Cover Page for Statistical Analysis Plan

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PROTOCOL SMA-002

Long Term Safety Study of Amifampridine Phosphate in Ambulatory Patients with Spinal Muscular Atrophy (SMA) Type 3

STATISTICAL ANALYSIS PLAN

Version 1.0

29 June 2020

CONFIDENTIAL

Submitted to:

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1 Statistical Analysis Plan Signature Form

Statistical Analysis Plan Final Version 1.0 (Dated 29 June 2020) for SMA-002 Study.

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Upon review of this document, the undersigned approves this version of the Statistical Analysis Plan, authorizing that the content is acceptable for the reporting of this study.

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2 Modification History

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1.0	29JUN2020	Luisa D'Alonzo	Approved version

3 List of Abbreviations and Definitions of Terms

Abbreviation	Definition
Abs	Antibodies
Ach	Acetylcholine
AChR	Acetylcholine receptor
ADL	Activities of Daily Living
AE(s)	adverse event(s)
ALT	alanine aminotransferase
AST	aspartate aminotransferase
ATU	Autorisations Temporaires d'Utilisation Normative
CFB	Change from baseline
CM	Centimeter(s)
CMS	congenital myasthenia syndromes
CRA(s)	clinical research associate(s)
CRF	case report form
CRO	contract research organization
CTFG	Clinical Trial Facilitation Group
DBP	diastolic blood pressure
ECG(s)	electrocardiogram(s)
eCRF	electronic case report form
FDA	Food and Drug Administration
GCP	Good Clinical Practice
HCG	human chorionic gonadotropin
ICF	informed consent form
ICH	International Conference on Harmonisation of Technical Requirements for Registration of Pharmaceuticals for Human Use
ICH E6	ICH Harmonised Tripartite Guideline: Guideline for Good Clinical Practice E6
IEC	independent ethics committee
INQoL	Individual quality of life
IRB	institutional review board
ITT	Intent-to-treat
K+	potassium ion
kg	kilogram
LEMS	Lambert-Eaton myasthenic syndrome
MedDRA	Medical Dictionary for Regulatory Activities

Abbreviation	Definition
mg	milligram
MG	Myasthenia Gravis
MI	myocardial infarction
mmHg	millimeters of mercury
MS	multiple sclerosis
MuSK	muscle-specific receptor tyrosine kinase
MuSK-MG	muscle-specific receptor tyrosine Kinase-Myasthenia Gravis
NMJ	neuromuscular junction
PEDSQL™	pediatric quality of life inventory
PI	Principal Investigator
PK	pharmacokinetic
QoL	Quality of life
REB	research ethics board
SAE(s)	serious adverse event(s)
SAP	statistical analysis plan
SAS	Statistical Analysis System (software)
SBP	systolic blood pressure
SMA	spinal muscular atrophy
TEAE(s)	treatment emergent adverse event(s)
TESAE(s)	treatment emergent serious adverse event(s)
WHO-DD	World Health Organization – Drug Dictionary

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4 Introduction

This Statistical Analysis Plan (SAP) is based on study procedures and analyses from the protocol, Version 2.0, dated 24 July 2018. This SAP takes as well into account the following protocol version/amendment:

- Version 3.0, dated 12 April 2019 (amendment)

Table shells and mock listings corresponding to the contents of this document will be prepared and included with the final version. This document, with table shells and mock listings, will be reviewed prior to final analyses, and revised if necessary.

4.1 Background

Information on disease background, amifampridine, previously completed nonclinical and clinical studies of amifampridine, is described in the Introduction of SMA-001 and in the Investigator Brochure supplied by Catalyst (March 2019). Investigators are to review this document before initiating this study.

Amifampridine is a non-specific voltage-dependent potassium (K+) channel blocker. Blockade of K+ channels causes depolarization of the presynaptic membrane and slows down or inhibits repolarization. Prolonged depolarization results in opening of slow voltage-dependent calcium (Ca2+) channels and allows a subsequent influx of Ca2+. The increased concentration of intracellular Ca2+ induces exocytosis of the synaptic vesicles containing acetylcholine (ACh), thus releasing an increased level of ACh into the synaptic cleft (Maddison, 1998a; Maddison, 1998b). The influx of ACh into the presynaptic cleft enhances neuromuscular transmission, providing improved muscle function.

Mori et al. (2012) found that amifampridine improved neuromuscular transmission by predominantly increasing ACh release in the neuromuscular junction of in mice with MG induced by antibodies against muscle-specific kinase (MuSK). Furthermore, a mouse model of MuSK-myasthenia gravis (MG), amifampridine significantly enhanced neuromuscular transmission after 1 week of treatment without exacerbating loss of endplate AChRs (Morsch et al. 2013).

Multiple studies were conducted to evaluate the cardiac safety, pharmacokinetics and toxicity profile in rats and dogs. Reproductive and developmental toxicity studies with amifampridine were also conducted in rats and rabbit as well a variety of in vitro and in vivo genotoxicity studies, and in vitro metabolism/transporter studies. The main nonclinical findings related to amifampridine were central and autonomic nervous system effects, the development of Schwannomas, and histologic changes in muscle tissues. Below is an abbreviated summary of the effectiveness of amifampridine in MuSK-MG and overall safety in other patient populations.

Spinal Muscular Atrophy (SMA), first described in the 1890s, is an autosomal recessive neuromuscular disease characterized by degeneration of alpha motor neurons in the anterior horn of spinal cord, leading to progressive denervation atrophy in the involved skeletal muscles, with weakness and paralysis. The disease is due to defects in the Survival Motor Neuron 1 (SMN1) gene in chromosome 5, with deficiency in the SMN protein, a ubiquitously expressed protein, critical to the health and survival of the motor neurons.

Recent studies in SMA animal models and SMA patients have shown that the NMJ displays significant structural and functional defects that precede overt disease symptoms, suggesting that impaired NMJ function may contribute to SMA pathogenesis and symptoms. Defects in the NMJ appear to precede degeneration of motor neurons suggesting that abnormal formation and/or maintenance of this structure may be a key event in disease pathogenesis. These defects have been observed at both the pre- and postsynaptic components of the NMJ, which likely contribute to the failure to maintain the NMJ and

muscle innervation in mouse models of SMA. Finally, NMJ dysfunction has been also demonstrated in SMA patients, mainly in Type 3 subgroup, contributing to the symptoms associated with the disease.

Small studies have demonstrated the beneficial effects of amifampridine in patients with muscle-specific receptor tyrosine kinase-myasthenia gravis (MuSK-MG) in 2 children, who achieved some benefit (Skjei, 2013) and 1 adult (Evoli, 2016). Following 4 months of treatment, the authors report the adult patient had obvious improvement of ptosis, decreased neck weakness and arm fatigability. In an Investigator-Sponsored MuSK-MG randomized, double-blind, placebo-controlled, cross-over study conducted in Italy, 7 MuSK-MG patients demonstrated statistically significantly higher functional performance in all validated assessment scales when they were administered amifampridine compared to placebo. Tolerability to amifampridine was excellent (unpublished data).

A review of the literature documents that amifampridine is a safe and effective treatment in multiple neurologic disorders and is recommended by the EFNS for first-line symptomatic treatment of patients with LEMS (Skeie, 2006; Skeie, 2010; Lindquist, 2011).

5 Trial Design

5.1 Study Objectives

The primary objectives of the study is to evaluate the long-term safety and tolerability of amifampridine in patients with SMA Type 3.

The secondary objective of the study is to assess the clinical efficacy of amifampridine phosphate over time in patients with SMA Type 3 based on changes in QoL.

5.2 Study Design

This is an open label outpatient extension study designed to evaluate the long-term safety, tolerability of amifampridine in patients diagnosed with SMA Type 3. In addition, evaluation of the effects of amifampridine on the QoL will be made. The study will enroll those patients who have completed the SMA-001 study and after all final evaluations for that study have been completed, or those who demonstrated benefit after completing the dose titration period but failed to meet the randomization criteria on Day 0 of SMA-001.

The duration of participation for each patient is expected to be at least 12 months as patients may continue in the study until amifampridine is approved by Regulatory Agencies or the clinical development of amifampridine is terminated for this indication. In addition to amifampridine, patients will continue to receive previous concomitant medications, as needed.

After a new informed consent is signed and inclusion / exclusion criteria for the current protocol are satisfied, eligible patients will be given the optimal dose and dosing schedule that was identified in the Run-in Period from Protocol SMA-001.

The study will be completed when amifampridine is approved by Regulatory Agencies for Spinal Muscular Atrophy or the clinical development of amifampridine is terminated for this indication.

5.3 Study Drug Dosage and Administration

The IP is amifampridine, and it will be provided in round, white-scored tablets, containing amifampridine phosphate formulated to be the equivalent of 10 mg amifampridine base per tablet. The product will be provided in bottles of bulk tablets to the Catalyst designee who will dispense to the site an amount for each portion of the study, in a patient-specific labeled bottle.

The dose of amifampridine will be based on optimal neuromuscular benefit determined from the Run-in Period from SMA-001 or may be modified as the discretion of the Investigator. The maximum single

dose is 20 mg. The dose range for patients 6 to 16 years of age is 15 to 60 mg and for those 17 years and older, the range is from 30 to 80 mg daily. The daily dose of amifampridine can be adjusted during this long-term at the discretion of the Investigator.

6 Sample Size, Randomization and Blinding

6.1 Sample Size

This study is not powered with respect to any endpoint and no hypothesis is being tested.

The study will enroll those patients who have completed the SMA-001 study and after all final evaluations for that study have been completed, or those who demonstrated benefit after completing the dose titration period but failed to meet the randomization criteria on Day 0 of SMA-001.

6.2 Randomization and Blinding

Not applicable. This is an open label study.

7 Selection and Discontinuation of Patients

7.1 Inclusion Criteria

Individuals eligible to participate in this study must meet all the following inclusion criteria:

1. Participated in the SMA-001 study
2. Willing and able to provide written informed consent after the nature of the study has been explained and before the start of any research-related procedures.
3. Female patients of childbearing potential defined according to the Clinical Trial Facilitation Group - CTFG guidelines* must have a negative pregnancy test (urine human chorionic gonadotropin [HCG] at the end of SMA-001 study); and must practice an effective, reliable contraceptive regimen during the study and for up to 30 days following discontinuation of treatment.
4. Ability to participate in the study based on overall health of the patient and disease prognosis, as applicable, in the opinion of the Investigator; and able to comply with all requirements of the protocol, including completion of study questionnaires.

* According to CTFG guidelines a woman is considered of childbearing potential, i.e. fertile, following menarche and until becoming post-menopausal unless permanently sterile. Permanent sterilisation methods include hysterectomy, bilateral salpingectomy and bilateral oophorectomy. A postmenopausal state is defined as no menses for 12 months without an alternative medical cause.

7.2 Exclusion Criteria

Individuals who met any of the exclusion criteria in the original protocol or those listed below are not eligible to participate in the study:

1. Epilepsy and currently on medication
2. Uncontrolled asthma
3. Concomitant use with sultopride
4. Concomitant use with medicinal products with a narrow therapeutic window
5. Clinically significant abnormalities in 12 lead ECG, in the opinion of the Investigator
6. Breastfeeding or pregnant at Screening or planning to become pregnant at any time during the study

7. Intolerable amifampridine-related side effects
8. Treatment with an investigational drug (other than amifampridine) or device while participating in this study
9. Any medical condition that, in the opinion of the Investigator, might interfere with the patient's participation in the study, poses an added risk for the patient, or confound the assessment of the patient
10. History of drug allergy to any pyridine-containing substances or any amifampridine excipient(s)

For inclusion/exclusion criteria of protocol version 2.0 please refer to paragraphs 8.2.1 and 8.2.2 of study protocol.

7.3 Removal of Patients from Treatment or Assessment

Patients may withdraw their consent to participate in the study or to receive treatment at any time without prejudice. The investigator or Catalyst may withdraw a patient from the treatment or from the study at any time.

8 Schedule of Events

The schedule of clinical procedures and assessments conducted during the study is presented in Appendix 1.

9 Study Endpoints

9.1 Efficacy Endpoints

The QoL questionnaire will be performed as described in the Schedule of Events (Appendix 1).

9.1.1 Quality of life assessment

The Individualized Quality of Life for neuromuscular disease (INQoL) or the Pediatric Quality of Life (PEDSQL^{TM}) will be assessed for adult or pediatric patients, respectively.

9.2 Safety Endpoints

Safety endpoints include the evaluation of treatment-emergent AE, vital signs, clinical laboratory assessments (hematology, chemistry, and urinalysis), ECGs, and physical examinations. Pregnancy testing is also required for females of childbearing potential. The timing of the required evaluations is described in the Schedule of Events (Appendix 1).

10 Data Analysis and Statistical Considerations

Statistical tables and by-patient data listings will be prepared using SAS System (SAS Institute, Cary, North Carolina, USA), Version 9.4 under Windows 10 PRO.

10.1 Analysis Populations

The following analysis populations have been defined for this clinical study:

a) **Enrolled Population**: all patients who provided informed consent for this study.
b) **Safety Population (SAF)**: this population will consist of all patients who are enrolled in this Long-Term study and have received at least one dose of amifampridine

c) **Efficacy Analysis Set (ITT_{QOL}):** this population consists of all patients who receive at least 1 dose of amifampridine and have at least one post-treatment efficacy assessment

The safety population will be used for the analysis of all safety variables and baseline characteristics. The ITT_{QOL} population will be used for efficacy analyses on Quality of Life Questionnaires (QoL).

10.2 General Statistical Considerations

The following standards will be applied for the analyses unless otherwise specified.

Simple summary statistics (descriptive statistics) for continuous data will be n (number of non-missing observations), mean, median, standard deviation, minimum, and maximum.

The frequency count and percentage will be used to summarize categorical data. Summary statistics will be presented.

Summary statistics for all safety and efficacy variables will be presented.

All data collected will be presented in the by-patient data listings, sorted by site, patient and, where applicable, by study visit.

Change From Baseline (CFB) will be summarized by post-dose time point for efficacy and safety assessments, where Baseline for this Long-Term study is defined as follow:

- **Safety Baseline values (physical exam, ECG, Labs, vitals)**
    - **Day 0** for patient not randomized in SMA-001 study
    - **Day 28** for patient randomized in SMA-001 study
    - If the **screening visit for SMA-002** will be performed on different day from SMA-001 study last visit (i.e. not on Day 0 or Day 28 or on Early termination visit with the full panel of assessments performed), assessments have to be repeated and they will be the baseline values for SMA-002
- **Efficacy Baseline values (QoL)**
    - **Day 0** for patients that completed SMA-001 study
    - **Day 0** for patients randomized but that not completed SMA-001 study (for example discontinued on Day 7)
    - **Day 0** for patients who had QoL for SMA-001 study, however, did not qualify for randomization; however, based on PI discretion, the patient had benefit and were allowed to enrol in the SMA-002 study
    - **SMA-001 screening** for patients who had QoL performed at Screening visit of the SMA-001 study, and no QoL was performed on Day 0 or thereafter.)

10.3 Multiple Comparisons/ Multiplicity

No multiple comparisons are foreseen for this study.

10.4 Examination of Subgroups

No subgroup analyses are foreseen for this study.

10.5 Interim Analysis

There will be no Interim Analysis for this study.

10.6 Final Analysis

All final, planned analyses identified in this SAP will be performed by SPARC Consulting, Milan, Italy on behalf of Catalyst Pharmaceuticals following Sponsor Authorization of this Statistical Analysis Plan, Database Lock, and Sponsor Authorization of Analysis Sets.

10.7 Retests, Unscheduled Visits and Early Termination Data

In general, for by-visit summaries, data recorded at the nominal visit will be presented. Unscheduled measurements will not be included in by-visit summaries.

Listings will include scheduled, unscheduled, and early discontinuation data.

10.8 Windowing Conventions

Actual dates of visits will be used for calculations of time intervals. No visit windowing will be performed for this study. Out-of-window visits will not be reallocated to the nearest timepoint.

10.9 Demographic Characteristics and Medical History

10.9.1 Demographic Characteristics

Demographic parameters, including age, gender, race, body weight, height, and BMI, at Screening, will be summarized for the SAF analysis set.

10.9.2 Medical History

Summary analysis will involve the SAF analysis set.

SMA history parameters, including time (years) from onset of SMA symptoms and SMN gene analysis results will be summarized. Time from onset of SMA symptoms will be calculated as the difference in years from the year of the date of the informed consent and the year of onset of SMA symptoms reported in CRF.

All verbatim terms reported in the Medical History form will be assigned to a Preferred Term (PT) and will be classified by the primary System Organ Class (SOC) according to the Medical Dictionary for Regulatory Activities (MedDRA) thesaurus, version 23.

Previous diseases are those reported in the "Medical History" form of the eCRF with item "*Present at study start*?" flagged as "No".

