Statistical Analysis Plan

A Phase 3, Multicenter, Double-Blind, Placebo-Controlled, Randomized, Outpatient, Two-period Two-treatment Crossover Study to Evaluate the Efficacy and Safety of Amifampridine Phosphate (3,4 Diaminopyridine Phosphate) in Patients with Congenital Myasthenic Syndromes (CMS)

Study No: CMS-001

Version 1.0

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Approval Page

By entering into this Statistical Analysis Plan (SAP), the parties acknowledge and agree that this SAP shall be incorporated into and subject to the terms of the Master Services Agreement (MSA). Any changes requested by Client to this SAP shall be subject to Section I.C of the MSA requiring a mutually agreed upon "Change Order" prior to any modification of the procedures set forth herein.

I agree to the format and content of this document.

Approved by:

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Revision History

N/A

Table of Contents

1.0 Synopsis of Study Design Procedure

This statistical analysis is based on and consistent with the CMS-001 Protocol Amendment 5, dated June 26, 2017.

1.1 Design and Treatment

This randomized (1:1), double-blind, placebo-controlled, outpatient two-period, two-treatment crossover study is designed to evaluate the efficacy and safety of amifampridine phosphate in patients diagnosed with certain genetic subtypes of CMS and demonstrated open label (amifampridine phosphate) or history of sustained amifampridine benefit from treatment. The primary objectives of this study are to:

- Characterize the overall safety and tolerability of amifampridine phosphate compared with placebo in individuals with CMS; and,
- Assess the clinical efficacy of amifampridine phosphate compared with placebo in individuals with CMS, based on change in Subject Global Impression (SGI) and Motor Function Measure (MFM 20 or 32) scores.

CMS-001 is a randomized double-blind, placebo controlled, two-period two- treatment crossover study designed to evaluate the efficacy and safety of amifampridine phosphate in children (2-16 years of age) and adults (17 years of age or more) diagnosed with CMS. Patients were randomized (1:1) to one of two sequences:

PA: Placebo – Amifampridine, or
AP: Amifampridine – Placebo.

The study was planned to include up to 23 male and female subjects. The planned duration for each patient is approximately 56 days, excluding the screening period. The screening period may last up to 14 days.

1.2 Study Procedures

Amifampridine phosphate oral dosing using 10 mg tablets starts on Day 1 of the open-label run-in and patients were titrated upward every 3 to 4 days, per guidelines in the protocol and at the discretion of the investigator, for up to 4 weeks, with 1 week of stable dose and frequency at the end of the run-in period before being randomized. Dosing and/or dosing frequency was increased or decreased by the investigator during the run-in phase prior to the last week of stable dosing. Per the protocol, randomization could only occur after the patient has been on a stable dose and dosing frequency for at least 7 days.

At the end of their Period 1 randomization treatment, patients will return to open-label amifampridine phosphate for 14 days between Periods 1 and 2 at the same dose and

dosing frequency established in the run-in phase. No dose adjustments were allowed during this re-stabilization period.

After completing Period 2, (the completion point for this trial) patients were eligible for an open-label amifampridine phosphate treatment protocol (CMS-002).

1.3 Sample Size

The primary analysis variable is the SGI Change from Baseline (CFB). It will be analyzed using the standard analysis for a two-by-two crossover design. The sample size required to control the power depends on the ratio of the between-treatment difference and the standard deviation of the difference (that is, the effect size). Assuming an effect size of 0.5, 23 patients per sequence will ensure power of 90% for the 2-sided test at the 0.05 level.

2.0 Data Analysis Considerations

2.1 Types of Analyses

The term summary statistics for quantitative data refers to the following list of statistics unless otherwise specified: n (number of non-missing observations), mean, standard deviation, minimum, median, and maximum. Categorical variables will be summarized with frequency counts and percentage of the relevant group. Summary statistics will be summarized by treatment sequence and study period. Unless otherwise stated, all hypotheses will be tested at a two-sided α = 0.05 significance level

2.2 Analysis Populations

The following analysis populations will be defined for the analysis:

- **Safety Population**: All randomized patients who have received at least one dose of study medication.
- **Full Analysis Set (FAS)**: The FAS consists of all randomized patients who receive at least one dose of study medication and have at least one post-treatment efficacy assessment.
- **Per Protocol (PP)**: The PP population will include all patients in the FAS who have no major protocol deviations.

