18 (56.3)	4 (100)	48 (60.0)
Grade 2	0	2 (22.2)	5 (23.8)	8 (25.0)	0	15 (18.8)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Asthenia	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Malaise	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Generalised oedema	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Multiple organ dysfunction syndrome	0	0	1 (4.8)	0	0	1 (1.3)
Grade 4	0	0	1 (4.8)	0	0	1 (1.3)
Hepatobiliary disorders	2 (14.3)	2 (22.2)	4 (19.0)	1 (3.1)	1 (25.0)	10 (12.5)
Grade 1	1 (7.1)	0	1 (4.8)	0	0	2 (2.5)
Grade 2	1 (7.1)	2 (22.2)	0	1 (3.1)	0	4 (5.0)
Grade 3	0	0	1 (4.8)	0	1 (25.0)	2 (2.5)
Grade 4	0	0	2 (9.5)	0	0	2 (2.5)
Hepatic function abnormal	2 (14.3)	2 (22.2)	4 (19.0)	1 (3.1)	1 (25.0)	10 (12.5)
Grade 1	1 (7.1)	0	2 (9.5)	0	0	3 (3.8)
Grade 2	1 (7.1)	2 (22.2)	0	1 (3.1)	0	4 (5.0)
Grade 3	0	0	1 (4.8)	0	1 (25.0)	2 (2.5)
Grade 4	0	0	1 (4.8)	0	0	1 (1.3)
Acute hepatic failure	0	0	1 (4.8)	0	0	1 (1.3)
Grade 4	0	0	1 (4.8)	0	0	1 (1.3)
Hepatomegaly	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Immune system disorders	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Hypersensitivity	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Infections and infestations	0	0	1 (4.8)	0	0	1 (1.3)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
Bronchitis	0	0	1 (4.8)	0	0	1 (1.3)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
Investigations	12 (85.7)	8 (88.9)	19 (90.5)	29 (90.6)	4 (100)	72 (90.0)
Grade 1	7 (50.0)	3 (33.3)	3 (14.3)	3 (9.4)	1 (25.0)	17 (21.3)
Grade 2	3 (21.4)	1 (11.1)	4 (19.0)	5 (15.6)	0	13 (16.3)
Grade 3	1 (7.1)	3 (33.3)	9 (42.9)	14 (43.8)	3 (75.0)	30 (37.5)
Grade 4	1 (7.1)	1 (11.1)	3 (14.3)	7 (21.9)	0	12 (15.0)
Aspartate aminotransferase increased	7 (50.0)	5 (55.6)	12 (57.1)	26 (81.3)	3 (75.0)	53 (66.3)
Grade 1	3 (21.4)	1 (11.1)	1 (4.8)	3 (9.4)	0	8 (10.0)
Grade 2	3 (21.4)	1 (11.1)	3 (14.3)	4 (12.5)	0	11 (13.8)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 3	1 (7.1)	2 (22.2)	6 (28.6)	16 (50.0)	3 (75.0)	28 (35.0)
Grade 4	0	1 (11.1)	2 (9.5)	3 (9.4)	0	6 (7.5)
Alanine aminotransferase increased	5 (35.7)	5 (55.6)	12 (57.1)	25 (78.1)	3 (75.0)	50 (62.5)
Grade 1	4 (28.6)	2 (22.2)	1 (4.8)	4 (12.5)	0	11 (13.8)
Grade 2	0	1 (11.1)	2 (9.5)	5 (15.6)	1 (25.0)	9 (11.3)
Grade 3	1 (7.1)	1 (11.1)	7 (33.3)	11 (34.4)	2 (50.0)	22 (27.5)
Grade 4	0	1 (11.1)	2 (9.5)	5 (15.6)	0	8 (10.0)
Platelet count decreased	3 (21.4)	5 (55.6)	10 (47.6)	19 (59.4)	3 (75.0)	40 (50.0)
Grade 1	2 (14.3)	5 (55.6)	8 (38.1)	7 (21.9)	1 (25.0)	23 (28.8)
Grade 2	1 (7.1)	0	0	8 (25.0)	1 (25.0)	10 (12.5)
Grade 3	0	0	2 (9.5)	2 (6.3)	1 (25.0)	5 (6.3)
Grade 4	0	0	0	2 (6.3)	0	2 (2.5)
Blood lactate dehydrogenase increased	0	4 (44.4)	6 (28.6)	10 (31.3)	1 (25.0)	21 (26.3)
Grade 1	0	4 (44.4)	5 (23.8)	10 (31.3)	1 (25.0)	20 (25.0)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
Serum ferritin increased	1 (7.1)	3 (33.3)	4 (19.0)	5 (15.6)	2 (50.0)	15 (18.8)
Grade 1	1 (7.1)	3 (33.3)	3 (14.3)	4 (12.5)	2 (50.0)	13 (16.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 2	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Troponin I increased	5 (35.7)	2 (22.2)	2 (9.5)	1 (3.1)	1 (25.0)	11 (13.8)
Grade 1	5 (35.7)	2 (22.2)	2 (9.5)	1 (3.1)	1 (25.0)	11 (13.8)
Blood creatine phosphokinase increased	1 (7.1)	1 (11.1)	1 (4.8)	3 (9.4)	1 (25.0)	7 (8.8)
Grade 1	1 (7.1)	1 (11.1)	1 (4.8)	2 (6.3)	1 (25.0)	6 (7.5)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Hepatic enzyme increased	1 (7.1)	2 (22.2)	2 (9.5)	2 (6.3)	0	7 (8.8)
Grade 1	1 (7.1)	1 (11.1)	1 (4.8)	1 (3.1)	0	4 (5.0)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
White blood cell count decreased	2 (14.3)	2 (22.2)	3 (14.3)	0	0	7 (8.8)
Grade 1	1 (7.1)	1 (11.1)	2 (9.5)	0	0	4 (5.0)
Grade 2	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Grade 3	0	0	1 (4.8)	0	0	1 (1.3)
Gamma-glutamyltransferase increased	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Grade 1	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Blood creatinine increased	0	0	0	2 (6.3)	0	2 (2.5)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Haemoglobin decreased	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 1	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Neutrophil count decreased	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Grade 4	1 (7.1)	0	0	0	0	1 (1.3)
Weight decreased	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	1 (11.1)	0	0	0	1 (1.3)
Amylase increased	0	0	1 (4.8)	0	0	1 (1.3)
Grade 2	0	0	1 (4.8)	0	0	1 (1.3)
Blood creatine phosphokinase MB increased	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Blood pressure increased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Blood urea increased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