Concomitant diseases are those reported in the "Medical History" form of the eCRF with item "*Present at study start*?" flagged as "Yes" or "Unknown".

The following data will be presented:

a) A default frequency table showing the number and percentage of patients who exhibited an abnormality by single body system;
b) A default frequency table showing the number and percentage of patients who exhibited at least one previous disease and the previous diseases by primary SOC and PT;
c) A default frequency table showing the number and percentage of patients who exhibited at least one concomitant disease and the concomitant diseases by primary SOC and PT;

Medical history will be listed for all enrolled patients.

10.10 Medication and Treatment Analysis

10.10.1 Patient Disposition and Accountability

The number and percentage of patients who are enrolled, in the SAF and ITT_{QOL} analysis set, and who complete Long-term study will be presented. The number and percentage of patients who discontinue dosing prematurely, along with the primary reason that dosing was discontinued prematurely, will be presented.

10.10.2 Duration of Treatment and Compliance

Duration of treatment will be summarized for the SAF analysis set.

The duration of treatment is the (*date of the last study drug dose intake* minus the *date of administration of the first dose of study drug*) +1.

Compliance is 100%*(Number consumed)/(Number prescribed). "Number consumed" is from Study Drug Accountability and "Number prescribed" is number of tablets to have been taken daily duration. Compliance will be summarized for the SAF analysis set.

10.10.3 Concomitant Medications

All prescription and over-the-counter medications and herbal and nutritional supplements taken during the Long-Term study will be recorded on the designated CRF. All prior medications taken within 14 days of initiation of this study are to be recorded on the CRF which is also recorded in the appropriate CRF pages of Study SMA-001 as concomitant medications.

The Investigator may prescribe additional medications during the study if the prescribed medication is not prohibited by the protocol. In the event of an emergency, any needed medications may be prescribed without prior approval, but the medical monitor must be notified of the use of any contraindicated medications immediately thereafter. Any concomitant medications added or discontinued during the study should be recorded on the CRF.

Concomitant medications will be only listed for all enrolled patients.

10.11 Efficacy Analyses

Efficacy will be assessed by changes in QoL over time.

Assessments of efficacy will be performed using the following endpoints:

1. CFB in the INQoL subscales scores:

   Symptoms (section 1):

   a) Weakness
   b) Muscle "locking"
   c) Pain
   d) Fatigue
   e) Droopy eyelids
   f) Double vision
   g) Swallowing difficulties

   Life domains (section 2):

   a) Activities
   b) Independence

c) Social relationship
d) Emotions
e) Body image

Life domains (section 3):

a) Perceived treatment effects
b) Expected treatment effects

Quality of Life

2. CFB in the PEDSQL score (where applicable).

The individual domain scores in INQoL will be calculated using the scoring algorithms outlined in Appendix 2.

Results from INQoL questionnaires and PEDSQL inventory of disease specific symptoms will be summarized with descriptive statistics (n, mean, standard deviation, minimum, median, and maximum) per time point (Months 3, 6, 9, 12, 15, 21 and 27) and CFB, using the Day 0 [in the case where patient completed SMA-001 study, was randomized but not completed SMA-001 study, was not randomized; however based on PI discretion, the patient had benefits and were allowed to enroll in the SMA-002] or the screening visit for the SMA-001 study [in the case where patient had QoL performed at Screening visit of the SMA-001 study, and No QoL was performed on Day 0 or thereafter] response as the baseline level.

The subscales of the INQoL questionnaires will be similarly summarized.

Efficacy analysis will be conducted on ITT_{QOL} analysis set. The efficacy analysis will be descriptive and will be presented on observed data only. Means with standard deviations of INQoL and PEDSQL scores will be also presented.

10.12 Safety Data

Summaries of safety data will be presented for the SAF analysis set using observed data only, i.e. without imputation. All observed safety data will be listed.

10.12.1 Adverse Events

For this protocol, a reportable AE is any untoward medical occurrence (e.g. sign, symptom, illness, disease or injury) in a patient administered the IP or other protocol-imposed intervention, regardless of attribution. This includes:

- AEs not previously observed in the patient, that emerge during the study.
- Pre-existing medical conditions judged by the Investigator to have worsened in severity or frequency or changed in character during the study.
- Complications that occur as a result of non-drug protocol-imposed interventions.

An adverse drug reaction is any AE for which there is a reasonable possibility that the IP caused the AE. “Reasonable possibility” means there is evidence to suggest a causal relationship between the IP and the AE.

Whenever possible, it is preferable to record a diagnosis as the AE term rather than a series of terms relating to a diagnosis.

All ongoing non-serious AEs, even those which began under SMA-001 study, will be reported from the end of the double-blind study (SMA-001) or from Day 0 for those not randomized to double blind medication in SMA-001 study through the termination visit or at the early termination visit and will be

recorded on the AE page of the eCRF. All ongoing SAEs from SMA-001 study will be reported. The criteria for determining, and the reporting of new SAEs is provided in Study Protocol Section 11.2.

The Investigator should follow all unresolved AEs until the events are resolved or stabilized, the patient is lost to follow-up, or it has been determined that the study treatment or participation is not the cause of the AE. Resolution of AEs (with dates) should be documented on the appropriate CRF page(s) and in the patient's medical record.

The Investigator responsible for the care of the patient or qualified designee will assess AEs for severity, relationship to IP, and seriousness (refer to Protocol Section 11.2 for SAE definition). Severity (as in mild, moderate or severe headache) is not equivalent to seriousness, which is based on patient/event outcome or action criteria usually associated with events that pose a threat to a patient's life or functioning. Seriousness (not severity) serves as a guide for defining regulatory reporting obligations.

The Investigator will determine the severity of each AE using grades defined in Table 1 (the event will be recorded on the source documents and AE CRF). Events that are Grades 4 and 5 are serious events and require completion of both an SAE form and AE CRF.

Table 1 - Categories of Severity for Adverse Events

Severity	Description	
Grade 1	Mild; asymptomatic or mild symptoms; clinical or diagnostic observations only; intervention not indicated.	
Grade 2	Moderate; minimal, local or noninvasive intervention indicated; limiting age-appropriate instrumental ADL*.	
Grade 3	Severe or medically significant but not immediately life-threatening; hospitalization or prolongation of hospitalization indicated; disabling; limiting self-care ADL**.	
Grade 4	Life-threatening consequences; urgent intervention indicated.	Note: Grade 4 and 5 adverse events should always be reported as serious adverse events
Grade 5	Death related to AE.	

Activities of Daily Living (ADL)

* Instrumental ADLs refer to preparing meals, shopping for groceries or clothes, using the telephone, managing money, etc.

** Self-care ADLs refer to bathing, dressing and undressing, feeding self, using the toilet, taking medications, and not bedridden.

A Treatment-Emergent Adverse Event (TEAE) is any AE that emerges after the first dose of study drug for the current Long-Term Study. All AEs will be coded using the Medical Dictionary for Regulatory Activities (MedDRA), Version 23.0. All TEAEs will be summarized. Counts and percentages will be presented for each observed System Organ Class (SOC) and Preferred Term (PT) as defined in MedDRA. A patient having more than one TEAE with the same PT will be counted only once in the incidence calculation for that PT. Similarly, if a patient has more than one TEAE in the same SOC, the patient will be counted only once in the total number of patients with a TEAE for that SOC. Each TEAE will be assessed with respect to severity and relationship to treatment. All AEs will be listed, regardless of whether they were study treatment related.

The relationship of an AE will be recorded using the specified relationship categories described in Table 2.

Table 2 - Description of relationship to adverse events categories

Relationship Category	Description
Not Related	Exposure to the IP has not occurred OR The administration of the IP and the occurrence of the AE are not reasonably related in time OR The AE is considered likely to be related to an etiology other than the use of the IP; that is, there are no facts [evidence] or arguments to suggest a causal relationship to the IP.
Possibly Related	The administration of the IP and the occurrence of the AE are reasonably related in time AND The AE could be explained equally well by factors or causes other than exposure to the IP.
Probably Related	The administration of IP and the occurrence of the AE are reasonably related in time AND The AE is more likely explained by exposure to the IP than by other factors or causes.

The following summary tables will be provided:

1. An overview of TEAEs including the number of TEAEs, related TEAEs, TESAEs, related TESAEs, TEAEs leading to premature discontinuation, TEAEs leading to hospitalization, life-threatening TEAEs, number of patients with at least one TEAE, at least one related TEAE, at least one TESAE, at least one related TESAE, at least one TEAE leading to premature discontinuation, at least one TEAE leading to hospitalization, at least one life-threatening TEAE, and at least one TEAE leading to death
2. Summary of all TEAEs by Primary SOC and PT
3. Summary of all related TEAEs by Primary SOC and PT
4. Summary of all TESAEs by Primary SOC and PT
5. Summary of all related TESAEs by Primary SOC and PT
6. Summary of all TEAEs leading to hospitalization by Primary SOC and PT
7. Summary of all life-threatening TEAEs by Primary SOC and PT
8. Summary of all TEAEs leading to premature discontinuation by Primary SOC and PT
9. Summary of all TEAEs by Primary SOC and PT and maximum severity
10. Summary of all TEAEs by Primary SOC and PT and relationship to treatment.

The following individual data listings will be produced:

1. A listing of all TEAEs
2. A listing of all TESAEs
3. A listing of all IMP related TEAEs
4. A listing of all IMP related TESAEs
5. A listing of all TEAEs leading to hospitalization
6. A listing of all life-threatening TEAEs
7. A listing of TEAEs leading to premature discontinuation
8. A listing of all TEAEs leading to death
9. A listing of all AEs started before the first dose of study drug for the current Long-Term Study

10.12.2 Vital Signs and Body Weight

Analysis will be performed in the SAF analysis set.

Vital signs, including sitting blood pressure (systolic and diastolic; mmHg), sitting heart rate (beats/minute), body temperature (ºC), respiration rate (breaths/minute), and weight (kg) will be summarized by time point using descriptive statistics. Changes from baseline will also be summarized by post-dose time point.

Height is recorded at Screening visit only.

BMI will be calculated at Screening visit according to the following formula:

BMI (kg/m^2) = ((body weight (kg)/(height (m))^2).

Height and BMI will be summarized using descriptive statistics.

10.12.3 12-Lead ECG

Analysis will be performed in the SAF analysis set.

A standard 12-lead ECG will be recorded at Screening, Months 3, 6, 9, 12, 15, 21 and 27 visit.

Each tracing will be classified as “Normal tracing”, “Abnormal tracing (not clinically significant)”, “Abnormal tracing (clinically significant)”, or “Unknown”.

ECG results will be summarized in a shift table with the following shifts presented (baseline versus post dose time point): normal/normal, normal/abnormal NCS, normal/abnormal CS, abnormal NCS/normal, abnormal NCS/abnormal NCS, abnormal NCS/abnormal CS, abnormal CS/normal, abnormal CS/abnormal NCS and abnormal CS/abnormal CS.

Clinically significant changes from baseline will be recorded as AEs.

Abnormalities reported will be coded using MedDRA thesaurus version 23.0, and the terms listed for all enrolled patients.

10.12.4 Physical Examination

Summary analysis will involve the SAF analysis set.

A complete physical examination is to be performed at each clinic visit or, if applicable, at early discontinuation from the study.

These assessments will be summarized by visit using counts and percentages.

All verbatim terms reported in the Physical Examination form will be assigned to a Preferred Term (PT) and will be classified by the primary System Organ Class (SOC) according to the Medical Dictionary for Regulatory Activities (MedDRA) thesaurus, version 23.

A summary table containing the frequency and percentage of patients who exhibited at least one abnormality as well as of the abnormalities reported at each clinic visit by primary SOC and PT will be prepared.

Physical examination abnormalities will be listed for all patients.

10.12.5 Laboratory Tests

For blood chemistry and hematology, descriptive statistics will be presented for the observed value and CFB by time point. In addition, a shift table will be constructed to show the shifts in laboratory results by parameter relative to the normal ranges. The number and percentage of patients with the following shifts will be presented by time point: normal/normal, normal/low, normal/high, low/low, low/normal, low/high, high/low, high/normal, and high/high. The laboratory parameters to be collected are given below in Table 3.

Table 3 - Clinical Laboratory Tests

Blood Chemistry	Hematology	Urine Tests
Albumin	Hemoglobin	Appearance
Alkaline phosphatase	Hematocrit	Color
ALT (SGPT)	WBC count	pH
AST (SGOT)	RBC count	Specific gravity
Direct bilirubin	Platelet count	Ketones
Total bilirubin	Differential cell count	Protein
BUN		Glucose
Calcium		Bilirubin
Chloride		Nitrite
Total cholesterol		Urobilinogen
CO_2 or bicarbonate		Blood in urine
Creatine phosphokinase		
Creatinine		
Glucose		
GGT		
LDH		
Phosphorus		
Potassium		
Total protein		
Sodium		
Uric acid		

ALT, alanine aminotransferase; AST, aspartate aminotransferase; BUN, blood urea nitrogen; CO_2, carbon dioxide; GGT, gamma-glutamyl transferase; LDH, lactate dehydrogenase; RBC, red blood cell; SGOT, serum glutamic-oxaloacetic transaminase; SGPT, serum glutamic-pyruvic transaminase; WBC, white blood cell.

Urinalysis parameters will not be included in summary tables.

All laboratory parameters will be listed for all patients.

10.12.5.1 Laboratory Specific Derivations

For each laboratory parameter, where appropriate, the reported values will be converted into SI units and if needed boundary values for reference ranges will be converted as well.

Conversion to SI will be provided using textbook ranges: *SI Unit Conversion Guide, M. Laposata, NEJM, 1992*. To convert from the conventional unit to the SI unit, multiply by the conversion factor:

Table 4 - Clinical Laboratory Tests SI Conversion Factors

Component	Conventional Unit	Conversion Factor	SI Unit
Hematology			
RBC count	x 10^6/µL	1	x 10^{12}/L
WBC count	x 10^3/µL	1	x 10^9/L
Neutrophils	x 10^3/µL	1	x 10^9/L
Lymphocytes	x 10^3/µL	1	x 10^9/L
Monocytes	x 10^3/µL	1	x 10^9/L
Eosinophils	x 10^3/µL	1	x 10^9/L
Basophils	x 10^3/µL	1	x 10^9/L
Platelet count	x 10^3/µL	1	x 10^9/L
Hemoglobin	g/dL	10	g/L
Hematocrit	%	0.01	Proportion of 1.0
Biochemistry			
Albumin	g/dL	10	g/L
Alkaline phosphatase (ALP)	U/L	1	U/L
ALT (SGPT)	U/L	1	U/L
AST (SGOT)	U/L	1	U/L
Direct Bilirubin	mg/dL	17.104	µmol/L
Total Bilirubin	mg/dL	17.104	µmol/L
BUN	mg/dL	0.357	mmol/L
Calcium	mg/dL	0.25	mmol/L
Chloride	mEq/L	1.0	mmol/L
Total cholesterol	mg/dL	0.02586	mmol/L
CO_2 or bicarbonate	mEq/L	1	mmol/L
Creatinine phosphokinase	U/L	0.01667	µkat/L
Creatinine	mg/dL	88.4	µmol/L
Glucose	mg/dL	0.05551	mmol/L
Gamma-GT (GGT)	U/L	1	U/L
LDH	U/L	0.01667	µkat/L
Phosphorus	mg/dL	0.3229	mmol/L
	mEq/L	1.0	mmol/L
Potassium	mg/dL	0.2558	
Total protein	g/dL	10	g/L
Sodium	mEq/L	1.0	mmol/L
Uric acid	mg/dL	59.48	µmol/L

10.13 Protocol Deviations

All the protocol deviations will be discussed case by case by the clinical team during the review of the data before the lock of the study database and described in the Data Review Report.

However, the following protocol deviations are anticipated to be considered as major:

- Inclusion and exclusion criteria not respected;
- Missing information on IMP administration that does not allow calculation of IMP exposure/compliance;
- Overall study treatment compliance < 80%;
- Use of prohibited medications during study (see Protocol Section 10.4).

The number and percentage of patients with each type of protocol deviation will be presented.

11 Data Handling

11.1 Multicenter Sites

This study is conducted by multiple Investigators in two investigational sites.

Site-related differences will not be evaluated and presented in the statistical output as the study does not foresee a stratification by Site.

11.2 Handling of Missing Data

Missing data due to discontinuation will not be imputed. Missing safety data will not be imputed.

Handling of missing data in INQoL is provided in the Appendix 2.

12 Changes from the Protocol

No changes from the statistical analyses planned in the study protocol have been introduced in this SAP.

13 Table Shells and Specifications

13.1 Table Specifications

Tables will be provided as defined by the table shells.

All output will be generated by means of SAS System and exported into a Microsoft Word document in RTF format. All output will be in landscape orientation. Left and right margins will be 2 cm from the side; the top and bottom margins will be 2.5 cm. Font size will be Courier New 7 pt.