Exclusion from the FAS and PP populations will be finalized prior to database lock and subsequent unblinding. Data listings will be created to list the subjects excluded from each population, along with the reasons for exclusion.

2.2.1 Subgroup Definitions

Patients will be categorized and grouped for analysis by:

- Genetic deficiencies and mechanistic localization (See Table 2.2-1 below);
- Whether their mutation is pre- or post- synaptic (See Table 2.2-1 below);
- Whether they are an adult (age ≥ 17 years) or pediatric (age < 17 years);
- Pediatric subject ages will be further broken down into categories (2 to less than 6, 7 to less than 12, and 12 to less than 17 years of age).

Table 2.2-1 Genetic Mutation Type and Neuromuscular Junction Localization Type

Mutation	Description	Localization
CHRNE	Codes for the epsilon subunit of the acetylcholine receptor.	Post-synaptic
SNAP 25B	One of the proteins in the SNARE complex responsible for acetylcholine vesicle fusion in presynaptic neurons.	Pre-synaptic
RAPSYN	Anchors and clusters acetylcholine receptors.	Post-synaptic
DOK7	Docking Protein 7: Activates the MuSK protein and catalyzes the development of Neuromuscular Junctions (NMJs) on the muscle.	Post-synaptic
AGRN	Agrin protein secreted by pre-synaptic motor neurons to start and maintain the formation of a NMJ.	Pre-synaptic
CHAT	Codes for choline acetyltransferase that synthesizes acetylcholine in the presynaptic nerve terminal.	Pre-synaptic

2.3 Missing Data Conventions

All analyses will be based on the observed data without imputation, i.e., a complete case analysis.

2.4 Interim Analyses

No unblinded interim analyses of the data were conducted.

2.5 Study Center Considerations

Although CMS-001 is a multi-center study, data from all sites will be pooled for this analysis. No provision is made for selective pooling of study sites.

2.6 Documentation and Other Considerations

All analyses will be conducted using SAS® software Version 9.4 or later.

3.0 Analysis of Baseline Subject Characteristics

Baseline and demographic characteristics will be summarized by treatment and overall for all subjects in the safety population. Age and baseline weight will be summarized with summary statistics. Gender, ethnicity, will be summarized with counts and percentages.

A detailed patient profile listing will be provided as shown in Appendix B. A detailed listing of demographic data for each subject will be provided as shown in Appendix B.

4.0 Analysis of Efficacy

4.1 Description of Efficacy Variables

4.1.1 Description of Primary Efficacy Variables

The following variables will be used to conduct the primary analysis of efficacy:

- Subject Global Impression assessments at Days 0, 8, 21, and 29;

4.1.2 Description of Secondary Efficacy Variables

The following variables will be secondary measures of efficacy:

- Motor Function Measure 20 (MFM-20, for Pediatric Subjects under 7 years) or Motor Function Measure 32 (MFM-32, for all other subjects). MFM-20 or MFM-32 Scores, depending on age, will be calculated as Totals for all items. MFM-20 and MFM-32 dimension scores are totals for item group D1 (Standing and Transfer), item group D2 (Axial and Proximal Motor Function), and item group D3 (Distal Motor Function). Additionally, these Motor Function Measure outcomes are also expressed as a percentage of the respective maximum possible total for all items and the three dimension groups, for both the MFM 20 and MFM 32. The percentages "normalize" the response between MFM 20 and MFM 32 so that

outcomes from these two groups of test subjects can be pooled for analysis across age groups;
- Clinical Global Impression-Severity;
- Clinical Global Impression-Improvement;
- Stimulated Single Fiber Electromyelogram: mean jitter, median jitter, and percent block (this optional test is described in the protocol but not conducted at all trial sites);
- Slurp Test: Time (seconds) to make slurp sound (this optional test is described in the protocol but not conducted at all trial sites).