C-reactive protein increased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Haptoglobin decreased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Troponin T increased	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Urine output decreased	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Weight increased	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Metabolism and nutrition disorders	0	2 (22.2)	4 (19.0)	8 (25.0)	1 (25.0)	15 (18.8)
Grade 1	0	2 (22.2)	3 (14.3)	4 (12.5)	1 (25.0)	10 (12.5)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 2	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Grade 3	0	0	0	2 (6.3)	0	2 (2.5)
Decreased appetite	0	1 (11.1)	4 (19.0)	7 (21.9)	1 (25.0)	13 (16.3)
Grade 1	0	1 (11.1)	3 (14.3)	4 (12.5)	1 (25.0)	9 (11.3)
Grade 2	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Grade 3	0	0	0	2 (6.3)	0	2 (2.5)
Hypercholesterolaemia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Hypertriglyceridaemia	0	1 (11.1)	0	0	0	1 (1.3)
Grade 1	0	1 (11.1)	0	0	0	1 (1.3)
Hypophagia	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Weight gain poor	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Nervous system disorders	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Cerebral atrophy	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Renal and urinary disorders	0	0	0	4 (12.5)	0	4 (5.0)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	2 (6.3)	0	2 (2.5)
Haematuria	0	0	0	2 (6.3)	0	2 (2.5)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Proteinuria	0	0	0	2 (6.3)	0	2 (2.5)
Grade 3	0	0	0	2 (6.3)	0	2 (2.5)
Acute kidney injury	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Chronic kidney disease	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Glycosuria	0	0	0	1 (3.1)	0	1 (1.3)
Grade 1	0	0	0	1 (3.1)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Respiratory, thoracic and mediastinal disorders	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Pleural effusion	0	0	1 (4.8)	0	0	1 (1.3)
Grade 1	0	0	1 (4.8)	0	0	1 (1.3)
Skin and subcutaneous tissue disorders	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Urticaria	0	0	0	0	1 (25.0)	1 (1.3)
Grade 1	0	0	0	0	1 (25.0)	1 (1.3)
Vascular disorders	0	0	0	2 (6.3)	0	2 (2.5)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Hypertension	0	0	0	2 (6.3)	0	2 (2.5)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.2.2.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Maximum Grade by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term Grade	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Grade 3	0	0	0	1 (3.1)	0	1 (1.3)
Not Coded	1 (7.1)	0	0	2 (6.3)	0	3 (3.8)
Grade 2	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Acute liver disorder(Grade 4)	0	0	0	1 (3.1)	0	1 (1.3)
Grade 4	0	0	0	1 (3.1)	0	1 (1.3)
Liver dysfunction(AST/ALT)	0	0	0	1 (3.1)	0	1 (1.3)
Grade 2	0	0	0	1 (3.1)	0	1 (1.3)
Motor developmental delay due to inadequate effect of Zolgensma	1 (7.1)	0	0	0	0	1 (1.3)
Grade 2	1 (7.1)	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.
Grade based on CTCAE version 4.03.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Any Serious Adverse Event	8 (57.1)	8 (88.9)	13 (61.9)	21 (65.6)	1 (25.0)	51 (63.8)
Blood and lymphatic system disorders	0	1 (11.1)	1 (4.8)	4 (12.5)	1 (25.0)	7 (8.8)
Thrombotic microangiopathy	0	0	0	4 (12.5)	0	4 (5.0)
Thrombocytopenia	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
Eosinophilia	0	0	0	0	1 (25.0)	1 (1.3)
Haemolytic anaemia	0	0	0	1 (3.1)	0	1 (1.3)
Cardiac disorders	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Cardiac failure congestive	0	0	0	1 (3.1)	0	1 (1.3)
Cardio-respiratory arrest	0	0	1 (4.8)	0	0	1 (1.3)
Congenital, familial and genetic disorders	2 (14.3)	0	1 (4.8)	1 (3.1)	0	4 (5.0)
Cryptorchism	2 (14.3)	0	1 (4.8)	1 (3.1)	0	4 (5.0)
Gastrointestinal disorders	2 (14.3)	1 (11.1)	3 (14.3)	1 (3.1)	0	7 (8.8)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Gastric fistula	1 (7.1)	0	3 (14.3)	0	0	4 (5.0)
Dysphagia	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Vomiting	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Constipation	1 (7.1)	0	0	0	0	1 (1.3)
General disorders and administration site conditions	0	1 (11.1)	1 (4.8)	2 (6.3)	0	4 (5.0)
Pyrexia	0	1 (11.1)	0	2 (6.3)	0	3 (3.8)
Inflammation	0	0	0	1 (3.1)	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (4.8)	0	0	1 (1.3)
Hepatobiliary disorders	0	0	2 (9.5)	0	0	2 (2.5)
Acute hepatic failure	0	0	1 (4.8)	0	0	1 (1.3)
Hepatic function abnormal	0	0	1 (4.8)	0	0	1 (1.3)
Infections and infestations	5 (35.7)	7 (77.8)	8 (38.1)	16 (50.0)	0	36 (45.0)
Pneumonia	2 (14.3)	3 (33.3)	1 (4.8)	5 (15.6)	0	11 (13.8)
Pneumonia aspiration	2 (14.3)	2 (22.2)	2 (9.5)	4 (12.5)	0	10 (12.5)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Bronchitis	0	1 (11.1)	3 (14.3)	3 (9.4)	0	7 (8.8)
Upper respiratory tract infection	2 (14.3)	1 (11.1)	2 (9.5)	2 (6.3)	0	7 (8.8)
COVID-19	2 (14.3)	2 (22.2)	2 (9.5)	0	0	6 (7.5)
Pneumonia respiratory syncytial viral	1 (7.1)	1 (11.1)	1 (4.8)	3 (9.4)	0	6 (7.5)
Respiratory syncytial virus infection	1 (7.1)	1 (11.1)	0	3 (9.4)	0	5 (6.3)