The header containing the sponsor name (Catalyst Pharmaceuticals) and protocol number will appear on the top left corner of each page of the output. The page number, in the format of “Page x of y”, will appear on the top right corner of the output, where y = last page of corresponding output.

Column headers in tables include the total possible numbers to be included in summaries for that table, designated as “(N=XX)”.

The SAS program name, the date of the creation of the output (run date) and the listings from which the tables were derived will appear on the bottom left corner as follows:

Source: [program name].sas, Run on ddmmmyyyy

Source: Listing(s) xx.x.x, xx.x.x

13.1.1 Table Format Specification

Maximum and minimum values will be reported with the same number of decimal places as collected. Means and medians will be reported to one additional decimal place. Standard deviations and standard errors will be reported to two decimal places more than the collected data. Percentages will be reported with one decimal place.

Data in the tables are formatted as follows:

Text fields in the body of the tables and listings will be left-justified.

When no data are available for a table, an empty page with the title will be produced with suitable text. Example: THERE WERE NO SERIOUS ADVERSE EVENTS.

13.2 Line Listings Specifications

Individual line listings will be provided as defined by the listing shells.

All output will be generated by SAS System and exported into a Microsoft Word document in RTF format. All output will be in landscape orientation. Left and right margins will be 2 cm from the side; the top and bottom margins will be 2.5 cm. Font size will be Courier New 7 pt.

The header containing the sponsor name (Catalyst Pharmaceuticals) and protocol number will appear on the top left corner of each page of the output. The page number, in the format of "Page x of y", will appear on the top right corner of the output, where y = last page of corresponding output.

The SAS program name, and the date of the creation of the output (run date) will appear on the bottom left corner as follows:

Source: [program name].sas, Run on ddmmmyyyy.

14 Programming Conventions for Outputs

Outputs will be presented according to the standard SPARC's layout of tables and line listings.

Dates & Times: depending on data available, dates and times will take the form ddmmmyyyy hh:mm (i.e. 01JAN2019 10:20)

Spelling Format: English US.

Listings: all listings will be ordered by the following (unless otherwise indicated in the template):

- Site ID
- Patient number
- Study visit
- Date and hours (where applicable)

In all listings missing data will be reported, according to the variable type, as follows:

- Character variables and dates will be presented as empty fields
- Numerical variables will be presented with a "-"

Appendix 1: Schedule of Events

Study Assessment or Event	Screening and Enrollment*	End of Study Month ± 1 week							
		3	6	9	12	15	21	27	EoS[g]
Informed consent [a]	x								
Inclusion/Exclusion Criteria	x								
Complete physical exam [b]	x	x	x	x	x	x	x	x	x
Vital signs	x	x	x	x	x	x	x	x	x
12-Lead ECG	x	x	x	x	x	x	x	x	x
Clinical laboratory tests [c]	x	x	x	x	x	x	x	x	x
Pregnancy test [d]	x	x	x	x	x	x	x	x	x
Amifampridine Treatment		x	x	x	x	x	x	x	
QoL	x	x	x	x	x	x	x	x	x
Dispense IP [e]	x	x	x	x	x	x	x		
IP accountability		x	x	x	x	x	x	x	x
Adverse events/SAEs [f]	x	x	x	x	x	x	x	x	x
Concomitant medications	x	x	x	x	x	x	x	x	x

EoS= End of Study is the patients last visit to the site and discontinuation of study medication; IP=investigational product; QoL=quality of life; SAE=serious adverse event.
a Informed consent must be obtained before any study procedures are performed.
b Complete physical examination includes evaluation of all major body systems, including weight at all visits.
c Clinical laboratory tests include serum chemistry, hematology, and urinalysis.
d Urine pregnancy tests will be obtained from female patients of childbearing potential.
e Pharmacist will dispense sufficient amount of medication until the next clinic visit. All unused medication must be returned to the clinic for drug accountability.
f SAE reporting commences after informed consent is signed. Non-serious adverse event reporting commences after the first dose of study drug for the current Long-Term Study.
g EoS or early termination visit if the last visit does not coincide the scheduled 3- or 6-month visit.
*These data may be obtained from Study SMA-001. If data not available, then the assessment should be repeated.

Appendix 2: INQoL Scaling and Scoring

Scaling and scoring INQoL_English.pdf

Scaling and scoring of PedsQL English.pdf

Table Number	Title of Table	Analysis Population	Listing Source
14.1.1.	Patients Enrollment by Site	Enrolled	16.2.1
14.1.2	Analysis Populations / All Enrolled Patients	Enrolled	16.2.1
14.1.3	Study Completion and Primary Reason for Premature Discontinuation of Study Medication	SAF	16.2.1.
14.1.4.	Protocol Deviations	SAF	16.2.3.1.
14.1.5.	Major Protocol Deviations	SAF	16.2.3.1.
14.1.6.	Demographic Characteristics	SAF	16.2.4.
14.1.7.	Childbearing Potential and Pregnancy Test at Screening	SAF	16.2.7.1.
14.1.8.	SMA History and SMN Gene Analysis	SAF	16.2.4.2.1
14.1.9.	Medical History	SAF	16.2.4.2.2.
14.1.10.	Previous Diseases from Medical History by SOC and PT	SAF	16.2.4.2.2.
14.1.11.	Concomitant Diseases from Medical History by SOC and PT	SAF	16.2.4.2.2.
14.1.12.	Physical Examination	SAF	16.2.7.4.
14.1.13.	Physical Examination Abnormalities by SOC and PT	SAF	16.2.7.4.
14.2.1.	Duration of Treatment	SAF	16.2.5.2.1.
14.2.3.	Amiframpridine Dosage by Visit	SAF	16.2.5.1.2.
14.2.4.	Treatment Compliance	SAF	16.2.5.2.1.
14.3.1.	Summary of Treatment Emergent Adverse Events (TEAEs)	SAF	16.2.5.1.
14.3.2.	Summary of TEAEs by SOC and PT	SAF	16.2.5.1.
14.3.3.	Summary of Related TEAEs by SOC and PT	SAF	16.2.5.3.
14.3.4.	Summary of TESAEs by SOC and PT	SAF	16.2.5.2.
14.3.5.	Summary of Related TESAEs by SOC and PT	SAF	16.2.5.4.
14.3.6.	Summary of TEAEs Leading to Hospitalization by SOC and PT	SAF	16.2.5.5.
14.3.7.	Summary of Life-Threatening TEAEs by SOC and PT	SAF	16.2.5.6.
14.3.8.	Summary of TEAEs Leading to Premature Discontinuation by SOC and PT	SAF	16.2.5.7.
14.3.9.	Summary of TEAEs by SOC and PT and Maximum Severity	SAF	16.2.5.1.

Table Number	Title of Table	Analysis Population	Listing Source
14.3.10.	Summary of TEAEs by SOC and PT and relationship to Treatment	SAF	16.2.5.1.
14.4.1.	Summary of Clinical Laboratory Tests: Haematology	SAF	16.2.6.2.
14.4.2.	Summary of Clinical Laboratory Tests: Blood Chemistry	SAF	16.2.6.1.
14.4.3.	Summary of Hematology Shift Results Relative to the Normal Range	SAF	16.2.6.2.
14.4.4.	Summary of Blood Chemistry Shift Results Relative to the Normal Range	SAF	16.2.6.1.
14.5.1.	Summary of Vital Signs: Systolic Blood Pressure	SAF	16.2.7.2.
14.5.2.	12-Lead ECG	SAF	16.2.7.3.
14.5.3.	Childbearing Potential and Pregnancy Test	SAF	16.2.7.1.
14.6.1.	Summary of INQoL: Weakness	ITT_{QOL}	16.2.7.4.1. 16.2.7.4.2. 16.2.7.4.3. 16.2.7.4.4. 16.2.7.4.5. 16.2.7.4.6.
14.6.2.	Summary of PEDSQL: About my Neuromuscular Disease	ITT_{QOL}	16.2.7.1.1. 16.2.7.1.2. 16.2.7.2.1. 16.2.7.2.2. 16.2.7.3.1. 16.2.7.3.2.

Listing Number	Title of Listing
16.2.1	Completed and Discontinued Patients with Primary Reason for Discontinuation
16.2.2.1	Inclusion Criteria
16.2.2.2	Exclusion Criteria
16.2.3.1	Protocol Deviations
16.2.3.2	Patients Excluded from the Analysis Populations
16.2.4.	Demographic Characteristics
16.2.4.2.1	Medical History: SMA Characteristics
16.2.4.2.2	Medical History: Previous and Concomitant Diseases
16.2.5.1.1	Amifampridine Treatment at Screening
16.2.5.1.2	Amifampridine Treatment Over the Study
16.2.5.2.1	Duration and Compliance Over the Study
16.2.5.2.2	Study Drug Accountability
16.2.6.1	Quality of Life (QoL): Type of Questionnaire
16.2.7.1.1	PEDSQL Parent Report for Young Child (5-7 years): About My Child's Neuromuscular Disease (Items from 1 to 17)
16.2.7.1.2	PEDSQL Parent Report for Young Child (5-7 years): Communication (Items from 1 to 3), About our Family Resources (Items from 1 to 5) and Questionnaire Scores
16.2.7.2.1	PEDSQL Parent Report for Child (8-12 years): About My Child's Neuromuscular Disease (Items from 1 to 17)
16.2.7.2.2	PEDSQL Parent Report for Child (8-12 years): Communication (Items from 1 to 3), About our Family Resources (Items from 1 to 5) and Questionnaire Scores
16.2.7.3.1	PEDSQL Teen Report (13-18 years): About My Neuromuscular Disease (Items from 1 to 17)
16.2.7.3.2	PEDSQL Teen Report (13-18 years): Communication (Items from 1 to 3), About our Family Resources (Items from 1 to 5) and Questionnaire Scores
16.2.7.4.1	INQOL: Items from Section 1-1 to Section 1-5C
16.2.7.4.2	INQOL: Items from Section 1-6 to Section 2-Question 1.B2
16.2.7.4.3	INQOL: Items from Section 2-Question 2.A to Section 2-Question 3.B6
16.2.7.4.4	INQOL: Items from Section 2-Question 4.A1 to Section 3-Question 1.A3

Table 14.1.1. Patients Enrolment by Site

	Statistic	N = XX
Number of Enrolled Patients		
Site 01	n (%)	xx (xx.x%)
Site 02	n (%)	xx (xx.x%)
Total	n (%)	xx (xx.x%)
Number of Screening Failures		
Site 01	n (%)	xx (xx.x%)
Site 02	n (%)	xx (xx.x%)
Total	n (%)	xx (xx.x%)

Percentages are calculated relative to the total number of patients enrolled.
a) FPFV: DDMONYYYY Patient XX.
b) LPLV: DDMONYYYY Patient YY.

Source: xxx.sas, Run on ddmmmyyyy
Reference Listing: 16.2.1.

Table 14.1.2. Analysis Populations / All Enrolled Patients

	N = XX
Patients Enrolled	xx
Safety Population (SAF)	xx (xx.x)
Efficacy Population (ITT_{QOL})	xx (xx.x)

Percentages are calculated on the number of enrolled patients.

a) Safety Population (SAF) consists of all patients who were enrolled in the Long-Term study and had received at least one dose of amifampridine.
b) Efficacy Population (ITT_{QOL}) consists of all patients who receive at least 1 dose of amifampridine and have at least one post-treatment efficacy assessment

Source: xxx.sas, Run on ddmmmyyyy
Reference Listing: 16.2.1.

Table 14.1.3. Study Completion and Primary Reason for Premature Discontinuation of Study Medication / SAF

	Amifampridine (N=xx)
No. patients who prematurely discontinued study medication	xx (xx.x)
Primary Reason for Discontinuation	
Drug inefficacy	xx (xx.x)
Adverse event	xx (xx.x)
Investigator's decision	xx (xx.x)
Consent withdrawal (not due to AE)	xx (xx.x)
Non study drug compliance	xx (xx.x)
Major protocol deviation	xx (xx.x)
Lost to follow-up during the study	xx (xx.x)
Disease progression	xx (xx.x)
Screening failure	xx (xx.x)
Other reason	xx (xx.x)
Total	xx

Percentages are calculated on the number of patients (N).

Source: xxx.sas, Run on ddmmmyyyy
Reference Listing: 16.2.1.

Table 14.1.4. Protocol Deviations / SAF

	Amifampridine (N=xx)
No. of patients with at least one protocol deviation	xx (xx.x)
Deviation 1	xx (xx.x)
Deviation 2	xx (xx.x)
…	xx (xx.x)
Deviation n	xx (xx.x)

A patient may have more than one deviation.

Source: xxx.sas, Run on ddmmmyyyy
Reference Listings: 16.2.1.

Table 14.1.5. Major Protocol Deviations / SAF

	Amifampridine (N=xx)
No. of patients with at least one major protocol deviation	xx (xx.x)
Deviation 1	xx (xx.x)
Deviation 2	xx (xx.x)
…	xx (xx.x)
Deviation n	xx (xx.x)

A patient may have more than one more deviation.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.3.1.

Table 14.1.6. Demographic Characteristics / SAF

	Statistic	Amifampridine (N=xx)
Age (years)	n	xx
	Mean (SD)	xx.x (xx.xx)
	Median	xx.x
	Min, Max	xx, xx
Gender		
Male	n (%)	xx (xx.x)
Female	n (%)	xx (xx.x)
Race		xx (xx.x)
Caucasian	n (%)	xx (xx.x)
Black	n (%)	xx (xx.x)
Asian	n (%)	xx (xx.x)
Other	n (%)	xx (xx.x)
Unknown	n (%)	xx (xx.x)
Height (cm)	n	xx
	Mean (SD)	xx.x (xx.xx)
	Median	xx.x
	Min, Max	xx, xx
Weight (kg)	n	xx
	Mean (SD)	xx.x (xx.xx)
	Median	xx.x
	Min, Max	xx, xx
BMI (kg/cm^2)	n	xx
	Mean (SD)	xx.x (xx.xx)
	Median	xx.x
	Min, Max	xx, xx

Source: xxx.sas, Run on ddmmmyyyy
Reference Listings: 16.2.4.

Table 14.1.7. Childbearing Potential and Pregnancy Test at Screening / SAF

	Statistic	Amifampridine (N=xx)
Childbearing potential		
Patient male	n (%)	xx (xx.x%)
Menopause	n (%)	xx (xx.x%)
Sterilized	n (%)	xx (xx.x%)
Total hysterectomy	n (%)	xx (xx.x%)
No contraception protection	n (%)	xx (xx.x%)
Contraception protection	n (%)	xx (xx.x%)
Child	n (%)	xx (xx.x%)
Unknown	n (%)	xx (xx.x%)
Serum pregnancy test performed?		
No	n (%)	xx (xx.x%)
Yes	n (%)	xx (xx.x%)
Not applicable	n (%)	xx (xx.x%)
Type of pregnancy test (#)		
Urine dipstick	n (%)	xx (xx.x%)
Serum	n (%)	xx (xx.x%)
Unknown	n (%)	xx (xx.x%)
Pregnancy test result (#)		
Negative	n (%)	xx (xx.x%)
Positive	n (%)	xx (xx.x%)
Unknown	n (%)	xx (xx.x%)

Percentages are calculated on the number of patients (N) in the SAF analysis set.
Percentages are based on the number of female patients performing pregnancy test.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.7.1.

Table 14.1.8. SMA History and SMN gene analysis / SAF

	Statistic	Amifampridine (N=xx)
Time from onset of SMA symptoms (years)*		
	n	xx
	Mean (SD)	xx.x (xx.xx)
	Median	xx.x
	Min, Max	xx, xx
Was the patient previously treated for SMA other than amifampridine phosphate?		
No	n (%)	xx (xx.x)
Yes	n (%)	xx (xx.x)
Unknown	n (%)	xx (xx.x)
SMN gene analysis result		
Negative	n (%)	xx (xx.x)
Positive (mutated)	n (%)	xx (xx.x)
Not examined	n (%)	xx (xx.x)
Unknown	n (%)	xx (xx.x)

Percentages are calculated on the number of patients (N) in the SAF analysis set.
* Time from onset of SMA symptoms was calculated as the difference in years from the year of the date of the informed consent and the year of onset of SMA symptoms reported in CRF.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.4.2.1

Table 14.1.9. Medical History / SAF

Body System	Statistic	Amifampridine (N=xx)
Cardiovascular	n (%)	xx (xx.x)
Respiratory	n (%)	xx (xx.x)
Muscle-Skeletal	n (%)	xx (xx.x)
Ears, nose, eyes and throat	n (%)	xx (xx.x)
Hepatobiliary	n (%)	xx (xx.x)
Endocrine & Metabolic	n (%)	xx (xx.x)
Haemapoietic	n (%)	xx (xx.x)
Central nervous	n (%)	xx (xx.x)
Dermatologic	n (%)	xx (xx.x)
Genitourinary	n (%)	xx (xx.x)
Gastrointestinal	n (%)	xx (xx.x)
Psychiatric Diseases	n (%)	xx (xx.x)
Allergies	n (%)	xx (xx.x)

The counts are the number of patients with at least one "Yes" response for the body system.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.4.2.2

Programming Note: "Other Body System" is also recorded, but is not included in the above table; it is listed only.