4.2 Analysis of Efficacy

4.2.1 Analysis of Primary Efficacy

Summary statistics for the SGI Day 0 assessment (baseline for period 1), SGI Day 8 assessment, and the corresponding change from baseline (CFB_1) will be presented by treatment and by treatment within mutation type (pre- vs. post-synaptic, see Table 2.2-1 above) as period 1. A Mann-Whitney-Wilcoxon test for equality of the CFB_1 in the two treatments in Period 1 will be presented. Summary statistics for the SGI Day 21 assessment (baseline for period 2), SGI Day 29 assessment, and the corresponding change from baseline (CFB_2) will also be presented by treatment and treatment within mutation type as period 2. Also, the change in CFB from Period 1 to Period 2 will be computed. This value estimates

$$\tau_1 - \tau_2$$

where τ_1 represents the treatment effect of the period 1 Investigational Product (IP) and τ_2 represents the treatment effect of period 2 IP. A Mann-Whitney-Wilcoxon test of the hypothesis that the above parameter is the same in both treatment sequences will be provided. Note that this is a nonparametric version of the treatment main effect in a linear model without carryover effects.

A mixed effects linear model will be fit to the data with SGI raw scores as the response. The model will include fixed effect terms for treatment, period, age group (2 to less than 6, 7 to less than 12, and 12 to less than 17 years of age, 17 years and over), mutation type and sequence*period, where sequence corresponds to the order in which the subject received treatments, and a random effect for subject. The LSMeans for the treatment groups will be calculated and provided, as will the estimated difference along with a 95% confidence interval for this difference and a formal hypothesis test that the difference in the means is 0.

Special care must be taken in crossover designs to ensure that there is not significant carryover effect; however, the pharmacological properties of amifampridine are such that

having a 12-day washout period should minimize any unequal carryover effects in the analysis.

4.2.2 Analysis of Secondary Efficacy

MFM (20 or 32)
The Motor function measure (MFM) consists of 32 "Items" (scored 0 to 3) for subjects aged 7 and older (the "MFM 32") or a subgroup of 20 items (scored 0 to 3) that are suitable for small children under the age of 7 (the "MFM 20"). Where summary statistics are required for groups of items, or all of them, the group or grand totals will be expressed as a percentage ("percent outcome") of the maximum possible total for the number of items being aggregated and analyzed. Where the individual items or groups of items are consistent across all subjects, raw item scores (or aggregate sums thereof) will be used, unless specified otherwise below in order to present data in a manner consistent with use of percent outcomes for ease of review.

The MFM consists of three motor function dimensions, Standing and Transfer (D1), Axial and Proximal Motor Function (D2), and Distal Motor Function (D3). A table of each of the 32 items, whether or not it is present in the MFM 20, and its dimension code is provided in Table 4.2-1.

Table 4.2-1. Item definitions for the subscales D1, D2, and D3 of the Motor Function Measure instruments

Item Number	MFM 32	MFM 20	Dimension
1	✓	✓	D2
2	✓		D2
3	✓	✓	D2
4	✓	✓	D3
5	✓	✓	D2
6	✓	✓	D1
7	✓	✓	D2
8	✓		D1
9	✓	✓	D2
10	✓	✓	D2
11	✓	✓	D1
12	✓	✓	D1
13	✓		D2
14	✓	✓	D2
15	✓		D2
16	✓		D2
17	✓		D3
18	✓	✓	D3
19	✓		D3
20	✓		D3
21	✓	✓	D3
22	✓	✓	D3
23	✓	✓	D2
24	✓	✓	D1
25	✓	✓	D1
26	✓		D1
27	✓	✓	D1
28	✓		D1
29	✓		D1
30	✓	✓	D1
31	✓		D1
32	✓	✓	D1

The maximum possible score for the entire group of items and for each of the three dimensions in the MFM 32 and MFM 20 are provided in Table 4.2-2.

Table 4.2-2. Motor Function Measure maximum scores overall and for the subscales

Grouping	MFM 32	MFM 20
Grand Total	96	60
Dimension 1 (D1)	39	24
Dimension 2 (D2)	36	24
Dimension 3 (D3)	21	12

Where summary statistics or hypothesis testing calls for the use of data that spans both MFM 32 and MFM 20 (items present in both MFM 32 and MFM 20 or the use of percent outcomes), all relevant subject data will be used. Where the required data is not part of the MFM 20, only relevant data from the subjects that were assessed with the MFM 32 will be used (see Table 2 and descriptions below).