Respiratory syncytial virus bronchiolitis	0	0	2 (9.5)	1 (3.1)	0	3 (3.8)
Bronchitis viral	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Gastroenteritis	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Pneumonia influenzal	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Respiratory tract infection	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Rhinovirus infection	0	0	2 (9.5)	0	0	2 (2.5)
Viral upper respiratory tract infection	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Bronchiolitis	0	0	1 (4.8)	0	0	1 (1.3)
Gastroenteritis norovirus	0	0	1 (4.8)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Lower respiratory tract infection	0	0	0	1 (3.1)	0	1 (1.3)
Metapneumovirus bronchiolitis	0	0	0	1 (3.1)	0	1 (1.3)
Metapneumovirus infection	0	0	1 (4.8)	0	0	1 (1.3)
Metapneumovirus pneumonia	0	1 (11.1)	0	0	0	1 (1.3)
Norovirus infection	0	0	1 (4.8)	0	0	1 (1.3)
Parainfluenzae virus infection	0	0	1 (4.8)	0	0	1 (1.3)
Pharyngitis	0	1 (11.1)	0	0	0	1 (1.3)
Pneumonia parainfluenzae viral	1 (7.1)	0	0	0	0	1 (1.3)
Pneumonia viral	0	0	1 (4.8)	0	0	1 (1.3)
Tonsillitis	0	0	1 (4.8)	0	0	1 (1.3)
Viral infection	0	1 (11.1)	0	0	0	1 (1.3)
Injury, poisoning and procedural complications	1 (7.1)	0	0	1 (3.1)	0	2 (2.5)
Femur fracture	1 (7.1)	0	0	0	0	1 (1.3)
Joint dislocation	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Investigations	1 (7.1)	2 (22.2)	2 (9.5)	7 (21.9)	0	12 (15.0)
Aspartate aminotransferase increased	1 (7.1)	2 (22.2)	1 (4.8)	5 (15.6)	0	9 (11.3)
Alanine aminotransferase increased	0	1 (11.1)	0	5 (15.6)	0	6 (7.5)
Platelet count decreased	0	0	0	2 (6.3)	0	2 (2.5)
Hepatic enzyme increased	0	0	1 (4.8)	0	0	1 (1.3)
Serum ferritin increased	0	0	0	1 (3.1)	0	1 (1.3)
Weight decreased	0	0	0	1 (3.1)	0	1 (1.3)
Metabolism and nutrition disorders	1 (7.1)	1 (11.1)	1 (4.8)	1 (3.1)	0	4 (5.0)
Weight gain poor	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
Feeding disorder	1 (7.1)	0	0	0	0	1 (1.3)
Hypoglycaemia	0	0	0	1 (3.1)	0	1 (1.3)
Musculoskeletal and connective tissue disorders	0	1 (11.1)	0	0	0	1 (1.3)
Kyphosis	0	1 (11.1)	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Scoliosis	0	1 (11.1)	0	0	0	1 (1.3)
Renal and urinary disorders	0	0	0	3 (9.4)	0	3 (3.8)
Acute kidney injury	0	0	0	1 (3.1)	0	1 (1.3)
Haematuria	0	0	0	1 (3.1)	0	1 (1.3)
Proteinuria	0	0	0	1 (3.1)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.1)	0	1 (1.3)
Respiratory, thoracic and mediastinal disorders	4 (28.6)	2 (22.2)	1 (4.8)	4 (12.5)	0	11 (13.8)
Acute respiratory failure	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Atelectasis	0	1 (11.1)	0	3 (9.4)	0	4 (5.0)
Respiratory failure	2 (14.3)	0	0	0	0	2 (2.5)
Apnoea	1 (7.1)	0	0	0	0	1 (1.3)
Hypoxia	1 (7.1)	0	0	0	0	1 (1.3)
Nocturnal dyspnoea	1 (7.1)	0	0	0	0	1 (1.3)
Respiration abnormal	0	1 (11.1)	0	0	0	1 (1.3)
Respiratory disorder	0	0	1 (4.8)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.3.oav.age3.jpn
Serious OAV101 Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Vascular disorders	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Kawasaki's disease	1 (7.1)	1 (11.1)	0	2 (6.3)	0	4 (5.0)
Not Coded	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Acute liver disorder(Grade 4)	0	0	0	1 (3.1)	0	1 (1.3)
Respiratory tract infections with human metapneumovirus	0	1 (11.1)	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Any OAV101 Related Adverse Event	13 (92.9)	9 (100)	21 (100)	32 (100)	4 (100)	79 (98.8)
Blood and lymphatic system disorders	0	1 (11.1)	1 (4.8)	10 (31.3)	1 (25.0)	13 (16.3)
Thrombocytopenia	0	1 (11.1)	1 (4.8)	6 (18.8)	1 (25.0)	9 (11.3)
Thrombotic microangiopathy	0	0	0	4 (12.5)	0	4 (5.0)
Haemolytic anaemia	0	0	0	1 (3.1)	0	1 (1.3)
Cardiac disorders	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Arrhythmia	0	0	0	1 (3.1)	0	1 (1.3)
Bradycardia	0	0	1 (4.8)	0	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.1)	0	1 (1.3)
Gastrointestinal disorders	3 (21.4)	3 (33.3)	10 (47.6)	12 (37.5)	2 (50.0)	30 (37.5)
Vomiting	3 (21.4)	3 (33.3)	10 (47.6)	10 (31.3)	2 (50.0)	28 (35.0)
Nausea	0	0	1 (4.8)	3 (9.4)	1 (25.0)	5 (6.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
General disorders and administration site conditions	9 (64.3)	6 (66.7)	19 (90.5)	26 (81.3)	4 (100)	64 (80.0)
Pyrexia	9 (64.3)	6 (66.7)	19 (90.5)	26 (81.3)	4 (100)	64 (80.0)
Asthenia	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Malaise	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
Generalised oedema	0	0	0	1 (3.1)	0	1 (1.3)
Multiple organ dysfunction syndrome	0	0	1 (4.8)	0	0	1 (1.3)
Hepatobiliary disorders	2 (14.3)	2 (22.2)	4 (19.0)	1 (3.1)	1 (25.0)	10 (12.5)
Hepatic function abnormal	2 (14.3)	2 (22.2)	4 (19.0)	1 (3.1)	1 (25.0)	10 (12.5)
Acute hepatic failure	0	0	1 (4.8)	0	0	1 (1.3)
Hepatomegaly	0	0	0	0	1 (25.0)	1 (1.3)
Immune system disorders	0	0	0	1 (3.1)	0	1 (1.3)
Hypersensitivity	0	0	0	1 (3.1)	0	1 (1.3)
Infections and infestations	0	0	1 (4.8)	0	0	1 (1.3)