Table 14.1.10. Previous Diseases from Medical History by SOC and PT / SAF

System Organ Class Preferred Term	Statistic	Amifampridine (N=xx)
No. of patients with at least one previous disease	n (%)	xx (xx.x%)
System Organ Class 1	n (%)	xx (xx.x%)
Preferred Term 1	n (%)	xx (xx.x%)
Preferred Term 2	n (%)	xx (xx.x%)
Preferred Term x	n (%)	xx (xx.x%)
System Organ Class 2	n (%)	xx (xx.x%)
Preferred Term 1	n (%)	xx (xx.x%)
Preferred Term 2	n (%)	xx (xx.x%)
Preferred Term x	n (%)	xx (xx.x%)
System Organ Class x	n (%)	xx (xx.x%)
Preferred Term 1	n (%)	xx (xx.x%)
Preferred Term 2	n (%)	xx (xx.x%)
Preferred Term x	n (%)	xx (xx.x%)

Previous diseases are those reported in the "Medical History" form of the eCRF with item "Present at study start?" flagged as "No".
Abnormalities were coded using the MedDRA dictionary version 23. Primary System Organ Class and Preferred Term are presented.
Patients are only counted once per SOC and PT.
Patients can have more than one previous disease.
Percentages are calculated on the number of patients (N) in the SAF analysis set.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.4.2.2

Table 14.1.11. Concomitant Diseases from Medical History by SOC and PT / SAF

System Organ Class Preferred Term	Statistic	Amifampridine (N=xx)
No. of patients with at least one concomitant disease	n (%)	xx (xx.x%)
System Organ Class 1	n (%)	xx (xx.x%)
Preferred Term 1	n (%)	xx (xx.x%)
Preferred Term 2	n (%)	xx (xx.x%)
Preferred Term x	n (%)	xx (xx.x%)
System Organ Class 2	n (%)	xx (xx.x%)
Preferred Term 1	n (%)	xx (xx.x%)
Preferred Term 2	n (%)	xx (xx.x%)
Preferred Term x	n (%)	xx (xx.x%)
System Organ Class x	n (%)	xx (xx.x%)
Preferred Term 1	n (%)	xx (xx.x%)
Preferred Term 2	n (%)	xx (xx.x%)
Preferred Term x	n (%)	xx (xx.x%)

Concomitant diseases are those reported in the "Medical History" form of the eCRF with item "Present at study start?" flagged as "Yes" or missing.
Abnormalities were coded using the MedDRA dictionary version 23. Primary System Organ Class and Preferred Term are presented.
Patients are only counted once per SOC and PT.
Patients can have more than one concomitant disease.
Percentages are calculated on the number of patients (N) in the SAF analysis set.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.4.2.2

Table 14.1.12. Physical Examination / SAF

	System	Statistic	Amifampridine (N=xx)
Screening	Head & Neck	n (%)	xx (xx.x)
	Eyes	n (%)	xx (xx.x)
	Ears	n (%)	xx (xx.x)
	Nose	n (%)	xx (xx.x)
	Throat	n (%)	xx (xx.x)
	Cardiovascular	n (%)	xx (xx.x)
	Dermatologic	n (%)	xx (xx.x)
	Lymphatic	n (%)	xx (xx.x)
	Respiratory	n (%)	xx (xx.x)
	Gastrointestinal	n (%)	xx (xx.x)
	Musculoskeletal	n (%)	xx (xx.x)
Month 3	Head & Neck	n (%)	xx (xx.x)
	Eyes	n (%)	xx (xx.x)
	Ears	n (%)	xx (xx.x)
	Nose	n (%)	xx (xx.x)
	Throat	n (%)	xx (xx.x)
	Cardiovascular	n (%)	xx (xx.x)
	Dermatologic	n (%)	xx (xx.x)
	Lymphatic	n (%)	xx (xx.x)
	Respiratory	n (%)	xx (xx.x)
	Gastrointestinal	n (%)	xx (xx.x)
	Musculoskeletal	n (%)	xx (xx.x)
…			
EOS	Head & Neck	n (%)	xx (xx.x)
	Eyes	n (%)	xx (xx.x)
	Ears	n (%)	xx (xx.x)
	Nose	n (%)	xx (xx.x)
	Throat	n (%)	xx (xx.x)
	Cardiovascular	n (%)	xx (xx.x)
	Dermatologic	n (%)	xx (xx.x)
	Lymphatic	n (%)	xx (xx.x)
	Respiratory	n (%)	xx (xx.x)
	Gastrointestinal	n (%)	xx (xx.x)
	Musculoskeletal	n (%)	xx (xx.x)

The counts are the number of patients with at least one "Yes" response for the body system.
Percentages are calculated using the number of patients with "Yes" or "No" recorded for the Body System as the denominator.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.7.4.

Programming Note: "Other Body System" is also recorded, but is not included in the above table; it is listed only.

Table 14.1.13. Physical Examination Abnormalities by SOC and PT / SAF

System Organ Class Preferred Term	Statistic	Amifampridine (N=xx)
No. of patients with at least one abnormality	n (%)	xx (xx.x%)
System Organ Class 1	n (%)	xx (xx.x%)
Preferred Term 1	n (%)	xx (xx.x%)
Preferred Term 2	n (%)	xx (xx.x%)
Preferred Term x	n (%)	xx (xx.x%)
System Organ Class 2	n (%)	xx (xx.x%)
Preferred Term 1	n (%)	xx (xx.x%)
Preferred Term 2	n (%)	xx (xx.x%)
Preferred Term x	n (%)	xx (xx.x%)
System Organ Class x	n (%)	xx (xx.x%)
Preferred Term 1	n (%)	xx (xx.x%)
Preferred Term 2	n (%)	xx (xx.x%)
Preferred Term x	n (%)	xx (xx.x%)

Abnormalities are those reported in the "Physical Examination" form of the eCRF.
Abnormalities were coded using the MedDRA dictionary version 23. Primary System Organ Class and Preferred Term are presented.
Patients are only counted once per SOC and PT.
Patients can have more than one abnormality.
Percentages are calculated on the number of patients (N) in the SAF analysis set.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.7.4.

Table 14.2.1. Duration of Treatment / SAF

	Amifampridine (N=xx)
Duration of treatment (days)	
n	xx
Mean (SD)	xx.x (xx.xx)
Median	xx.x
Min, Max	xx, xx

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.2.1.

Table 14.2.3. Amifampridine Dosage by Visit / SAF

Visit: Screening	Statistic	Amifampridine (N=xx)
Dose frequency		
3 times per day	n (%)	xx (xx.x)
4 times per day	n (%)	xx (xx.x)
Amifampridine total daily dose (mg)		
	n	xx
	Mean (SD)	xx.x (xx.xx)
	Median	xx.x
	Min, Max	xx, xx

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.1.2.

Programming Note: This table will be repeated for all visits (Screening, End of study month 3, End of study month 6, End of study month 9, End of study month 12, End of study month 15, End of study month 21, End of study month 27, EoS, Extra visit).

Table 14.2.4. Treatment Compliance / SAF

	Statistic	Amifampridine (N=xx)
Compliance (%)	n	xx
	Mean (SD)	xx.xx (xx.xxx)
	Median	xx.xx
	Min, Max	xx.x, xx.x

Compliance is 100%*(Number consumed)/(Number prescribed).

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.2.1.

Table 14.3.1. Summary of Treatment Emergent Adverse Events (TEAEs) / SAF

	Statistic	Amifampridine (N=XX)
No. of patients in SAF Population	n	xx
No. of TEAEs	n	xx
No. of related TEAEs	n	xx
No. of serious TEAEs	n	xx
No. of serious related TEAEs	n	xx
No. of TEAEs leading to premature discontinuation	n	xx
No. of TEAEs leading to hospitalization	n	xx
No. of life-threatening TEAEs	n	xx
No. of patients with at least one TEAE	n (%)	xx (xx.x%)
No. of patients with at least one related TEAE	n (%)	xx (xx.x%)
No. of patients with at least one TESAE	n (%)	xx (xx.x%)
No. of patients with at least one related TESAE	n (%)	xx (xx.x%)
No. of patients with at least one TEAE leading to premature discontinuation	n (%)	xx (xx.x%)
No. of patients hospitalized due to a TEAE	n (%)	xx (xx.x%)
No. of patients with at least one life-threatening	n (%)	xx (xx.x%)
No. of patients dead	n (%)	xx (xx.x%)

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
TESAE = Treatment Emergent Serious Adverse Events.
Treatment Emergent Adverse Events (TEAE) were defined as "Related" if the relationship to study drug was assessed as "Possible" or "Probable".
Missing relationship category was considered as related to the study drug.
Percentages are calculated relative to the number of patients in SAF population.
N = number of patients, % = percentage of patients.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.1.

Table 14.3.2. Summary of TEAEs by SOC and PT / SAF

	Statistic	Amifampridine (N=XX)
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
The denominator for the calculation of the percentage is the total number of patients who received the treatment and the numerator is the number of patients who received the treatment with at least one TEAE in the given System Organ Class (SOC)
n = number of patients, % = percentage of patients, E = number of events.
Each patient is counted at most once within each SOC and PT.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.1.

Table 14.3.3. Summary of Related TEAEs by SOC and PT / SAF

	Statistic	Amifampridine (N=XX)
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
TEAEs were defined as "Related" if the relationship to study drug was assessed as "Possible" or "Probable".
Missing relationship category was considered as related to the study drug.
The denominator for the calculation of the percentage is the total number of patients who received the treatment and the numerator is the number of patients who received the treatment with at least one TEAE in the given System Organ Class (SOC)
n = number of patients, % = percentage of patients, E = number of events.
Each patient is counted at most once within each SOC and PT.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.3.

Table 14.3.4. Summary of TESAEs by SOC and PT / SAF

	Statistic	Amifampridine (N=XX)
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
The denominator for the calculation of the percentage is the total number of patients who received the treatment and the numerator is the number of patients who received the treatment with at least one TEAE in the given System Organ Class (SOC)
n = number of patients, % = percentage of patients, E = number of events.
Each patient is counted at most once within each SOC and PT.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.2.

Table 14.3.5. Summary of Related TESAEs by SOC and PT / SAF

	Statistic	Amifampridine (N=XX)
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
TESAEs were defined as “Related” if the relationship to study drug was assessed as “Possible” or “Probable”.
Missing relationship category was considered as related to the study drug.
The denominator for the calculation of the percentage is the total number of patients who received the treatment and the numerator is the number of patients who received the treatment with at least one TEAE in the given System Organ Class (SOC)
n = number of patients, % = percentage of patients, E = number of events.
Each patient is counted at most once within each SOC, PT.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.4.

Table 14.3.6. Summary of TEAEs Leading to Hospitalization by SOC and PT / SAF

	Statistic	Amifampridine (N=XX)
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
The denominator for the calculation of the percentage is the total number of patients who received the treatment and the numerator is the number of patients who received the treatment with at least one TEAE in the given System Organ Class (SOC)
n = number of patients, % = percentage of patients, E = number of events.
Each patient is counted at most once within each SOC and PT.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.5.

Table 14.3.7. Summary of Life-Threatening TEAEs by SOC and PT / SAF

	Statistic	Amifampridine (N=XX)
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
The denominator for the calculation of the percentage is the total number of patients who received the treatment and the numerator is the number of patients who received the treatment with at least one TEAE in the given System Organ Class (SOC)
n = number of patients, % = percentage of patients, E = number of events.
Each patient is counted at most once within each SOC and PT.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.6.

Table 14.3.8. Summary of TEAEs Leading to Premature Discontinuation by SOC and PT / SAF

	Statistic	Amifampridine (N=XX)
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<Primary SOC>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx
<PT>	n (%) E	xx (xx.x%) xx

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
The denominator for the calculation of the percentage is the total number of patients who received the treatment and the numerator is the number of patients who received the treatment with at least one TEAE in the given System Organ Class (SOC)
n = number of patients, % = percentage of patients, E = number of events.
Each patient is counted at most once within each SOC and PT.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.7.

Table 14.3.9. Summary of TEAEs by SOC and PT and Maximum Severity / SAF

		Amifampridine				
	Statistic	Grade 1	Grade 2	Grade 3	Grade 4	Grade 5
<Primary SOC>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<Primary SOC>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<Primary SOC>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
The denominator for the calculation of the percentage is the total number of patients who received the treatment and the numerator is the number of patients who received the treatment with at least one TEAE in the given System Organ Class (SOC)
n = number of patients, % = percentage of patients, E = number of events.
Each patient is counted at most once within each SOC and PT.
If a patient experienced more than one TEAE with the same PT or primary SOC, the TEAE with the maximum severity was counted.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.1.

Table 14.3.10. Summary of TEAEs by SOC and PT and Relationship to Treatment / SAF

		Amifampridine		
	Statistic	Not related	Possibly	Probably
<Primary SOC>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<Primary SOC>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<Primary SOC>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx
<PT>	n (%) E	xx(xx.x%)xx	xx(xx.x%)xx	xx(xx.x%)xx

TEAE = any AE started at or after the first dose of study drug for the current Long-Term Study.
The denominator for the calculation of the percentage is the total number of patients who received the treatment
and the numerator is the number of patients who received the treatment with at least one TEAE in the given System Organ Class (SOC)
n = number of patients, % = percentage of patients, E = number of events.
Each patient is counted at most once within each SOC and PT.
If a patient experienced more than one TEAE with the same PT or primary SOC, the TEAE with the worst relationship was counted.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.5.1.

Table 14.4.1. Summary of Clinical Laboratory Tests: Haematology / SAF

	Actual Values						Change from Screening					
Visit	n	Mean	SD	Min	Median	Max	n	Mean	SD	Min	Median	Max
Screening	xx	xx.x	xx.x	xx.x	xx.x	xx.x						
Month 3	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 6	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 9	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 12	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 15	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 21	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 27	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
EOS	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x

Change from Screening includes only those patients with both a Screening value and a value for summarized time period.
n represents number of patients contributing to summary statistics.
Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.6.2.

Programming Notes: Continue for the following haematology parameters:

a) Haemoglobin
b) Haematocrit
c) WBC count
d) RBC count
e) Platelet count
f) Differential cell count

Values must be presented in SI unit.

Table 14.4.2. Summary of Clinical Laboratory Tests: Blood Chemistry / SAF

	Actual Values						Change from Screening					
Visit	n	Mean	SD	Min	Median	Max	n	Mean	SD	Min	Median	Max
Screening	xx	xx.x	xx.x	xx.x	xx.x	xx.x						
Month 3	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 6	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 9	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 12	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 15	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 21	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 27	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
EOS	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x

Change from Screening includes only those patients with both a Screening value and a value for summarized time period.
n represents number of patients contributing to summary statistics.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.6.1.

Programming Notes: Continue for the following blood chemistry parameters:

g) *Albumin*
h) *Alkaline phosphatase*
i) *ALT (SGPT)*
j) *AST (SGOT)*
k) *Direct bilirubin*
l) *Total bilirubin*
m) *BUN*
n) *Calcium*
o) *Chloride*
p) *Total cholesterol*
q) *Bicarbonate*
r) *Creatine phosphokinase*
s) *Creatinine*
t) *Glucose*
u) *GGT*
v) *LDH*
w) *Phosphorus*
x) *Potassium*
y) *Total protein*
z) *Sodium*
aa) *Uric Acid*

Values must be presented in SI unit.

Table 14.4.3. Summary of Haematology Shift Result Relative to the Normal Range / SAF

			Screening		
Parameter (Units)	Visit		Low	Normal	High
Haemoglobin (g/L)	Month 3	n	xx	xx	xx
		Low	xx (xx.x)	xx (xx.x)	xx (xx.x)
		Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
		High	xx (xx.x)	xx (xx.x)	xx (xx.x)
	…	n	xx	xx	xx
		Low	xx (xx.x)	xx (xx.x)	xx (xx.x)
		Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
		High	xx (xx.x)	xx (xx.x)	xx (xx.x)
	EOS	n	xx	xx	xx
		Low	xx (xx.x)	xx (xx.x)	xx (xx.x)
		Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
		High	xx (xx.x)	xx (xx.x)	xx (xx.x)

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.6.2.

Programming Notes: Continue for all Haematology parameters.