Summary statistics for the Day 0 assessment (baseline for period 1), the Day 8 assessment, and the corresponding change from baseline (CFB_1) will be presented by treatment, presented by treatment and genetic mutation (data aggregation by both treatment and genetic mutations within each treatment), and separately by treatment and by Pre- and Post-synaptic Mechanism (see Table 2.2-1), for the following MFM Data.

- Total MFM percent outcome, all age groups
- MFM percent outcome for Dimension 1 (D1), all age groups
- MFM percent outcome for Dimension 2 (D1), all age groups
- MFM percent outcome for Dimension 3 (D3), all age groups
- Total MFM percent outcome, ages 2 to less than 17
- MFM percent outcome for Dimension 1 (D1), ages 2 to less than 17
- MFM percent outcome for Dimension 2 (D1), ages 2 to less than 17
- MFM percent outcome for Dimension 3 (D3), ages 2 to less than 17
- Total MFM percent outcome, ages 17 and above
- MFM percent outcome for Dimension 1 (D1), ages 17 and above
- MFM percent outcome for Dimension 2 (D1), ages 17 and above
- MFM percent outcome for Dimension 3 (D3), ages 17 and above
- Each individual item (a total of 32 domains, see Table 3). Where a subject was assessed with the MFM 20 and the individual item is not a component of the MFM 20, that subject will be excluded and any tables and listing of the data will have a foot note indicating that subjects assessed with the MFM 20 have been excluded from that specific table and/or listing. During meetings with the Agency, it was pointed out that MFM might not be completely appropriate for CMS, but was chosen as the only validated, objective assessment of motor function suitable both adult and pediatric subject populations. The analysis of these individual item datasets is intended to fully assess the suitability of the MFM and its component items.

A Mann-Whitney-Wilcoxon test for equality of the CFB for the two treatments in Period 1 for the data types shown above will be presented.

Summary statistics for the Day 21 assessment (baseline for period 2), the Day 29 assessment, and the corresponding change from baseline (CFB_2) for the same MFM datasets analyzed for Period 1 above will also be presented by treatment, presented by

treatment and genetic mutation, and separately by treatment and by Pre- and Post-synaptic Mechanism (see Table 2.2-1).

The change in CFB from Period 1 to Period 2 (the "Individual Treatment Effect") will be computed for each of the MFM datasets described above, with correction for the order of treatment (placebo-amifampridine versus amifampridine-placebo), as needed so that potential effect of the active treatment, using each subject as their own control, can be evaluated. This value estimates

$$\tau_1 - \tau_2$$

where τ_1 represents the treatment effect for each dataset of the Amifampridine Investigational Product Period and τ_2 represents the treatment effect for each dataset of the Placebo Investigational Product Period. A Mann-Whitney-Wilcoxon test of the hypothesis that the above parameter is the same in both treatment sequences will be provided for each dataset. (Note that this is a nonparametric version of the treatment main effect in a linear model without carryover effects or order of treatment effects).

Summary statistics will also be provided for the Individual Treatment Effect derived from the datasets above and will be presented for all relevant subjects by Genetic Defect and separately by Pre- and Post-synaptic Mechanism (see Table 2.2-1). For the MFM individual item analyses results will be sorted by mutation within mutation type and estimated effect size (defined as the estimated difference in the marginal treatment means divided by the standard error of this difference).

The analysis summaries for the above analyses will be sorted by effect size, defined as the T-score associated with the hypothesis test that the difference between treatment group LSMeans is 0.

A mixed effects linear model will be fit to each of the MFM datasets described above with percent outcome or individual item scores (as appropriate for each dataset) as the response variable. The model will include fixed effect terms for treatment, period, age group (2 to less than 6 years, 7 to less than 12 years, 12 to less than 17 years of age, and 17 years of age and above), genetic mutation type (see Table 2.2-1), and sequence*period, where sequence corresponds to the order in which the subject received treatments, and a random effect for subject. No carryover term will be fit. In the case of the MFM percent outcome datasets for the adult only patients, the age group fixed effect term will not be included in the model. The LSMeans for the treatment groups will be calculated and provided, as will the estimated difference along with a 95% confidence interval for this difference and a formal hypothesis test that difference in the means is 0. Note that it is highly likely that some specific mutations will have insufficient subjects to fit the specified model. In that case a notation will be made in the relevant table(s) that there are not sufficient observations to fit the model.