Bronchitis	0	0	1 (4.8)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Investigations	12 (85.7)	8 (88.9)	19 (90.5)	29 (90.6)	4 (100)	72 (90.0)
Aspartate aminotransferase increased	7 (50.0)	5 (55.6)	12 (57.1)	26 (81.3)	3 (75.0)	53 (66.3)
Alanine aminotransferase increased	5 (35.7)	5 (55.6)	12 (57.1)	25 (78.1)	3 (75.0)	50 (62.5)
Platelet count decreased	3 (21.4)	5 (55.6)	10 (47.6)	19 (59.4)	3 (75.0)	40 (50.0)
Blood lactate dehydrogenase increased	0	4 (44.4)	6 (28.6)	10 (31.3)	1 (25.0)	21 (26.3)
Serum ferritin increased	1 (7.1)	3 (33.3)	4 (19.0)	5 (15.6)	2 (50.0)	15 (18.8)
Troponin I increased	5 (35.7)	2 (22.2)	2 (9.5)	1 (3.1)	1 (25.0)	11 (13.8)
Blood creatine phosphokinase increased	1 (7.1)	1 (11.1)	1 (4.8)	3 (9.4)	1 (25.0)	7 (8.8)
Hepatic enzyme increased	1 (7.1)	2 (22.2)	2 (9.5)	2 (6.3)	0	7 (8.8)
White blood cell count decreased	2 (14.3)	2 (22.2)	3 (14.3)	0	0	7 (8.8)
Gamma-glutamyltransferase increased	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Blood creatinine increased	0	0	0	2 (6.3)	0	2 (2.5)
Haemoglobin decreased	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Neutrophil count decreased	1 (7.1)	1 (11.1)	0	0	0	2 (2.5)
Weight decreased	0	1 (11.1)	0	1 (3.1)	0	2 (2.5)
Amylase increased	0	0	1 (4.8)	0	0	1 (1.3)
Blood creatine phosphokinase MB increased	0	0	1 (4.8)	0	0	1 (1.3)
Blood pressure increased	0	0	0	1 (3.1)	0	1 (1.3)
Blood urea increased	0	0	0	1 (3.1)	0	1 (1.3)
C-reactive protein increased	0	0	0	1 (3.1)	0	1 (1.3)
Haptoglobin decreased	0	0	0	1 (3.1)	0	1 (1.3)
N-terminal prohormone brain natriuretic peptide increased	0	0	1 (4.8)	0	0	1 (1.3)
Troponin T increased	0	1 (11.1)	0	0	0	1 (1.3)
Urine output decreased	0	0	1 (4.8)	0	0	1 (1.3)
Weight increased	0	0	0	1 (3.1)	0	1 (1.3)
Metabolism and nutrition disorders	0	2 (22.2)	4 (19.0)	8 (25.0)	1 (25.0)	15 (18.8)
Decreased appetite	0	1 (11.1)	4 (19.0)	7 (21.9)	1 (25.0)	13 (16.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Hypercholesterolaemia	0	1 (11.1)	0	0	0	1 (1.3)
Hypertriglyceridaemia	0	1 (11.1)	0	0	0	1 (1.3)
Hypophagia	0	0	0	1 (3.1)	0	1 (1.3)
Weight gain poor	0	0	1 (4.8)	0	0	1 (1.3)
Nervous system disorders	0	0	0	1 (3.1)	0	1 (1.3)
Cerebral atrophy	0	0	0	1 (3.1)	0	1 (1.3)
Renal and urinary disorders	0	0	0	4 (12.5)	0	4 (5.0)
Haematuria	0	0	0	2 (6.3)	0	2 (2.5)
Proteinuria	0	0	0	2 (6.3)	0	2 (2.5)
Acute kidney injury	0	0	0	1 (3.1)	0	1 (1.3)
Chronic kidney disease	0	0	0	1 (3.1)	0	1 (1.3)
Glycosuria	0	0	0	1 (3.1)	0	1 (1.3)
Renal impairment	0	0	0	1 (3.1)	0	1 (1.3)
Respiratory, thoracic and mediastinal disorders	0	0	1 (4.8)	0	0	1 (1.3)
Pleural effusion	0	0	1 (4.8)	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.4.oav.age3.jpn
OAV101 Related Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Skin and subcutaneous tissue disorders	0	0	0	0	1 (25.0)	1 (1.3)
Urticaria	0	0	0	0	1 (25.0)	1 (1.3)
Vascular disorders	0	0	0	2 (6.3)	0	2 (2.5)
Hypertension	0	0	0	2 (6.3)	0	2 (2.5)
Not Coded	1 (7.1)	0	0	2 (6.3)	0	3 (3.8)
Acute liver disorder(Grade 4)	0	0	0	1 (3.1)	0	1 (1.3)
Liver dysfunction(AST/ALT)	0	0	0	1 (3.1)	0	1 (1.3)
Motor developmental delay due to inadequate effect of Zolgensma	1 (7.1)	0	0	0	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.5.oav.age3.jpn
OAV101 Related Serious Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Any Related Serious Adverse Event	1 (7.1)	2 (22.2)	5 (23.8)	10 (31.3)	0	18 (22.5)
Blood and lymphatic system disorders	0	1 (11.1)	1 (4.8)	4 (12.5)	0	6 (7.5)
Thrombotic microangiopathy	0	0	0	4 (12.5)	0	4 (5.0)
Thrombocytopenia	0	1 (11.1)	1 (4.8)	0	0	2 (2.5)
Haemolytic anaemia	0	0	0	1 (3.1)	0	1 (1.3)
Cardiac disorders	0	0	0	1 (3.1)	0	1 (1.3)
Cardiac failure congestive	0	0	0	1 (3.1)	0	1 (1.3)
Gastrointestinal disorders	0	0	0	1 (3.1)	0	1 (1.3)
Vomiting	0	0	0	1 (3.1)	0	1 (1.3)
General disorders and administration site conditions	0	0	1 (4.8)	2 (6.3)	0	3 (3.8)
Pyrexia	0	0	0	2 (6.3)	0	2 (2.5)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.5.oav.age3.jpn
OAV101 Related Serious Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Multiple organ dysfunction syndrome	0	0	1 (4.8)	0	0	1 (1.3)
Hepatobiliary disorders	0	0	2 (9.5)	0	0	2 (2.5)
Acute hepatic failure	0	0	1 (4.8)	0	0	1 (1.3)
Hepatic function abnormal	0	0	1 (4.8)	0	0	1 (1.3)
Investigations	1 (7.1)	2 (22.2)	2 (9.5)	7 (21.9)	0	12 (15.0)
Aspartate aminotransferase increased	1 (7.1)	2 (22.2)	1 (4.8)	5 (15.6)	0	9 (11.3)
Alanine aminotransferase increased	0	1 (11.1)	0	5 (15.6)	0	6 (7.5)
Platelet count decreased	0	0	0	2 (6.3)	0	2 (2.5)
Hepatic enzyme increased	0	0	1 (4.8)	0	0	1 (1.3)
Serum ferritin increased	0	0	0	1 (3.1)	0	1 (1.3)
Renal and urinary disorders	0	0	0	3 (9.4)	0	3 (3.8)
Acute kidney injury	0	0	0	1 (3.1)	0	1 (1.3)
Haematuria	0	0	0	1 (3.1)	0	1 (1.3)
Proteinuria	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.5.oav.age3.jpn
OAV101 Related Serious Treatment Emergent Adverse Events by Age at OAV101 Infusion
Japan Safety Analysis Set