Table 14.4.4. Summary of Blood Chemistry Shift Result Relative to the Normal Range / SAF

Parameter (Units)	Visit		Screening		
			Low	Normal	High
AST (U/L)	Month 3	n	xx	xx	xx
		Low	xx (xx.x)	xx (xx.x)	xx (xx.x)
		Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
		High	xx (xx.x)	xx (xx.x)	xx (xx.x)
	…	n	xx	xx	xx
		Low	xx (xx.x)	xx (xx.x)	xx (xx.x)
		Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
		High	xx (xx.x)	xx (xx.x)	xx (xx.x)
	EOS	n	xx	xx	xx
		Low	xx (xx.x)	xx (xx.x)	xx (xx.x)
		Normal	xx (xx.x)	xx (xx.x)	xx (xx.x)
		High	xx (xx.x)	xx (xx.x)	xx (xx.x)

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.6.1.

Programming Notes: Continue for all Blood Chemistry parameters.

Table 14.5.1. Summary of Vital Signs: Systolic Blood Pressure / SAF

	Actual Values						Change from Screening					
Visit	n	Mean	SD	Min	Median	Max	n	Mean	SD	Min	Median	Max
Screening	xx	xx.x	xx.x	xx.x	xx.x	xx.x						
Month 3	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 6	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 9	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 12	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 15	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 21	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 27	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
EOS	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x

Change from Baseline includes only those patients with both a Baseline value and a value for summarized time period.
n represents number of patients contributing to summary statistics.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.7.2.

Programming Notes: Continue for the following parameters:

a) Diastolic Blood Pressure (mmHg)
b) Heart Rate (beats/min)
c) Respiration Rate (breaths/min)
d) Body Temperature (°C)
e) Body weight (Kg)

Table 14.5.2. 12-Lead ECG / SAF

	Statistic	Amifampridine (N=XX)
Screening		xx (x.x)
Normal tracing	n (%)	xx (x.x)
Abnormal tracing, NCS	n (%)	xx (x.x)
Abnormal tracing, CS	n (%)	xx (x.x)
Unknown	n (%)	xx (x.x)
Month 3		xx (x.x)
Normal tracing	n (%)	xx (x.x)
Abnormal tracing, NCS	n (%)	xx (x.x)
Abnormal tracing, CS	n (%)	xx (x.x)
Unknown	n (%)	xx (x.x)
…		
EOS		xx (x.x)
Normal tracing	n (%)	xx (x.x)
Abnormal tracing, NCS	n (%)	xx (x.x)
Abnormal tracing, CS	n (%)	xx (x.x)
Unknown	n (%)	xx (x.x)

CS: Clinically Significant.
NCS: Not Clinically Significant.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.7.3.

Table 14.5.3. Childbearing Potential and Pregnancy Test / SAF

Visit		Statistic	Amifampridine (N=xx)
Screening	Childbearing potential		
	Patient male	n (%)	xx (xx.x%)
	Menopause	n (%)	xx (xx.x%)
	Sterilized	n (%)	xx (xx.x%)
	Total hysterectomy	n (%)	xx (xx.x%)
	No contraception protection	n (%)	xx (xx.x%)
	Contraception protection	n (%)	xx (xx.x%)
	Child	n (%)	xx (xx.x%)
	Unknown	n (%)	xx (xx.x%)
	Pregnancy test performed?		
	No	n (%)	xx (xx.x%)
	Yes	n (%)	xx (xx.x%)
	Not applicable	n (%)	xx (xx.x%)
	Type of pregnancy test (#)		
	Urine dipstick	n (%)	xx (xx.x%)
	Serum	n (%)	xx (xx.x%)
	Unknown	n (%)	xx (xx.x%)
	Pregnancy test result (#)		
	Negative	n (%)	xx (xx.x%)
	Positive	n (%)	xx (xx.x%)
	Unknown	n (%)	xx (xx.x%)

Percentages are calculated on the number of patients (N) in the SAF analysis set.
Percentages are based on the number of female patients performing pregnancy test.
Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.7.1.

Programming Notes: To be repeated for Month 3, Month 6, Month 9, Month 12, Month 15, Month 21, Month 27 and EOS visits.

Table 14.6.1. Summary of INQoL: Weakness / ITT_{QOL}

	Actual Values						Change from Screening					
Visit	n	Mean	SD	Min	Median	Max	n	Mean	SD	Min	Median	Max
Screening	xx	xx.x	xx.x	xx.x	xx.x	xx.x						
Month 3	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 6	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 9	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 12	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 15	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 21	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 27	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
EOS	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x

Change from Baseline includes only those patients with both a Baseline value and a value for summarized time period.
n represents number of patients contributing to summary statistics.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.7.4.1., 16.2.7.4.2., 16.2.7.4.3., 16.2.7.4.4., 16.2.7.4.5., 16.2.7.4.6.

Programming Notes: Continue for all INQoL domains:

a) Symptoms (section 1): Muscle "locking", Pain, Fatigue, Droopy eyelids, Double vision, and Swallowing difficulties.
b) Life domains (section 2): Activities, Independence, Social relationship, Emotions, and Body image.
c) Life domains (section 3): Perceived treatment effects and Expected treatment effects.
d) Quality of Life.
e) Provide graphs of means and SD values over the study as follows:

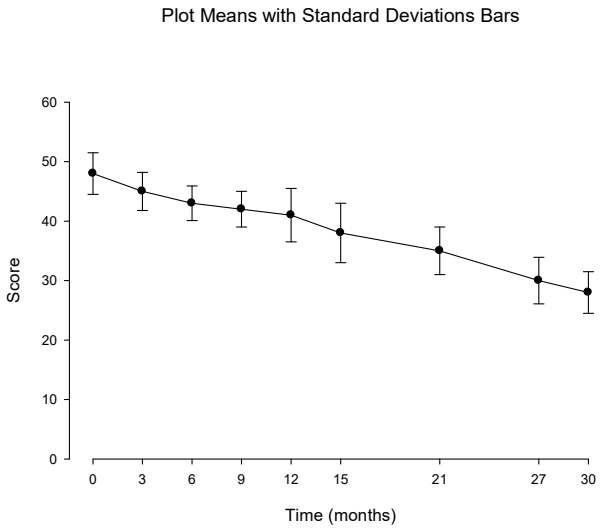


Table 14.6.2. Summary of PEDSQL: About my Neuromuscular Disease / ITT_{QOL}

	Actual Values						Change from Screening					
Visit	n	Mean	SD	Min	Median	Max	n	Mean	SD	Min	Median	Max
Screening	xx	xx.x	xx.x	xx.x	xx.x	xx.x						
Month 3	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 6	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 9	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 12	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 15	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 21	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
Month 27	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x
EOS	xx	xx.x	xx.x	xx.x	xx.x	xx.x	xx	xx.x	xx.x	xx.x	xx.x	xx.x

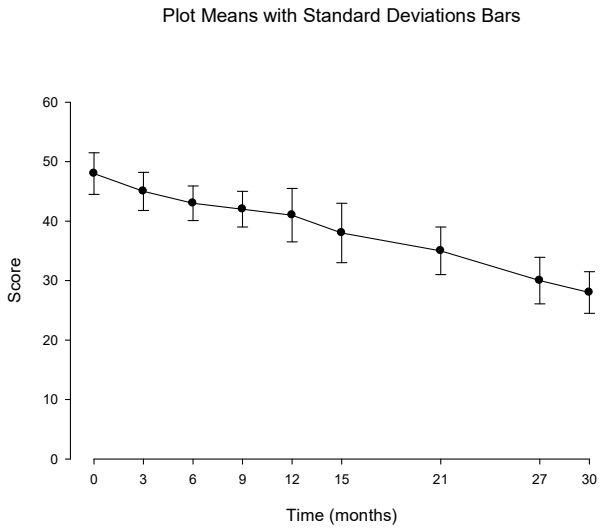
Change from Baseline includes only those patients with both a Baseline value and a value for summarized time period.
n represents number of patients contributing to summary statistics.

Source: xxx.sas, Run on ddmmmyyyy

Reference Listings: 16.2.7.1.1., 16.2.7.1.2., 16.2.7.2.1., 16.2.7.2.2., 16.2.7.3.1., 16.2.7.3.2.

Programming Notes: Continue for all PEDSQL domains:

a) *Communication*
b) *About our family resources*
c) *Total score*
d) *Provide graphs of means and SD values over the study as follows:*



Listing 16.2.1. Completed and Discontinued Patients with Primary Reason for Discontinuation

Site	Patient	SAF population	ITT_{QOL} population	Complete the treatment? (1)	If No, primary reason for discontinuation	If reason is AE, Major Protocol Deviation, or Other Reason, specify	If reason is Consent Withdrawal, specify the date
xxx	xxx	Yes	Yes	Yes			
xxx	xxx	Yes	Yes	Yes			
xxx	xxx	No	No	No	Consent Withdrawal		DDMMMYYYY
xxx	xxx	Yes	Yes	No	Adverse event	Xxxxxxxxxx	
xxx	xxx	Yes	Yes	No	xxxxxxxxxx		

SAF=Safety Population
ITT_{QOL}=Efficacy Population

(1) Did the patient complete the full course of treatment?

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number.

Listing 16.2.2.1 Inclusion Criteria

Individuals eligible to participate in this study must meet all the following inclusion criteria:

1 Participated in the SMA-001 study

2 Willing and able to provide written informed consent after the nature of the study has been explained and before the start of any research-related procedures.

3 Female patients of childbearing potential defined according to the Clinical Trial Facilitation Group - CTFG guidelines# must have a negative pregnancy test (urine human chorionic gonadotropin [HCG] at the end of SMA-001 study); and must practice an effective, reliable contraceptive regimen during the study and for up to 30 days following discontinuation of treatment

4 Ability to participate in the study based on overall health of the patient and disease prognosis, as applicable, in the opinion of the Investigator; and able to comply with all requirements of the protocol, including completion of study questionnaires.

Listing 16.2.2.1. Inclusion Criteria

					Criterion (1)			
Site	Patient	Date of visit	Consent date	Study Subject ID in SMA-001 study	1.	2.	3.	4.
xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx
xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx
xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx
xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx

See first page for criteria.
(1) Response for each criterion: Yes, No, Not Applicable
Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number. Place the criteria on the first page.

Listing 16.2.2.2. Exclusion Criteria

Individuals who meet any of the following exclusion criteria are not eligible to participate in the study:

[According to Study Protocol version 2.0]

1 Epilepsy and currently on medication.
2 Uncontrolled asthma.
3 Concomitant use with sultopride.
4 Concomitant use with medicinal products with a narrow therapeutic window.
5 Concomitant use with medicinal products with a known to cause QTc prolongation.
6 Clinically significant abnormalities in 12 lead ECG, in the opinion of the Investigator.
7 Subjects with congenital QT syndromes.
8 Breastfeeding or pregnant at Screening or planning to become pregnant at any time during the study.
9 Intolerable amifampridine-related side effects
10 Treatment with an investigational drug (other than amifampridine) or device while participating in this study.
11 Any medical condition that, in the opinion of the Investigator, might interfere with the patient's participation in the study, poses an added risk for the patient, or confound the assessment of the patient.
12 History of drug allergy to any pyridine-containing substances or any amifampridine excipient(s).

[According to Study Protocol version 3.0]

1 Epilepsy and currently on medication.
2 Uncontrolled asthma.
3 Concomitant use with sultopride.
4 Concomitant use with medicinal products with a narrow therapeutic window.
5 Clinically significant abnormalities in 12 lead ECG, in the opinion of the Investigator.
6 Breastfeeding or pregnant at Screening or planning to become pregnant at any time during the study.
7 Intolerable amifampridine-related side effects
8 Treatment with an investigational drug (other than amifampridine) or device while participating in this study.
9 Any medical condition that, in the opinion of the Investigator, might interfere with the patient's participation in the study, poses an added risk for the patient, or confound the assessment of the patient.
10 History of drug allergy to any pyridine-containing substances or any amifampridine excipient(s).

Listing 16.2.2.2. Exclusion Criteria

				Criterion (1)												
Site	Patient	Date of visit	Consent date	Protocol version	1.	2.	3.	4.	5.	6.	7.	8.	9.	10.	11.	12
xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xx
xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xx
xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xx
xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xx

See first page for criteria.
(1) Response for each criterion: Yes, No, Not Applicable
Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number. Place the criteria on the first page.

Listing 16.2.3.1. Protocol Deviations

Site	Patient	PD number	Deviation date	Date identified	Deviation identified by	Deviation description	Follow-up/ Resolution	Type of deviation
xxx	xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	Xxxxxxxxxx	Xxxxxxxxxx	Major
xxx	xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	Xxxxxxxxxx	Xxxxxxxxxx	Minor
xxx	xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	Xxxxxxxxxx	Xxxxxxxxxx	xxxxxxxxxx
		xxx	DDMMMYYYY	DDMMMYYYY	xxx	Xxxxxxxxxx	Xxxxxxxxxx	xxxxxxxxxx
		xxx	DDMMMYYYY	DDMMMYYYY	xxx	Xxxxxxxxxx	Xxxxxxxxxx	xxxxxxxxxx
xxx	xxx	xxx	DDMMMYYYY	DDMMMYYYY	xxx	Xxxxxxxxxx	Xxxxxxxxxx	xxxxxxxxxx

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number and deviation date.

Listing 16.2.3.2. Patients Excluded from the Analysis Populations

Site	Patient	Set from which patient is excluded (1)	Reason for exclusion
xxx	xxx	xxxxxxx	Xxxxxxx
xxx	xxx	xxxxxxx	Xxxxxxx
xxx	xxx	xxxxxxx	Xxxxxxx
xxx	xxx	xxxxxxx	Xxxxxxx

(1) SAF=Safety Population, ITT_{QOL}=Efficacy Population

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number.

Listing 16.2.4. Demographic Characteristics

Site	Patient	Age (years)	Date of birth	Race	If Other, specify	Gender	Height (cm)	Weight (kg)	BMI (kg/cm^2)
xxx	xxx	xx	DDMMMYYYY	xxxxx		xxxxx	xxx	xxx	xxx
xxx	xxx	xx	DDMMMYYYY	xxxxx		xxxxx	xxx	xxx	xxx
xxx	xxx	xx	DDMMMYYYY	xxxxx		xxxxx	xxx	xxx	xxx
xxx	xxx	xx	DDMMMYYYY	Other	Xxxxx	xxxxx	xxx	xxx	xxx

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number.

Listing 16.2.4.2.1. Medical History: SMA Characteristics

Site	Patient	Year of onset of SMA symptoms	Was the patient previously treated for SMA other than amifampridine phosphate?	If YES, treatment specify	Date of SMN gene analysis	SMN gene analysis result
xxx	xxx	xx	xxx	xxx	DDMMMYYYY	xxxxx
xxx	xxx	xx	xxx	xxx	DDMMMYYYY	xxxxx
xxx	xxx	xx	xxx	xxx	DDMMMYYYY	xxxxx
xxx	xxx	xx	xxx	xxx	DDMMMYYYY	xxxxx

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number.

Listing 16.2.4.2.2. Medical History: Previous and Concomitant Diseases

Site	Patient	Body system	Involved (1)	If Yes, specify diagnosis	System Organ Class	Preferred Term	Onset date	Present at study start? (2)
xxx	xxx	Cardiovascular	Yes	xxxxxxxxx	xxxxxxxxx	xxxxxxxxx	DDMMMYYYY	xxx
		Respiratory	No					
		Muscle-Skeletal	No					
		Ears, nose, eyes and throat	Yes	xxxxxxxxx	xxxxxxxxx	xxxxxxxxx	DDMMMYYYY	xxx
		.						
		.						
xxx	xxx	Cardiovascular	Yes	xxxxxxxxx	xxxxxxxxx	xxxxxxxxx	DDMMMYYYY	xxx
		Respiratory	Yes	xxxxxxxxx	xxxxxxxxx	xxxxxxxxx	DDMMMYYYY	xxx
		Muscle-Skeletal	Yes	xxxxxxxxx	xxxxxxxxx	xxxxxxxxx	DDMMMYYYY	xxx
		Ears, nose, eyes and throat	Yes	xxxxxxxxx	xxxxxxxxx	xxxxxxxxx	DDMMMYYYY	xxx
		.						
		.						

(1) Involved: No, Yes, Not Examined
(2) Present at study start: No, Yes, UNK
Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Continue for Other Body systems ("Hepatobiliary", "Endocrine & Metabolic", "Haemapoietic", "Central Nervous", "Dermatologic", "Genitourinary", "Gastrointestinal", "Psychiatric Diseases", "Allergies" and "Other Body System"). There may be multiple entries for "Other". Sort by site and patient number.