Special care must be taken in crossover designs to ensure that there is no significant carryover effect; however, the pharmacological properties of amifampridine (an elimination half-life of 1.8-2.5 hours) are such that having a 12-day restabilization period will minimize any unequal carryover effects in the analysis.

Clinical Global Impression
CGI-S and CGI-I will be summarized by frequency tables and percentages for each sequence group and period. The values will be analyzed with a Wilcoxon rank-sum test as outlined in Putt and Chinchilli (2004). The treatment effects will be estimated using the Hodges-Lehmann estimator and a 90% asymptotic confidence interval.

Single Fiber Electromyelogram
Summary statistics for mean jitter, median jitter, and percent block will be presented by treatment and visit. No formal inferential procedures will be performed for these data.

Slurp Test
Summary statistics for the time to slurp sound from the slurp test and its CFB (Day 0 for Period 1 and Day 21 for Period 2) will be presented. No formal inferential procedures will be performed for these data.

5.0 Analysis of Safety

The following describes the statistical analysis of safety data.

Adverse Events
Prior to analysis, all AEs will be coded using the Medical Dictionary for Regulatory Activities (MedDRA). A treatment-emergent adverse event (TEAE) is any AE that occurs after the start of treatment. All TEAEs will be summarized by the treatment at the time of onset. Counts and percentages will be presented by treatment for each observed system organ class (SOC) and preferred term (PT) as defined in MedDRA. Summaries by level of severity and relationship to treatment will be presented by treatment at the time of occurrence. Listings of all deaths, serious AEs (SAEs), and discontinuations due to AE will be provided.

An overall summary table will provide the highest relationship and maximum severity observed per subject, as well as the counts of patients who: discontinued early due to an AE, experienced death, or had at least one SAE.

Laboratory Tests
For each laboratory parameter, descriptive statistics will be presented for the observed value and change from baseline (CFB) by treatment, where CFB is the change from the previous assessment. The frequency and percentage of patients who experience

abnormal (i.e. outside of reference ranges) laboratory values will be presented by treatment and time point.

Concomitant Medications
A table of the WHO-coded medications will be constructed by treatment given in the study period (amifampridine or placebo) and overall with medications summarized by level 4 Anatomical Therapeutic Chemical (ATC) term and Preferred Term. The number and percent of patients on each drug will be summarized. A data listing for all concomitant medications will be provided.

Vital Signs
Summary statistics (mean, median, sample size, standard deviation, minimum, and maximum) will be computed on the raw and change from baseline values for each vital sign parameter by time point, for each treatment. The screening time point will serve as baseline. If there are multiple vital signs taken at any time point, then the latest set of vital signs will be used for the analysis. All vital sign data will be listed.

ECG Results
ECG parameters will be summarized with descriptive statistics by time point and treatment sequence. A shift table of investigator classification of the ECG will be created showing Normal/Normal, Normal/Abnormal, Abnormal/Normal, and Abnormal/Abnormal frequencies by time point using the screening value as the baseline. All ECG data will be listed.

Physical Examination Findings
All Physical Examination data will be listed as shown in Appendix B.

6.0 Other Relevant Data Analyses/Summaries

6.1 Subject Disposition

A table will be constructed to show the counts and percentages of the following disposition categories:

- Screen failures
- Enrolled
- Completed
- Withdrawn

For those subjects who withdrew early, counts and percentages will be provided for each early withdrawal reason.

6.2 Subject Profiles

Two subject profiles combining all the available data for each subject will be provided as shown in Appendix B. The first will combine all available data and display the MFM results in item order (i.e., 1, 2, 3, etc.). The second will include only the MFM results displaying them sorted by decreasing Treatment main effect difference.