SOC Preferred Term	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Renal impairment	0	0	0	1 (3.1)	0	1 (1.3)
Not Coded	0	0	0	1 (3.1)	0	1 (1.3)
Acute liver disorder(Grade 4)	0	0	0	1 (3.1)	0	1 (1.3)

Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Percentages are based on the number of patients who had at least one Adverse Event Form completed.
Uncoded events have not yet been mapped within MedDRA, and the verbatim term is given in place of the preferred term.

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Hepatotoxicity[a]						
n (%)	11 (78.6)	8 (88.9)	18 (85.7)	29 (90.6)	4 (100)	70 (87.5)
n / person-year	0.34	0.32	0.35	0.29	0.27	0.31
Transient Thrombocytopenia						
n (%)	3 (21.4)	6 (66.7)	11 (52.4)	26 (81.3)	4 (100)	50 (62.5)
n / person-year	0.09	0.24	0.21	0.26	0.27	0.22

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Thrombotic microangiopathy						
n (%)	0	0	0	4 (12.5)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.04	N/A	0.02
Cardiac Adverse Events						
n (%)	5 (35.7)	4 (44.4)	6 (28.6)	8 (25.0)	3 (75.0)	26 (32.5)
n / person-year	0.15	0.16	0.12	0.08	0.20	0.12