Listing 16.2.5.1.1. Amifampridine Treatment at Screening

Site	Patient	Screening failure?	Are all inclusion and exclusion criteria satisfied?	Inclusion/ Exclusion criteria number(s) not met	First dose (mg)	Timing of first dose (hh:mm)	Second dose (mg)	Timing of second dose (hh:mm)	Third dose (mg)	Timing of third dose (hh:mm)	Fourth dose prescr ibed?	Fourth dose (mg)	Timing of fourth dose (hh:mm)	Total daily dose (mg)	Date of adminsit ation of the firs dose
xxx	xxx	xxx	xxx	xxx	xxx	xx:xx	xxx	xx:xx	xxx	xx:xx	xxx	xxx	xx:xx	xxx	DDMMMYYY
xxx	xxx	xxx	xxx	xxx	xxx	xx:xx	xxx	xx:xx	xxx	xx:xx	xxx	xxx	xx:xx	xxx	DDMMMYYY
xxx	xxx	xxx	xxx	xxx	xxx	xx:xx	xxx	xx:xx	xxx	xx:xx	xxx	xxx	xx:xx	xxx	DDMMMYYY
xxx	xxx	xxx	xxx	xxx	xxx	xx:xx					No				

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number.

Listing 16.2.5.1.2. Amifampridine Treatment Over the Study

Site	Patient	First dose (mg)	Timing of first dose (hh:mm)	Second dose (mg)	Timing of second dose (hh:mm)	Third dose (mg)	Timing of third dose (hh:mm)	Fourth dose prescribed?	Fourth dose (mg)	Timing of fourth dose (hh:mm)	Total daily dose (mg)
xxx	xxx	xxx	xx:xx	xxx	xx:xx	xxx	xx:xx	xxx	xxx	xx:xx	xxx
xxx	xxx	xxx	xx:xx	xxx	xx:xx	xxx	xx:xx	xxx	xxx	xx:xx	xxx
xxx	xxx	xxx	xx:xx	xxx	xx:xx	xxx	xx:xx	xxx	xxx	xx:xx	xxx
xxx	xxx	xxx	xx:xx					No			

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number.

Listing 16.2.5.2.1 Duration and Compliance over the Study

Site	Patient	Number of tablets dispensed	Number of tablets used	Treatment duration (days)	Compliance (%)
xxx	xxx	xxx	xxx	xxx	xxx.x
xxx	xxx	xxx	xxx	xxx	xxx.x
xxx	xxx	xxx	xxx	xxx	xxx.x
xxx	xxx	xxx	xxx	xxx	xxx.x
xxx	xxx	xxx	xxx	xxx	xxx.x

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site and patient number. Number of Tablets Dispensed, Number of Tablets Used, and Treatment Duration are totals from next listing. Compliance is determined from these totals.

Listing 16.2.5.2.2 Study Drug Accountability

Site	Patient	Date of IP dispensing	Visit (1)	Box kit number	No. of tablets dispensed	Start date of period covered with this box	End date of period covered with this box	No. of tablets returned	No. of tablets used	No. of tablets lost	Total tablets used, returned and lost (calculated)	Divided in the following frequency (2)	Total daily dose (mg) (3)
xxx	xxx	DDMMMYYYY	xx	xx	xx	DDMMMYYYY	DDMMMYYYY	xx	xx	xx	xx	xx	xx
		DDMMMYYYY	xx	xx	xx	DDMMMYYYY	DDMMMYYYY	xx	xx	xx	xx	xx	xx
		DDMMMYYYY	xx	xx	xx	DDMMMYYYY	DDMMMYYYY	xx	xx	xx	xx	xx	xx
xxx	xxx	DDMMMYYYY	xx	xx	xx	DDMMMYYYY	DDMMMYYYY	xx	xx	xx	xx	xx	xx
		DDMMMYYYY	xx	xx	xx	DDMMMYYYY	DDMMMYYYY	xx	xx	xx	xx	xx	xx
		DDMMMYYYY	xx	xx	xx	DDMMMYYYY	DDMMMYYYY	xx	xx	xx	xx	xx	xx

(1) Visit: Screening, End of study month 3, End of study month 6, End of study month 9, End of study month 12, End of study month 15, End of study month 21, End of study month 27, EoS, Extra visit

(2) Divided in the following frequency: 3 times per day, 4 times per day

(3) Total Daily Dose (mg): 5, 10, 15, 20, 25, 30, 35, 40, 45, 50, 55, 60, 65, 70, 75, 80

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number and date of IP dispensing.

Listing 16.2.6.1. Quality of Life (QoL): Type of Questionnaire

Site	Patient	Visit	QoL questionnaire performed?	If No, specify reason	Date of test performed	Time of test performed (hh:mm)	Type of questionnaire (1)
xxx	xxx	Screening	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
		Month 3	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
		Month 6	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
		Month 9	No	xxxxxxxxx			
		…	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
		EOS	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
xxx	xxx	Screening	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
		Month 3	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
		Month 6	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
		Month 9	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
		…	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx
		EOS	xxx		DDMMMYYYY	xx:xx	xxxxxxxxx

(1) Type of questionnaire: PEDSQL Parent Report for Young Child (ages 5-7), PEDSQL Parent Report for Child (ages 8-12), PEDSQL teen (ages 13-18), INQOL (ages > 18)

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

Listing 16.2.7.1.1 PEDSQL Parent Report for Young Child (5-7 years): About My Child's Neuromuscular Disease (Items from 1 to 17)

			Items Section About My Child's Neuromuscular Disease (1)															
Site	Patient	Visit	1	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 3	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 6	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 9	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		…	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		EOS	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx

Statements are given after the next listing.
(1) Each item response: 1=Never, 2=Almost never, 3=Sometimes, 4=Often, 5=Almost always, -1=UNK
Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

Listing 16.2.7.1.2. PEDSQL Parent Report for Young Child (5-7 years): Communication (Items from 1 to 3), About our Family Resources (Items from 1 to 5) and Questionnaire Scores

			Items Section Communication (1)			Items Section About our Family Resources (1)					Score			
Site	Patient	Visit	1	2	3	1	2	3	4	5	Score section 1	Score section 2	Score section 3	Total score
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 3	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 6	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 9	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		…	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		EOS	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx

Items follow this listing.

(1) Each item response: 1=Never, 2=Almost never, 3=Sometimes, 4=Often, 5=Almost always, -1=UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

PEDSQL Parent Report for Young Child (5-7 years) Items

	About My Child's neuromuscular disease
1	It's hard for my child to breathe
2	My child gets sick easily
3	My child gets sores and/or rashes
4	My child's leg hurt
5	My child feels tired
6	My child's back feels stiff
7	My child wakes up tired
8	My child's hands are weak
9	It is hard for my child to use the bathroom
10	It's hard for my child to gain/lose weight when he/she wants to
11	It's hard for my child to use his/her hands
12	It's hard for my child to swallow food
13	It takes my child a long time to bathe/shower
14	My child gets hurt accidentally
15	My child takes a long time to eat
16	It's hard for my child to turn him/herself during the night
17	It's hard for my child to go places with his/her equipment
	Communication
1	It's hard for my child to tell the doctors/nurses how he/she feels
2	It's hard for my child to ask the doctors/nurses questions
3	It's hard for my child to explain his/her illness to other people
	About our Family Resources
1	It's hard for our family to plan activities like vacations
2	It's hard for our family to get enough rest
3	I think money is a problem in our family
4	I think our family has a lot of problems
5	My child does not have the equipment he/she needs

Listing 16.2.7.2.1. PEDSQL Parent Report for Child (8-12 years): About My Child's Neuromuscular Disease (Items from 1 to 17)

			Items Section About My Child's Neuromuscular Disease (1)															
Site	Patient	Visit	1	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 3	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 6	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 9	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		…	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		EOS	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx

Statements are given after the next listing.
(1) Each item response: 1=Never, 2=Almost never, 3=Sometimes, 4=Often, 5=Almost always, -1=UNK
Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

Listing 16.2.7.2.2. PEDSQL parent Report for Child (8-12 years): Communication (Items from 1 to 3), About our Family Resources (Items from 1 to 5) and Questionnaire Scores

			Items Section Communication (1)			Items Section About our Family Resources (1)					Score			
Site	Patient	Visit	1	2	3	1	2	3	4	5	Score section 1	Score section 2	Score section 3	Total score
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 3	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 6	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 9	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		…	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		EOS	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx

Items follow this listing.

(1) Each item response: 1=Never, 2=Almost never, 3=Sometimes, 4=Often, 5=Almost always, -1=UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

PEDSQL parent Report for Child (8-12 years) Items

	About My Child's neuromuscular disease
1	It's hard for my child to breathe
2	My child gets sick easily
3	My child gets sores and/or rashes
4	My child's leg hurt
5	My child feels tired
6	My child's back feels stiff
7	My child wakes up tired
8	My child's hands are weak
9	It is hard for my child to use the bathroom
10	It's hard for my child to gain/lose weight when he/she wants to
11	It's hard for my child to use his/her hands
12	It's hard for my child to swallow food
13	It takes my child a long time to bathe/shower
14	My child gets hurt accidentally
15	My child takes a long time to eat
16	It's hard for my child to turn him/herself during the night
17	It's hard for my child to go places with his/her equipment
	Communication
1	It's hard for my child to tell the doctors/nurses how he/she feels
2	It's hard for my child to ask the doctors/nurses questions
3	It's hard for my child to explain his/her illness to other people
	About our Family Resources
1	It's hard for our family to plan activities like vacations
2	It's hard for our family to get enough rest
3	I think money is a problem in our family
4	I think our family has a lot of problems
5	My child does not have the equipment he/she needs

Listing 16.2.7.3.1. PEDSQL Teen Report (13-18 years): About My Neuromuscular Disease (Items from 1 to 17)

			Items section About My Neuromuscular Disease (1)																
Site	Patient	Visit	1	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 3	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 6	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 9	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		…	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		EOS	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx

Statements are given after the next listing.
(1) Each item response: 1=Never, 2=Almost never, 3=Sometimes, 4=Often, 5=Almost always, -1=UNK
Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

Listing 16.2.7.3.2. PEDSQL Teen Report (13-18 years): Communication (Items from 1 to 3), About our Family Resources (Items from 1 to 5) and Questionnaire Scores

			Items Section Communication (1)			Items Section About our Family Resources (1)					Score			
Site	Patient	Visit	1	2	3	1	2	3	4	5	Score section 1	Score section 2	Score section 3	Total score
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 3	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 6	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 9	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		…	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		EOS	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx

Items follow this listing.

(1) Each item response: 1=Never, 2=Almost never, 3=Sometimes, 4=Often, 5=Almost always, -1=UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

PEDSQL Teen Report (13-18 years) Items

	About My Neuromuscular Disease
1	It's hard to breathe
2	I get sick easily
3	I get sores and/or rashes
4	My legs hurt
5	I feel tired
6	My back feels stiff
7	I wake up tired
8	My hands are weak
9	It's hard to use the bathroom
10	It's hard to gain or lose weight when I want to
11	It's hard to use my hands
12	It's hard to swallow food
13	It takes me a long time to bathe or shower
14	I get hurt accidentally
15	I take a long time to eat
16	It's hard to turn myself during the night
17	It's hard for me to go places with my equipment
	Communication
1	It's hard for me to tell the doctors and nurses how I feel
2	It's hard for me to ask the doctors and nurses questions
3	It's hard for me to explain my illness to other people
	About our Family Resources
1	It's hard for my family to plan activities like vacations
2	It's hard for my family to get enough rest
3	I think money is a problem in our family
4	I think my family has a lot of problems
5	I do not have the equipment I need

Listing 16.2.7.4.1. INQOL: Items from Section 1-1 to Section 1-5C

			Items section 1																		
Site	Patient	Visit	1	1A	1B	1C	2	2A	2B	2C	3	3A	3B	3C	4	4A	4B	4C	5	5A	5B	5C
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 3	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 6	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 9	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		…	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		EOS	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx

Statements are given after the next listings.

Items 1/2/3/4/5 response: 1=Yes, 0=No, -1=UNK

Items 1A/2A/3A/4A/5A response: 1=Very little, 2=Some, 3=A fair amount, 4=A moderate amount, 5=A considerable amount, 6=A lot, 7=An extreme amount, -1=UNK

Items 1B/2B/3B/4B/5B response: 0=Not at all, 1=Some, 2=A fair amount, 3=A moderate amount, 4=A considerable amount, 5=Very many, 6=An extreme amount, -1=UNK

Items 1C/2C/3C/4C/5C response: 0=Not at all important, 1=Slightly important, 2=Reasonably important, 3=Moderately important, 4=Considerably important, 5=Very important, 6=Extremely important, -1=UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

Listing 16.2.7.4.2. INQOL: Items from Section 1-6 to Section 2-Question 1.B2

			Items section 1								Items section 2							
Site	Patient	Visit	6	6A	6B	6C	7	7A	7B	7C	1.A1	1.A2	1.A3	1.A3 specify 1	1.A3 specify 2	1.B1	1.B2	1.B2 specify
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xxxx	xxxx	xx	xx	xxxx
		Month 3	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xxxx	xxxx	xx	xx	xxxx
		Month 6	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xxxx	xxxx	xx	xx	xxxx
		Month 9	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xxxx	xxxx	xx	xx	xxxx
		…	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xxxx	xxxx	xx	xx	xxxx
		EOS	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xxxx	xxxx	xx	xx	xxxx

Statements are given after the next listings.
Items 6/7 response: 1=Yes, 0=No, -1=UNK
Items 6A/7A response: 1=Very little, 2=Some, 3=A fair amount, 4=A moderate amount, 5=A considerable amount, 6=A lot, 7=An extreme amount, -1=UNK
Items 6B/7B response: 0=None at all, 1=Some, 2=A fair amount, 3=A moderate amount, 4=A considerable amount, 5=Very many, 6=An extreme amount, -1=UNK
Items 6C/7C/1.B2 response: 0=Not at all important, 1=Slightly important, 2=Reasonably important, 3=Moderately important, 4=Considerably important, 5=Very important, 6=Extremely important, -1=UNK
Items 1.A1/1.A2/1.A3 response: 0=Not at all, 1=Slightly, 2=A fair amount, 3=Moderately, 4=Considerably, 5=Very much, 6=Extremely, -1=UNK
Items 1.B1 response: 0= Exactly as I would like it to be, 1=Good but not quite how I would like it to be, 2=OK but not how I would like it to be, 3=Neither good nor bad, 4=Quite bad but it could be much worse, 5=Bad but it could be worse, 6=The worst it could possibly be, -1=UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

Listing 16.2.7.4.3. INQOL: Items from Section 2-Question 2.A to Section 2-Question 3.B6

			Items section 2																	
Site	Patient	Visit	2.A	2.B1	2.B2	2.B2 Specify	3.A1	3.A1 specify	3.A2	3.A3	3.A4	3.B1	3.B2	3.B2 specify	3.B3	3.B4	3.B4 specify	3.B5	3.B6	3.B6 specify
xxx	xxx	Screening	xx	xx	xx	xxxx	xx	xxxx	xx	xx	xx	xx	xx	xxxx	xx	xx	xxxx	xx	xx	xxxx
		Month 3	xx	xx	xx	xxxx	xx	xxxx	xx	xx	xx	xx	xx	xxxx	xx	xx	xxxx	xx	xx	xxxx
		Month 6	xx	xx	xx	xxxx	xx	xxxx	xx	xx	xx	xx	xx	xxxx	xx	xx	xxxx	xx	xx	xxxx
		Month 9	xx	xx	xx	xxxx	xx	xxxx	xx	xx	xx	xx	xx	xxxx	xx	xx	xxxx	xx	xx	xxxx
		…	xx	xx	xx	xxxx	xx	xxxx	xx	xx	xx	xx	xx	xxxx	xx	xx	xxxx	xx	xx	xxxx
		EOS	xx	xx	xx	xxxx	xx	xxxx	xx	xx	xx	xx	xx	xxxx	xx	xx	xxxx	xx	xx	xxxx

Statements are given after the next listings.

Items 2.A/3.A1/3.A2/3.A3/3.A4 response: 0=None at all, 1=Some, 2=A fair amount, 3=A moderate amount, 4=A considerable amount, 5=Very many, 6=An extreme amount, -1=UNK

Items 2.B2/3.B2/3.B4/3.B6 response: 0=Not at all important, 1=Slightly important, 2=Reasonably important, 3=Moderately important, 4=Considerably important, 5=Very important, 6=Extremely important, -1=UNK

Items 2.B1/3.B1/3.B3/3.B5 response: 0= Exactly as I would like it to be, 1=Good but not quite how I would like it to be, 2=OK but not how I would like it to be, 3=Neither good nor bad, 4=Quite bad but it could be much worse, 5=Bad but it could be worse, 6=The worst it could possibly be, -1=UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

Listing 16.2.7.4.4. INQOL: Items from Section 2-Question 4.A1 to Section 3-Question 1.A3

			Items section 2											Items section 3						
Site	Patient	Visit	4.A1	4.A2	4.A3	4.A4	4.B1	4.B2	4.B2 specify	5.A	5.B1	5.B2	5.B2 specify	1.A	1.A1	1.A1 specify 1	1.A1 specify 2	1.A2	1.A2 specify	1.A3
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xxxx	xx	xx	xx	xxxx	xx	xx	xxxx	xxxx	xx	xxxx	xx
		Month 3	xx	xx	xx	xx	xx	xx	xxxx	xx	xx	xx	xxxx	xx	xx	xxxx	xxxx	xx	xxxx	xx
		Month 6	xx	xx	xx	xx	xx	xx	xxxx	xx	xx	xx	xxxx	xx	xx	xxxx	xxxx	xx	xxxx	xx
		Month 9	xx	xx	xx	xx	xx	xx	xxxx	xx	xx	xx	xxxx	xx	xx	xxxx	xxxx	xx	xxxx	xx
		…	xx	xx	xx	xx	xx	xx	xxxx	xx	xx	xx	xxxx	xx	xx	xxxx	xxxx	xx	xxxx	xx
		EOS	xx	xx	xx	xx	xx	xx	xxxx	xx	xx	xx	xxxx	xx	xx	xxxx	xxxx	xx	xxxx	xx

Statements are given after the next listings.