7.0 List of Analysis Tables, Figures and Listings

Table No.	Table Title	Included in Tables	Shown in Appendix B
1	Subject Disposition	X	X
2	Baseline Disease Characteristics by Age Group (Safety Population)	X	X
3	Demographics and Baseline Data Summary Statistics – Continuous Variables (Safety Population)	X	X
4	Demographics and Baseline Data Summary Statistics – Categorical Variables (Safety Population)	X	X
5	SGI Score Summary Statistics by Treatment, Period and Time Point (FAS Population)	X	X
6	SGI Score Summary Statistics by Treatment, Period and Time Point (PP Population)	X	
7	SGI Score Summary Mann-Whitney Main Effects Test Results (FAS Population)	X	X
8	SGI Score Summary Mann-Whitney Main Effects Test Results (PP Population)	X	
9	SGI Score Mixed Model Analysis (FAS Population)	X	X
10	SGI Score Mixed Model Analysis (PP Population)	X	
11	MFM-32 Score Summary Statistics by Treatment, Period and Time Point (FAS Population)	X	X
12	MFM-32 Score Summary Statistics by Treatment, Period and Time Point (PP Population)	X	
13	MFM-32 Score Summary Mann-Whitney Main Effects Test Results (FAS Population)	X	X
14	MFM-32 Score Summary Mann-Whitney Main Effects Test Results (PP Population)	X	
15	MFM-32 Score Mixed Model Analysis (FAS Population)	X	X
16	MFM-32 Score Mixed Model Analysis (PP Population)	X	
17	MFM-32 Percent Mixed Model Analysis (FAS Population)	X	X
18	MFM-32 Percent Mixed Model Analysis (PP Population)	X	
19	MFM-20 Score Summary Statistics by Treatment, Period and Time Point (FAS Population)	X	X
20	MFM-20 Score Summary Statistics by Treatment, Period and Time Point (PP Population)	X	
21	MFM-20 Score Summary Mann-Whitney Main Effects Test Results (FAS Population)	X	X
22	MFM-20 Score Summary Mann-Whitney Main Effects Test Results (PP Population)	X	
23	MFM-20 Score Mixed Model Analysis (FAS Population)	X	X
24	MFM-20 Score Mixed Model Analysis (PP Population)	X	
25	MFM-32 Score Mixed Model Analysis by Age Group (FAS Population)	X	X
26	MFM-32 Score Mixed Model Analysis by Age Group (PP Population)	X	

Table No.	Table Title	Included in Tables	Shown in Appendix B
27	MFM Percent-of-Possible Score Mixed Model Analysis (FAS Population)	X	X
28	MFM Percent-of-Possible Score Mixed Model Analysis (PP Population)	X	
29	MFM Percent-of-Possible Score Mixed Model Analysis by Age Group (FAS Population)	X	X
30	MFM Percent-of-Possible Score Mixed Model Analysis by Age Group (PP Population)	X	
31	MFM Percent-of-Possible Score Mixed Model Analysis by Mutation Type (FAS Population)	X	X
32	MFM Percent-of-Possible Score Mixed Model Analysis by Mutation Type (PP Population)	X	
33	Clinical Global Impression Summary by Mutation Type (FAS Population)	X	X
34	Clinical Global Impression Summary by Mutation Type (PP Population)	X	
35	Clinical Global Impression Score Mann-Whitney Main Effects Analysis (FAS Population)	X	X
36	Clinical Global Impression Score Mann-Whitney Main Effects Analysis (PP Population)	X	
37	Summary Statistics for Single Fiber Electromyogram Measurements (FAS Population)	X	X
38	Summary Statistics for Single Fiber Electromyogram Measurements (PP Population)	X	
39	Summary Statistics for Slurp Test Measurement by Treatment, Period, and Time Point (FAS Population)	X	X
40	Summary Statistics for Slurp Test Measurement by Treatment, Period, and Time Point (PP Population)	X	
41	Summary of Treatment Emergent Adverse Events (Safety Population)	X	X
42	Number and Percent of Patients with Treatment Emergent Adverse Events by System Organ Class and Preferred Term (Safety Population)	X	X
43	Number and Percent of Patients with Treatment Emergent Adverse Events by Severity and System Organ Class and Preferred Term (Safety Population)	X	X
44	Number and Percent of Patients with Treatment Emergent Adverse Events by Highest Relationship to Treatment and System Organ Class and Preferred Term (Safety Population)	X	X
45	Number and Percent of Patients Taking Concomitant Medications (Safety Population)	X	X
46	Clinical Laboratory Summary Statistics by Treatment and Time Point (Safety Population)	X	X
47	Number and Percent of Patients with Abnormal Laboratory Results by Treatment and Time Point (Safety Population)	X	X