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Sensory Abnormalities Suggestive of Ganglionopathy						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New malignancies						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
New incidence of neurological disorders						
n (%)	0	0	1 (4.8)	1 (3.1)	0	2 (2.5)
n / person-year	N/A	N/A	0.02	0.01	N/A	0.01
New incidence of autoimmune disorders						
n (%)	0	0	0	1 (3.1)	0	1 (1.3)
n / person-year	N/A	N/A	N/A	0.01	N/A	0.00

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.1.age3.jpn
Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
New incidence of hematological disorders						
n (%)	0	1 (11.1)	1 (4.8)	10 (31.3)	1 (25.0)	13 (16.3)
n / person-year	N/A	0.04	0.02	0.10	0.07	0.06

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.2.age3.jpn
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Hepatotoxicity[a]						
n (%)	10 (71.4)	8 (88.9)	18 (85.7)	29 (90.6)	4 (100)	69 (86.3)
n / person-year	0.31	0.32	0.35	0.29	0.27	0.31
Transient Thrombocytopenia						
n (%)	3 (21.4)	6 (66.7)	11 (52.4)	26 (81.3)	4 (100)	50 (62.5)
n / person-year	0.09	0.24	0.21	0.26	0.27	0.22

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.2.age3.jpn
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Thrombotic microangiopathy						
n (%)	0	0	0	4 (12.5)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.04	N/A	0.02
Cardiac Adverse Events						
n (%)	5 (35.7)	4 (44.4)	5 (23.8)	8 (25.0)	3 (75.0)	25 (31.3)
n / person-year	0.15	0.16	0.10	0.08	0.20	0.11

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.2.age3.jpn
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Sensory Abnormalities Suggestive of Ganglionopathy						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New malignancies						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.2.age3.jpn
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
New incidence of neurological disorders						
n (%)	0	0	0	1 (3.1)	0	1 (1.3)
n / person-year	N/A	N/A	N/A	0.01	N/A	0.00
New incidence of autoimmune disorders						
n (%)	0	0	0	1 (3.1)	0	1 (1.3)
n / person-year	N/A	N/A	N/A	0.01	N/A	0.00

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.2.age3.jpn
OAV101 Related Treatment Emergent Adverse Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
New incidence of hematological disorders						
n (%)	0	1 (11.1)	1 (4.8)	10 (31.3)	1 (25.0)	13 (16.3)
n / person-year	N/A	0.04	0.02	0.10	0.07	0.06

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Hepatotoxicity[a]						
n (%)	1 (7.1)	2 (22.2)	4 (19.0)	5 (15.6)	0	12 (15.0)
n / person-year	0.03	0.08	0.08	0.05	N/A	0.05
Transient Thrombocytopenia						
n (%)	0	1 (11.1)	1 (4.8)	3 (9.4)	0	5 (6.3)
n / person-year	N/A	0.04	0.02	0.03	N/A	0.02

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Thrombotic microangiopathy						
n (%)	0	0	0	4 (12.5)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.04	N/A	0.02
Cardiac Adverse Events						
n (%)	1 (7.1)	0	1 (4.8)	4 (12.5)	0	6 (7.5)
n / person-year	0.03	N/A	0.02	0.04	N/A	0.03

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Sensory Abnormalities Suggestive of Ganglionopathy						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New malignancies						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
New incidence of neurological disorders						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New incidence of autoimmune disorders						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.3.age3.jpn
Serious Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
New incidence of hematological disorders						
n (%)	0	1 (11.1)	1 (4.8)	4 (12.5)	1 (25.0)	7 (8.8)
n / person-year	N/A	0.04	0.02	0.04	0.07	0.03