Items 4.A1/4.A2/4.A3/4.A4 response: 0=Not at all, 1=Slightly, 2=A fair bit, 3=Moderately, 4=Considerably, 5=Very much, 6=Extremely, -1=UNK

Item 5.A response: 0=Not at all, 1=Slightly, 2=A fair amount, 3=A moderate amount, 4=A considerable amount, 5=Very much, 6=An extreme amount, -1=UNK

Items 4.B2/5.B2/1.A3 response: 0=Not at all important, 1=Slightly important, 2=Reasonably important, 3=Moderately important, 4=Considerably important, 5=Very important, 6=Extremely important, -1=UNK

Items 4.B1/5.B1 response: 0= Exactly as I would like to be, 1=Good but not quite how I would like to be, 2=OK but not how I would like to be, 3=Neither good nor bad, 4=Quite bad but I could be much worse, 5=Bad but I could be worse, 6=The worst I could possibly be, -1=UNK

Item 1.A response: 0=No, 1=Yes, -1=UNK

Items 1.A1/1.A2 response: 0=None at all, 1=Some, 2=A fair amount, 3=A moderate amount, 4=A considerable amount, 5=Very many, 6=An extreme amount, -1=UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

Listing 16.2.7.4.5. INQOL: Items from Section 3-Question 1.B1 to Section 3-Question 1.B3 and Comments

			Items section 3						
Site	Patient	Visit	1.B1	1.B1 specify 1	1.B1 specify 2	1.B2	1.B2 specify	1.B3	Comments
xxx	xxx	Screening	xx	xxxx	xxxx	xx	xxxx	xx	xxxxxxxxxxxxx
		Month 3	xx	xxxx	xxxx	xx	xxxx	xx	xxxxxxxxxxxxx
		Month 6	xx	xxxx	xxxx	xx	xxxx	xx	xxxxxxxxxxxxx
		Month 9	xx	xxxx	xxxx	xx	xxxx	xx	xxxxxxxxxxxxx
		…	xx	xxxx	xxxx	xx	xxxx	xx	xxxxxxxxxxxxx
		EOS	xx	xxxx	xxxx	xx	xxxx	xx	xxxxxxxxxxxxx

Statements are given after the next listing.

Items 1.B1/1.B2 response: 0=Not at all, 1=Some, 2=A fair amount, 3=A moderate amount, 4=A considerable amount, 5=Very many, 6=An extreme amount, -1=UNK

Items 1.B3 response: 0=Not at all important, 1=Slightly important, 2=Reasonably important, 3=Moderately important, 4=Considerably important, 5=Very important, 6=Extremely important, -1=UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

Listing 16.2.7.4.6. INQOL: Questionnaire Scores

			Score section 1							Score section 2						Score section 3	
Site	Patient	Visit	1.1	1.2	1.3	1.4	1.5	1.6	1.7	2.1	2.2	2.3	2.4	2.5	2.6	3.1	3.2
xxx	xxx	Screening	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 3	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 6	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		Month 9	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		…	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx
		EOS	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx	xx

Scores follow the listing.
Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit.

INQOL Items

	Section 1
1	Do you have any muscle weakness due to your muscle condition?
1a	How much weakness would you say you have in the muscles affected by your condition?
1b	Does your muscle weakness cause difficulties in your life at the moment?
1c	How important to you are any difficulties caused by your muscle weakness?
2	Do you have any pain as a result of your muscle condition?
2a	How much pain would you say you have at the moment?
2b	Does your pain cause difficulties in your life at the moment?
2c	How important to you are any difficulties caused by your pain?
3	Do you feel tired/fatigued as a result of your muscle condition?
3a	How much tiredness/fatigue would you say you have at the moment?
3b	Does your tiredness/fatigue cause difficulties in your life at the moment?
3c	How important to you are any difficulties caused by your tiredness/fatigue?
4	Do you have any "locking" (seizing up) of your muscles as a result of your muscle condition? By locking we mean a specific muscle symptom of myotonia which refers to difficulty relaxing the muscles after voluntary muscle contraction. Your particular muscle disease may not be associated with myotonia in which case tick NO and go on the next question.
4a	How much muscle "locking" would you say you have at the moment?
4b	Does the "locking" of your muscles cause difficulties in your life at the moment?
4c	How important to you are any difficulties caused by the "locking" of your muscles?
5	Do you have any drooping of the eyelids as a result of your muscle condition?
5a	How much drooping of the eyelids cause difficulties in your life at the moment?
5b	Does the drooping of the eyelids cause difficulties in your life at the moment?
5c	How important to you are any difficulties caused by the drooping of the eyelids?
6	Do you have any double vision as a result of your muscle condition?
6a	How much double vision would you say you have at the moment?
6b	Does the double vision cause difficulties in your life at the moment?
6c	How important to you are any difficulties caused by the double vision?
7	Do you have any swallowing difficulty as a result of your muscle condition?
7a	How much swallowing difficulty would you say you have at the moment?
7b	Does the swallowing difficulty cause difficulties in your life at the moment?
7c	How important to you are any difficulties caused by the swallowing difficulty?
	Section 2
1.A I	Daily activities
1.A II	Leisure activities
1.A III	Work activities
1.A III specify 1	If you have **no paid employment** (ex. unemployed or retired or do house-work) please tick here
1.A III specify 2	If you are **not working due to your condition,** please tick here
1.B I	In the face of my condition, my ability to do all the things I want to do is:

1.B II	How important to you is the effect of your muscle condition on your ability to do all the things you want to do?
1.B II specify	OR, If your ability is **"exactly as you would like"**, please tick here
2.A	At the moment, how much help do you need from other people in carrying out your activities?
2.B I	In the face of my condition, my level of independence is:
2.B II	How important to you is the effect of your muscle condition on your level of independence?
2.B II specify	OR, if your independence is **"exactly as you would like"** please tick here
3.A I	Partner / Spouse
3.A I specify	If you are **not married or in a relationship** at the moment **OR** if you are **widowed** please tick here
3.A II	Other family members
3.A III	Friends
3.A IV	Other people
3.B I	In the face of my condition, my close family relationships are:
3.B II	How important to you is the effect of your muscle condition on your close family relationships?
3.B II specify	OR, if your close family relationships are **"exactly as you would like"**, please tick here
3.B III	In the face of my condition, my close friendships are:
3.B IV	How important to you is the effect of your muscle condition on your close friendships?
3.B IV specify	OR, If your close friendships are **"exactly as you would like"** please tick here
3.B V	In the face of my condition, my relationships with other people (acquaintances, strangers, and colleagues) are:
3.B VI	How important to you is the effect of your muscle condition on your relationships with these other people?
3.B VI specify	OR, If your relationships with others are **"exactly as you would like"** please tick here
4.A I	Anxious / Worried
4.A II	Depressed
4.A III	Frustrated
4.A IV	Low in confidence / self-esteem
4.B I	In the face of my condition, the way I feel emotionally is:
4.B II	How important to you is the effect of your muscle condition upon the way you feel emotionally?
4.B II specify	OR, If the way you feel emotionally is **"exactly as you would like"** please tick here
5.A	At the moment, does your muscle condition affect the way you look?
5.B I	In the face of my condition, the way I look is:
5.B II	How important to you is the effect of your muscle condition upon the way you look?
5.B II specify	OR, If the way you look is **"exactly as you would like"**, please tick here
	Section 3
A	Do you receive, or are you about to start receiving treatment for your muscle condition? (Fr example surgery, tablets, injections or physiotherapy)
1.A I	Do you feel the treatment you receive for your muscle condition has had beneficial effects?
1.A I specify 1	If you are **not receiving treatment**, please tick here
1.A I specify 2	If you are **unsure**, please tick here
1.A II	Do you feel the treatment you receive for your muscle condition will have beneficial effects in the future?
1.A II specify	If you are **unsure**, or if you have **not thought about** it please tick here

1.A III	How important to you are the beneficial effects of treatment?
1.B I	Do you feel the treatment you receive for your muscle condition has had harmful side effects?
1.B I specify 1	If you are **not yet receiving treatment**, please tick here
1.B I specify 2	If you are **unsure**, please tick here
1.B II	Do you think the treatment you receive for your muscle condition will have side effects in the future?
1.B II specify	If you are **unsure**, or if you have **not thought about** it please tick here
1.B III	How impo**rtant** to you are the side effects of the treatment?
Comments	If you have any comments you would like to make about your condition and the way it affects you, please use the apace below

	Scores
1.1	Weakness
1.2	Pain
1.3	Fatigue
1.4	Locking
1.5	Eyelids
1.6	Double vision
1.7	Swallowing
2.1	Activities
2.2	Independence
2.3	Social relationship
2.4	Emotions
2.5	Body imagine
2.6	QoL
3.1	Perceived treatment
3.2	Expected treatment

Listing 16.2.5.1. Treatment Emergent Adverse Events

Site	Patient	System Organ Class	Preferred Term	Verbatim Term	SAE (1)	Was AE started in SMA-001 study?	Start date	End date	Ongoing (2)	Duration (3)	Severity (4)	Relationship (5)	Action taken (6)	Outcome (7)	Date of death
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	No	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	
		xxxxxx	xxxxxx	xxxxxx	xxx	No	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	Death	DDMMMYYYY
		xxxxxx	xxxxxx	xxxxxx	xxx	Yes	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	No	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	
		xxxxxx	xxxxxx	xxxxxx	xxx	No	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	

(1) SAE: No, Yes, UNK
(2) Ongoing: No, Yes, UNK
(3) Duration= End Date – Start Date + 1; Duration =Date of Last Dose – Start Date + 1 if AE is ongoing
(4) Severity: 1= Mild, 2= Moderate, 3= Severe, 4= Life-threatening, 5= Death, UNK
(5) Relationship: Not related, Possibly, Probably, UNK
(6) Action Taken: None, Treatment required, Study drug dose reduction, Study drug dose discontinued & restarted, Study drug permanently discontinued, UNK
(7) Outcome: Resolved, Resolved w/sequelae, Not resolved, Death, UNK
(8) Seriousness: Fatal, Life threatening, Hospitalization, Persistent disability, Congenital anomaly, Important medical Event, UNK
Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.5.2. Treatment Emergent Serious Adverse Events

Site	Patient	SOC	PT	Verbatim Term	SAE (1)	Start date	End date	Ongoing (2)	Duration (3)	Severity (4)	Relationship (5)	Action taken (6)	Outcome (7)	Date of death	Seriousness (8)
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	DDMMMYYYY	xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx

(1) SAE: No, Yes, UNK
(2) Ongoing: No, Yes, UNK
(3) Duration= End Date – Start Date + 1; Duration =Date of Last Dose – Start Date + 1 if AE is ongoing
(4) Severity: 1= Mild, 2= Moderate, 3= Severe, 4= Life-threatening, 5= Death, UNK
(5) Relationship: Not related, Possibly, Probably, UNK
(6) Action Taken: None, Treatment required, Study drug dose reduction, Study drug dose discontinued & restarted, Study drug permanently discontinued, UNK
(7) Outcome: Resolved, Resolved w/sequelae, Not resolved, Death, UNK
(8) Seriousness: Fatal, Life threatening, Hospitalization, Persistent disability, Congenital anomaly, Important medical Event, UNK
Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.5.3. IMP Related Treatment Emergent Adverse Events

Site	Patient	SOC	PT	Verbatim Term	SAE (1)	Start date	End date	Ongoing (2)	Duration (3)	Severity (4)	Relationship (5)	Action taken (6)	Outcome (7)	Date of death	Seriousness (8)
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	DDMMMYYYY	xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx

(1) SAE: No, Yes, UNK
(2) Ongoing: No, Yes, UNK
(3) Duration= End Date – Start Date + 1; Duration =Date of Last Dose – Start Date + 1 if AE is ongoing
(4) Severity: 1= Mild, 2= Moderate, 3= Severe, 4= Life-threatening, 5= Death, UNK
(5) Relationship: Not related, Possibly, Probably, UNK
(6) Action Taken: None, Treatment required, Study drug dose reduction, Study drug dose discontinued & restarted, Study drug permanently discontinued, UNK
(7) Outcome: Resolved, Resolved w/sequelae, Not resolved, Death, UNK
(8) Seriousness: Fatal, Life threatening, Hospitalization, Persistent disability, Congenital anomaly, Important medical Event, UNK
Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.5.4. IMP Related Treatment Emergent Serious Adverse Events

Site	Patient	SOC	PT	Verbatim Term	SAE (1)	Start date	End date	Ongoing (2)	Duration (3)	Severity (4)	Relationship (5)	Action taken (6)	Outcome (7)	Date of death	Seriousness (8)
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	DDMMMYYYY	xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx

(1) SAE: No, Yes, UNK
(2) Ongoing: No, Yes, UNK
(3) Duration= End Date – Start Date + 1; Duration =Date of Last Dose – Start Date + 1 if AE is ongoing
(4) Severity: 1= Mild, 2= Moderate, 3= Severe, 4= Life-threatening, 5= Death, UNK
(5) Relationship: Not related, Possibly, Probably, UNK
(6) Action Taken: None, Treatment required, Study drug dose reduction, Study drug dose discontinued & restarted, Study drug permanently discontinued, UNK
(7) Outcome: Resolved, Resolved w/sequelae, Not resolved, Death, UNK
(8) Seriousness: Fatal, Life threatening, Hospitalization, Persistent disability, Congenital anomaly, Important medical Event, UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.5.5. Treatment Emergent Adverse Events Leading to Hospitalization

Site	Patient	SOC	PT	Verbatim Term	SAE (1)	Start date	End date	Ongoing (2)	Duration (3)	Severity (4)	Relationship (5)	Action taken (6)	Outcome (7)	Date of death	Seriousness (8)
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	DDMMMYYYY	xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx

(1) SAE: No, Yes, UNK
(2) Ongoing: No, Yes, UNK
(3) Duration= End Date – Start Date + 1; Duration =Date of Last Dose – Start Date + 1 if AE is ongoing
(4) Severity: 1= Mild, 2= Moderate, 3= Severe, 4= Life-threatening, 5= Death, UNK
(5) Relationship: Not related, Possibly, Probably, UNK
(6) Action Taken: None, Treatment required, Study drug dose reduction, Study drug dose discontinued & restarted, Study drug permanently discontinued, UNK
(7) Outcome: Resolved, Resolved w/sequelae, Not resolved, Death, UNK
(8) Seriousness: Fatal, Life threatening, Hospitalization, Persistent disability, Congenital anomaly, Important medical Event, UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.5.6. Life-Threatening Treatment Emergent Adverse Events

Site	Patient	SOC	PT	Verbatim Term	SAE (1)	Start date	End date	Ongoing (2)	Duration (3)	Severity (4)	Relationship (5)	Action taken (6)	Outcome (7)	Date of death	Seriousness (8)
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	DDMMMYYYY	xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx

(1) SAE: No, Yes, UNK
(2) Ongoing: No, Yes, UNK
(3) Duration= End Date – Start Date + 1; Duration =Date of Last Dose – Start Date + 1 if AE is ongoing
(4) Severity: 1= Mild, 2= Moderate, 3= Severe, 4= Life-threatening, 5= Death, UNK
(5) Relationship: Not related, Possibly, Probably, UNK
(6) Action Taken: None, Treatment required, Study drug dose reduction, Study drug dose discontinued & restarted, Study drug permanently discontinued, UNK
(7) Outcome: Resolved, Resolved w/sequelae, Not resolved, Death, UNK
(8) Seriousness: Fatal, Life threatening, Hospitalization, Persistent disability, Congenital anomaly, Important medical Event, UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.5.7. Treatment Emergent Adverse Events Leading to Premature Discontinuation

Site	Patient	SOC	PT	Verbatim Term	SAE (1)	Start date	End date	Ongoing (2)	Duration (3)	Severity (4)	Relationship (5)	Action taken (6)	Outcome (7)	Date of death	Seriousness (8)
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	DDMMMYYYY	xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx

(1) SAE: No, Yes, UNK
(2) Ongoing: No, Yes, UNK
(3) Duration= End Date – Start Date + 1; Duration =Date of Last Dose – Start Date + 1 if AE is ongoing
(4) Severity: 1= Mild, 2= Moderate, 3= Severe, 4= Life-threatening, 5= Death, UNK
(5) Relationship: Not related, Possibly, Probably, UNK
(6) Action Taken: None, Treatment required, Study drug dose reduction, Study drug dose discontinued & restarted, Study drug permanently discontinued, UNK
(7) Outcome: Resolved, Resolved w/sequelae, Not resolved, Death, UNK
(8) Seriousness: Fatal, Life threatening, Hospitalization, Persistent disability, Congenital anomaly, Important medical Event, UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.5.8. Treatment Emergent Adverse Events Leading to Death

Site	Patient	SOC	PT	Verbatim Term	SAE (1)	Start date	End date	Ongoing (2)	Duration (3)	Severity (4)	Relationship (5)	Action taken (6)	Outcome (7)	Date of death	Seriousness (8)
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	DDMMMYYYY	xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx

(1) SAE: No, Yes, UNK
(2) Ongoing: No, Yes, UNK
(3) Duration= End Date – Start Date + 1; Duration =Date of Last Dose – Start Date + 1 if AE is ongoing
(4) Severity: 1= Mild, 2= Moderate, 3= Severe, 4= Life-threatening, 5= Death, UNK
(5) Relationship: Not related, Possibly, Probably, UNK
(6) Action Taken: None, Treatment required, Study drug dose reduction, Study drug dose discontinued & restarted, Study drug permanently discontinued, UNK
(7) Outcome: Resolved, Resolved w/sequelae, Not resolved, Death, UNK
(8) Seriousness: Fatal, Life threatening, Hospitalization, Persistent disability, Congenital anomaly, Important medical Event, UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.5.9. Adverse Events Started Before the First Dose of Study Drug for the Current Long-Term Study

Site	Patient	SOC	PT	Verbatim Term	SAE (1)	Was AE started in SMA-001 study?	Start date	End date	Ongoing (2)	Duration (3)	Severity (4)	Relationship (5)	Action taken (6)	Outcome (7)	Date of death	Seriousness (8)
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	No	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	No	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	DDMMMYYYY	xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	Yes	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
xxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxx	No	DDMMMYYYY	DDMMMYYYY	No	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx
		xxxxxx	xxxxxx	xxxxxx	xxx	No	DDMMMYYYY		Yes	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx		xxxxx

(1) SAE: No, Yes, UNK
(2) Ongoing: No, Yes, UNK
(3) Duration= End Date – Start Date + 1; Duration =Date of Last Dose – Start Date + 1 if AE is ongoing
(4) Severity: 1= Mild, 2= Moderate, 3= Severe, 4= Life-threatening, 5= Death, UNK
(5) Relationship: Not related, Possibly, Probably, UNK
(6) Action Taken: None, Treatment required, Study drug dose reduction, Study drug dose discontinued & restarted, Study drug permanently discontinued, UNK
(7) Outcome: Resolved, Resolved w/sequelae, Not resolved, Death, UNK
(8) Seriousness: Fatal, Life threatening, Hospitalization, Persistent disability, Congenital anomaly, Important medical Event, UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.6.1. Clinical Laboratory Evaluations: Blood Chemistry

						Original Unit						Standard Unit (SI)		
Site	Patient	Visit	Blood sample taken?	Sample date	Laboratory parameter	Values	Unit (1)	NRLL	NRUL	Clinical assessment (2)	Assessment respect to Normal Ranges (3)	Value	Unit	CFB
xxx	xxx	Screening	xxx	DDMMMYYYY	Albumin	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					Alkaline Phosphatase	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					ALT	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					AST	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					…									
		Month 3	No		Albumin									
					Alkaline Phosphatase									
					ALT									
					AST									
					…									
		…	xxx	DDMMMYYYY	Albumin	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					Alkaline Phosphatase	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					ALT	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					AST	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					…									
		EOS	xxx	DDMMMYYYY	Albumin	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					Alkaline Phosphatase	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					ALT	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					AST	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx

CFB=Change from Baseline.
NRLL: Normal Range Lower Limit; NRUL: Normal Range Upper Limit.
(1) Unit: mg/dL, µmol/L, g/dL, g/L, mmol/L, IU/L, meq/L, sec, 10^3/µL, 10^6/µL, 10^9/L, 10^12/L, 10^3/mm^3, 10^6/mm^3, %, Other, UNK, NA
(2) Clinical Assessment: Within normal range, Out of range (not clinically significant), Out of range (clinically significant), UNK
(3) Assessment respect to normal ranges: Below normal range, Within normal range, Above normal range, UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Continue for other blood chemistry parameters: direct bilirubin, total bilirubin, BUN, calcium, chloride, total cholesterol, bicarbonate, creatine phosphokinase, creatinine, glucose, GGT, LDH, phosphorus, potassium, total protein, sodium and uric acid.

Sort by site, patient number and visit.

Listing 16.2.6.2. Clinical Laboratory Evaluations: Hematology

						Original Unit						Standard Unit (SI)		
Site	Patient	Visit	Blood sample taken?	Sample date	Laboratory parameter	Values	Unit (1)	NRLL	NRUL	Clinical assessment (2)	Assessment respect to Normal Ranges (3)	Value	Unit	CFB
xxx	xxx	Screening	xxx	DDMMMYYYY	Hemoglobin	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					Hematocrit	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					WBC count	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					RBC count	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					…									
		Month 3	No		Hemoglobin									
					Hematocrit									
					WBC count									
					RBC count									
					…									
		…	xxx	DDMMMYYYY	Hemoglobin	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					Hematocrit	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					WBC count	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					RBC count	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					…									
		EOS	xxx	DDMMMYYYY	Hemoglobin	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					Hematocrit	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					WBC count	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
					RBC count	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx

CFB=Change from Baseline.
NRLL: Normal Range Lower Limit; NRUL: Normal Range Upper Limit.
(1) Unit: mg/dL, µmol/L, g/dL, g/L, mmol/L, IU/L, meq/L, sec, 10^3/µL, 10^6/µL, 10^9/L, 10^12/L, 10^3/mm^3, 10^6/mm^3, %, Other, UNK, NA
(2) Clinical Assessment: Within normal range, Out of range (not clinically significant), Out of range (clinically significant), UNK
(3) Assessment respect to normal ranges: Below normal range, Within normal range, Above normal range, UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Continue for other hematology parameters: platelets count, neutrophils, lymphocytes, monocytes, eosinophils and basophils.

Sort by site, patient number and visit.

Listing 16.2.6.3. Clinical Laboratory Evaluations: Urinalysis

Site	Patient	Visit	Urine sample taken?	Appearance	Color	Sample date	Laboratory parameter	Values	Unit (1)	Other unit	Clinical assessment (2)	CFB
xxx	xxx	Screening	xxx	xxxxx	xxxxx	DDMMMYYYY	pH	xxx			xxx	xxx
							Specific gravity	xxx	xxx	xxx	xxx	xxx
							Ketones	xxx	xxx	xxx	xxx	xxx
							Protein	xxx	xxx	xxx	xxx	xxx
							…					
		Month 3	No				pH					xxx
							Specific gravity					xxx
							Ketones					xxx
							Protein					xxx
							…					
		…	xxx	xxxxx	xxxxx	DDMMMYYYY	pH	xxx			xxx	xxx
							Specific gravity	xxx	xxx	xxx	xxx	xxx
							Ketones	xxx	xxx	xxx	xxx	xxx
							Protein	xxx	xxx	xxx	xxx	xxx
							…					
		EOS	xxx	xxxxx	xxxxx	DDMMMYYYY	pH	xxx			xxx	xxx
							Specific gravity	xxx	xxx	xxx	xxx	xxx
							Ketones	xxx	xxx	xxx	xxx	xxx
							Protein	xxx	xxx	xxx	xxx	xxx
							…					

CFB=Change from Baseline.

(1) Unit: mg/dL, µmol/L, g/dL, g/L, mmol/L, IU/L, meq/L, sec, 10^3/µL, 10^6/µL, 10^9/L, 10^12/L, 10^3/mm^3, 10^6/mm^3, %, Other, UNK, NA
Specific gravity Unit: g/L, Kg/L, Other, UNK, NA

(2) Clinical Assessment: Within normal range, Out of range (not clinically significant), Out of range (clinically significant), UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Continue for other urine parameters: glucose, bilirubin, nitrite, urobilinogen and hemoglobin.

Sort by site, patient number and visit.

Listing 16.2.7.1. Pregnancy Test

Site	Patient	Visit	Childbearing potential	Test performed?	Type of pregnancy test	Date of test	Result
xxx	xxx	Screening	xxxxxxxx	xxx	Urine	DDMMMYYYY	xxxxxxxx
		Month 3	xxxxxxxx	No			
		Month 6	xxxxxxxx	xxx	Urine	DDMMMYYYY	xxxxxxxx
		…	xxxxxxxx	xxx	Serum	DDMMMYYYY	xxxxxxxx
		EOS	xxxxxxxx	xxx	xxx	DDMMMYYYY	xxxxxxxx
xxx	xxx	Screening	xxxxxxxx	xxx	xxx	DDMMMYYYY	xxxxxxxx
		Month 3	xxxxxxxx	xxx	xxx	DDMMMYYYY	xxxxxxxx
		Month 6	xxxxxxxx	xxx	xxx	DDMMMYYYY	xxxxxxxx
		…	xxxxxxxx	xxx	xxx	DDMMMYYYY	xxxxxxxx
		EOS	xxxxxxxx	xxx	xxx	DDMMMYYYY	xxxxxxxx

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site, patient number and visit.

Listing 16.2.7.2. Vital Signs

Site	Patient	Visit	Were vital signs collected?	Date of examination	Sitting SBP (mmHg)		Sitting DBP (mmHg)		Sitting heart rate (bpm)		Respiration rate (breaths/min)		Body temperature (°C)		Body weight (kg)	
					Value	CFB	Value	CFB	Value	CFB	Value	CFB	Value	CFB	Value	CFB
xxx	xxx	Screening	xxx	DDMMMYYYY	xxx		xxx		xxx		xxx		xxx		xxx	
		Month 3	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Month 6	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Month 9	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Month 12	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		…	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		EOS	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
xxx	xxx	Screening	xxx	DDMMMYYYY	xxx		xxx		xxx		xxx		xxx		xxx	
		Month 3	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Month 6	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Month 9	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Month 12	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		…	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		EOS	xxx	DDMMMYYYY	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx	xxx

CFB=Change from Baseline.
Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and visit. Height is recorded with Demography at Screening only.

Listing 16.2.7.3. ECG

Site	Patient	Visit	Was an ECG performed?	Date performed	Tracing (1)	If Abnormal, please specify	System Organ Class	Preferred Term
xxx	xxx	Screening	xxx	DDMMMYYYY	xxxxxxxxxxx			
		Month 3	xxx	DDMMMYYYY	Abnormal	Xxxxxxxxx	xxxxxxxxxxx	xxxxxxxxxxx
		…	No					
		EOS	xxx	DDMMMYYYY	xxxxxxxxxxx			
xxx	xxx	Screening	xxx	DDMMMYYYY	xxxxxxxxxxx			
		Month 3	xxx	DDMMMYYYY	Abnormal	Xxxxxxxxx	xxxxxxxxxxx	xxxxxxxxxxx
		…	xxx	DDMMMYYYY	xxxxxxxxxxx			
		EOS	xxx	DDMMMYYYY	xxxxxxxxxxx			

(1) Tracing Assessment: Normal tracing, Abnormal tracing (not clinically significant), Abnormal tracing (clinically significant), UNK.

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site, patient number and visit.

Listing 16.2.7.4. Physical Examination

Site	Patient	Visit	Physical examination performed? (1)	If not performed, reason	Body system	Result (2)	If Abnormal findings, specify	System Organ Class	Preferred Term
xxx	xxx	Screening	xxx		Head/Neck	xxxxx			
					Eyes	Abnormal	Xxxxxxxxxxxxxx	xxxxxxxxxx	xxxxxxxxxx
					Ears	Abnormal	Xxxxxxxxxxxxxx	xxxxxxxxxx	xxxxxxxxxx
					Nose	xxxxx			
					.				
		Month 3	No	Xxxxxxxx	Head/Neck				
					Eyes				
					Ears				
					Nose				
					.				
		…	xxx		Head/Neck	xxxxx			
					Eyes	xxxxx			
					Ears	Abnormal	Xxxxxxxxxxxxxx	xxxxxxxxxx	xxxxxxxxxx
					Nose	xxxxx			
					.				
		EOS	xxx		Head/Neck	Abnormal	Xxxxxxxxxxxxxx	xxxxxxxxxx	xxxxxxxxxx
					Eyes	xxxxx			
					Ears	xxxxx			
					Nose	xxxxx			
					.				

(1) Was a complete physical examination performed?
(2) Result: Normal, Abnormal, Not examined

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Continue for other body system: throat, cardiovascular, dermatologic, lymphatic, respiratory, gastrointestinal, musculoskeletal and other. Sort by site, patient number and visit.

Listing 16.2.8. Concomitant Medications

Site	Patient	Patient receive medication? (1)	#	Generic name	Total daily dose	Units	Route (2)	If Other Route, specify	Start date	End date	Ongoing at end of study?	Reason for administration (3)	Indication for use
xxx	xxx	xxx	x	xxxxxxxxx	xxxx	xxxxx	xxxx	xxxxxx	DDMMMYYYY	DDMMMYYYY	No	xxxxxxxxx	xxxxxxxxx
			x	xxxxxxxxx	xxxx	xxxxx	xxxx	xxxxxx	DDMMMYYYY		Yes	xxxxxxxxx	xxxxxxxxx
xxx	xxx	xxx	x	xxxxxxxxx	xxxx	xxxxx	xxxx	xxxxxx	DDMMMYYYY	DDMMMYYYY	No	xxxxxxxxx	xxxxxxxxx
			x	xxxxxxxxx	xxxx	xxxxx	xxxx	xxxxxx	DDMMMYYYY	DDMMMYYYY	No	xxxxxxxxx	xxxxxxxxx
			x	xxxxxxxxx	xxxx	xxxxx	xxxx	xxxxxx	DDMMMYYYY	DDMMMYYYY	No	xxxxxxxxx	xxxxxxxxx
xxx	xxx	No											

(1) Did the patient receive Previous and/or Concomitant medications?
(2) Route: PO, IM, IV, SC, Rectal, Topical, Nasal, Inhaled, Other, UNK
(3) Reason for administration?: Therapeutic, Prophylaxis, Other, AE treatment, UNK

Source: [program name].sas, Run on ddmmmyyyy.

Programming Notes: Sort by site, patient number and start date.

Listing 16.2.9. End of Study

Site	Patient	End of study assessment date	Did the patient take at least one dose of study medication?	Date of last study drug dose intake	Complete the treatment? (1)	If No, report the primary reason for discontinuation	If reason is AE, Major Protocol Deviation, or Other Reason, specify	If reason is Consent Withdrawal, specify the date
xxx	xxx	DDMMMYYYY	xxx	DDMMMYYYY	Yes			
xxx	xxx	DDMMMYYYY	xxx	DDMMMYYYY	Yes			
xxx	xxx	DDMMMYYYY	No		No	Consent Withdrawal		DDMMMYYYY
xxx	xxx	DDMMMYYYY	xxx	DDMMMYYYY	No	Adverse event	Xxxxxxxxxx	
xxx	xxx	DDMMMYYYY	xxx	DDMMMYYYY	No	xxxxxxxxxx		

(1) Did the patient complete the full course of treatment?

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number.

Listing 16.2.10. Comments

Site	Patient	Presence of comments?	Visit (1)	Form (2)	Comment
xxx	xxx	xxx	xxx	xxx	Xxxxxxxxxxx
xxx	xxx	xxx	xxx	xxx	Xxxxxxxxxxx
xxx	xxx	xxx	xxx	xxx	Xxxxxxxxxxx
xxx	xxx	xxx	xxx	xxx	Xxxxxxxxxxx
xxx	xxx	xxx	xxx	xxx	Xxxxxxxxxxx

(1) Visit: Screening, End of study month 3, End of study month 6, End of study month 9, End of study month 12, End of study month 15, End of study month 21, End of study month 27, EoS, Extra visit

(2) Form: Inclusion Criteria, Exclusion Criteria, Demographics characteristics, Medical History, Physical Examination, Vital signs, ECG, Pregnancy Test, Blood Chemistry, Hematology, Urinalysis, Amifampridine Treatment, QoL, IP Treatment Dispensing & Accountability, End of Study, Adverse Events, Previous and Concomitant Medications, Investigator's Declaration, Other

Source: [program name].sas, Run on ddmmmyyyy.

Programming Note: Sort by site and patient number.