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.4.age3.jpn
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Hepatotoxicity[a]						
n (%)	1 (7.1)	2 (22.2)	4 (19.0)	5 (15.6)	0	12 (15.0)
n / person-year	0.03	0.08	0.08	0.05	N/A	0.05
Transient Thrombocytopenia						
n (%)	0	1 (11.1)	1 (4.8)	3 (9.4)	0	5 (6.3)
n / person-year	N/A	0.04	0.02	0.03	N/A	0.02

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.4.age3.jpn
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Thrombotic microangiopathy						
n (%)	0	0	0	4 (12.5)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.04	N/A	0.02
Cardiac Adverse Events						
n (%)	0	0	0	4 (12.5)	0	4 (5.0)
n / person-year	N/A	N/A	N/A	0.04	N/A	0.02

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.4.age3.jpn
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Sensory Abnormalities Suggestive of Ganglionopathy						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New malignancies						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.4.age3.jpn
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
New incidence of neurological disorders						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A
New incidence of autoimmune disorders						
n (%)	0	0	0	0	0	0
n / person-year	N/A	N/A	N/A	N/A	N/A	N/A

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.1.6.4.age3.jpn
OAV101 Serious Related Events of Special Interest by Age at OAV101 Infusion
Japan Safety Analysis Set

AESI Category	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
New incidence of hematological disorders						
n (%)	0	1 (11.1)	1 (4.8)	4 (12.5)	0	6 (7.5)
n / person-year	N/A	0.04	0.02	0.04	N/A	0.03

Note: 'N' is number of patients that had AE or Lab records and used as the denominator for calculating percent.
Adverse Events were coded using MedDRA, version 27.0.
Patients are only counted once at each level of summarization.
Treatment emergent adverse events are defined as all adverse events with onset date on or after the date of the OAV101 infusion.
[a] Includes hepatotoxicity AEs or hepatotoxicity from labs [ALT or AST >3 x upper limit of normal (ULN) with associated bilirubin >2 x ULN].

Table 14.3.2.age3.jpn
Clinically Significant Abnormal Laboratory Results any Time During Follow-up by Age at OAV101 Infusion
Japan Safety Analysis Set

Laboratory Test	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Albumin						
n	1	5	14	16	3	39
Yes	0	0	0	2 (12.5)	0	2 (5.1)
No	1 (100)	5 (100)	14 (100)	14 (87.5)	3 (100)	37 (94.9)
Aspartate aminotransferase (AST)						
n	7	8	20	27	3	65
Yes	6 (85.7)	5 (62.5)	13 (65.0)	22 (81.5)	3 (100)	49 (75.4)
No	1 (14.3)	3 (37.5)	7 (35.0)	5 (18.5)	0	16 (24.6)
Alanine aminotransferase (ALT)						
n	6	8	20	26	3	63
Yes	5 (83.3)	4 (50.0)	14 (70.0)	22 (84.6)	3 (100)	48 (76.2)
No	1 (16.7)	4 (50.0)	6 (30.0)	4 (15.4)	0	15 (23.8)
Alkaline phosphatase						
n	0	4	13	12	3	32
Yes		0	0	0	0	0
No		4 (100)	13 (100)	12 (100)	3 (100)	32 (100)

Note: Percent is based on the non-missing responses for each laboratory parameter.

Table 14.3.2.age3.jpn
Clinically Significant Abnormal Laboratory Results any Time During Follow-up by Age at OAV101 Infusion
Japan Safety Analysis Set

Laboratory Test	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
Total Bilirubin						
n	1	6	14	15	3	39
Yes	0	0	0	0	0	0
No	1 (100)	6 (100)	14 (100)	15 (100)	3 (100)	39 (100)
Direct Bilirubin						
n	1	3	4	5	1	14
Yes	0	0	0	0	0	0
No	1 (100)	3 (100)	4 (100)	5 (100)	1 (100)	14 (100)
Total Protein						
n	1	6	15	15	3	40
Yes	0	0	0	2 (13.3)	0	2 (5.0)
No	1 (100)	6 (100)	15 (100)	13 (86.7)	3 (100)	38 (95.0)
Platelets						
n	2	7	17	21	3	50
Yes	0	3 (42.9)	7 (41.2)	11 (52.4)	3 (100)	24 (48.0)
No	2 (100)	4 (57.1)	10 (58.8)	10 (47.6)	0	26 (52.0)

Note: Percent is based on the non-missing responses for each laboratory parameter.

Table 14.3.2.age3.jpn
Clinically Significant Abnormal Laboratory Results any Time During Follow-up by Age at OAV101 Infusion
Japan Safety Analysis Set

Laboratory Test	< 3 months (N=14)	≥ 3 and < 6 months (N=9)	≥ 6 and < 12 months (N=21)	≥ 12 and < 24 months (N=32)	≥ 24 months (N=4)	Total (N=80)
White Blood Count (WBC)						
n	2	8	16	17	3	46
Yes	1 (50.0)	3 (37.5)	3 (18.8)	3 (17.6)	0	10 (21.7)
No	1 (50.0)	5 (62.5)	13 (81.3)	14 (82.4)	3 (100)	36 (78.3)
Red Blood Count (RBC)						
n	1	6	15	17	3	42
Yes	0	1 (16.7)	0	1 (5.9)	0	2 (4.8)
No	1 (100)	5 (83.3)	15 (100)	16 (94.1)	3 (100)	40 (95.2)
Hemoglobin						
n	1	6	15	17	3	42
Yes	0	1 (16.7)	0	3 (17.6)	0	4 (9.5)
No	1 (100)	5 (83.3)	15 (100)	14 (82.4)	3 (100)	38 (90.5)
Hematocrit (HCT)						
n	1	6	14	15	3	39
Yes	0	1 (16.7)	0	2 (13.3)	0	3 (7.7)
No	1 (100)	5 (83.3)	14 (100)	13 (86.7)	3 (100)	36 (92.3)

Note: Percent is based on the non-missing responses for each laboratory parameter.

Table 14.3.3.jpn
Patients with Elevated AST or ALT After OAV101 Infusion
Japan Safety Analysis Set

Elevated AST or ALT	OAV101 mono (N=25)	Add-on (N=1)	Transient add-on (N=1)	Combo w/OAV101 (N=7)	Bridge to OAV101 (N=30)	Switch to OAV101 (N=16)	Total OAV101 (N=80)
Within 3 Months of OAV101 Infusion	22 (88.0)	1 (100)	1 (100)	6 (85.7)	26 (86.7)	16 (100)	72 (90.0)
> 3 Months to 6 Months after OAV101 Infusion	8 (32.0)	0	0	1 (14.3)	3 (10.0)	2 (12.5)	14 (17.5)
> 6 Months to 12 Months after OAV101 Infusion	2 (8.0)	0	0	1 (14.3)	3 (10.0)	8 (50.0)	14 (17.5)
> 12 Months to 18 Months after OAV101 Infusion	1 (4.0)	0	0	1 (14.3)	2 (6.7)	0	4 (5.0)
At Any Time Post OAV101 Infusion	22 (88.0)	1 (100)	1 (100)	7 (100)	26 (86.7)	16 (100)	73 (91.3)

A patient may be reported in multiple time frames.

安全性検討事項の各リスクの定義

安全性検討事項	定義
Hepatotoxicity	肝障害 (Broad) (SMQ)
Transient thrombocytopenia	出血 (Broad) (SMQ)
	造血障害による血小板減少症 (Broad) (SMQ)
	血小板障害ＮＥＣ (HLT)
Thrombotic microangiopathy	血栓性微小血管症 (PT)
	溶血性尿毒症症候群 (PT)
	非定型溶血性尿毒症症候群 (PT)
Cardiac events	心筋症 (Broad) (SMQ)
	虚血性心疾患 (Broad) (SMQ)
	不整脈 (Broad) (SMQ)
	塞栓および血栓 (Broad) (SMQ)
	心筋梗塞 (Broad) (SMQ)
	心不全 (Broad) (SMQ)
	高血圧 (Broad) (SMQ)
Sensory Abnormalities Suggestive of Ganglionopathy	運動感覚消失 (PT)
	異痛症 (PT)
	無感覚 (PT)
	有痛性感覚消失 (PT)
	反射消失 (PT)
	灼熱足症候群 (PT)
	灼熱感 (PT)
	中枢痛症候群 (PT)
	複合性局所疼痛症候群 (PT)
	振動覚低下 (PT)
	不快感 (PT)
	異常感覚 (PT)
	蟻走感 (PT)
	知覚過敏 (PT)
	ヒペルパチー (PT)
	感覚鈍麻 (PT)
	反射減弱 (PT)
	刺激反応低下 (PT)
	肋間神経痛 (PT)
	関節位置覚低下 (PT)
	固有感覚の欠如 (PT)
	神経伝導検査異常 (PT)
	神経刺激検査異常 (PT)
	神経痛 (PT)
	神経炎 (PT)
	神経学的症状 (PT)
	神経筋痛 (PT)
	背部異常感覚 (PT)
	疼痛 (PT)
	痛覚閾値低下 (PT)
	逆説疼痛 (PT)
	錯感覚 (PT)
	末梢神経系機能検査異常 (PT)
	末梢性感覚運動ニューロパチー (PT)
	末梢性感覚ニューロパチー (PT)
	神経根痛 (PT)
	反射試験異常 (PT)
	反射異常 (PT)
	熱感－冷感逆転 (PT)
	感覚運動障害 (PT)
	感覚障害 (PT)
	感覚神経節炎 (PT)
	感覚統合機能障害 (PT)
	感覚レベル異常 (PT)
	感覚消失 (PT)
	皮膚灼熱感 (PT)
	刺激反応遅滞 (PT)
	共感覚 (PT)
	温度覚検査値低下 (PT)
	圧痛 (PT)
	温度覚消失 (PT)
	温度覚鈍麻 (PT)
	ティネル徴候 (PT)
	毛髪痛 (PT)
	刺激無反応 (PT)
	振動覚亢進 (PT)
New malignancies	良性、悪性および詳細不明の新生物（嚢胞およびポリープを含む） (SOC)
New incidence of neurological disorders	神経系障害 (SOC)
New incidence of autoimmune disorders	免疫系障害 (SOC)
New incidence of hematological disorders	血液およびリンパ系障害 (SOC)

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