Table No.	Table Title	Included in Tables	Shown in Appendix B
48	Vital Sign Parameters Summary Statistics (Safety Population)	X	X
49	Electrocardiogram Parameters Summary Statistics by Treatment and Time Point (Safety Population)	X	X
50	Electrocardiogram Shift Table (Safety Population)	X	X

Listing No.	Data Listing Title	Included in Final Listings	Shown in Appendix B
DL1	Subject Disposition Data Listing	X	X
DL2	Demographics and Baseline Characteristics Data Listing	X	X
DL3	Adverse Events Data Listing	X	X
DL4	Clinical Laboratory Tests Data Listing	X	X
DL5	Vital Signs Data Listing	X	X
DL6	SGI Data Listing	X	X
DL7	MFM 32 Data Listing	X	X
DL8	MFM 20 Data Listing	X	X
DL9	MFM 32 Summary Data Listing	X	X
DL10	MFM 20 Summary Data Listing	X	X
DL11	Physical Examination Data Listing	X	X
DL12	Medical History Data Listing	X	X
DL13	Medication Accountability Data Listing	X	X
DL14	Single Fiber Electromyogram and Slurp Test Data Listing	X	X
DL15	Electrocardiogram Data Listing	X	X
DL16	Prior and Concomitant Medications Data Listing	X	X
DL17	Population Exclusions Data Listing	X	X
DL18	Subject Data Profile	X	X
DL 19	Subject Motor Function Measure Data Profile	X	X

8.0 References

Putt, ME and Chinchilli, VM (2004) Nonparametric approaches to the analysis of crossover studies. *Statistical Science* 19(4):712- 719.

Appendix A – Tables, Figures and Listing Specifications

Orientation

Tables and figures will be displayed in landscape.

Margins

Margins will be 1 inch on all sides. Table and listing boundaries will not extend into the margins.

Font

Courier New, 8 point.

Headers

The table number will be on the first line of the title. The title area will contain the Sponsor name, the study number, and the name of the table. The title area will contain the page number (Page x of y) on the far right, one line above the name of the table.

Footers

- The first line will be a solid line.
- Next will be any footnotes regarding information displayed in the table.
- Below these footnotes will be displayed “STATKING Clinical Services (Date)” on the far left.
- The last line will display the name of the SAS program that generated the table and (if applicable) the source data reference.

Table Disclaimer

The format of the mock tables shown in the appendix of this Statistical Analysis Plan (SAP) will be the format of the deliverable tables to the extent that Word document constructed tables can match production tables produced by SAS. This formatting includes the content and format of the header and footer areas of the tables. The Sponsor agrees to the format of the tables as shown in the appendix.

Further programming charges will be applicable for changes in the format of tables (including title statements, notes, data dependent footnotes, etc.) made after the approval of the SAP.

Missing Values

All missing values will be displayed on the output tables/listings as blanks.

Display of Study Dates

The date format to be used is yyyy-mm-dd. Missing parts of dates are not shown (i.e., for a missing day value, the value displayed is in yyyy-mm format).

Appendix B – Table, Listing, and Figure Shells

Table 1. Subject Disposition
Catalyst Pharmaceuticals, Inc. - CMS-001

		Amifampridine/ Placebo (N = xx)	Placebo/ Amifampridine (N = xx)	Overall (N = xx)
Screen Failures				xx
Enrolled		xx	xx	xx
	Pre-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Post-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
Completed		xx (xxx%)	xx (xxx%)	xx (xxx%)
	Pre-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Post-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
Withdrawn		xx (xxx%)	xx (xxx%)	xx (xxx%)
	Pre-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Post-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
Reason for Withdrawal	Adverse Event	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Pre-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Post-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Lost To Follow-Up	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Pre-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Post-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Physician Decision	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Pre-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Post-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Withdrawal by Subject	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Pre-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Post-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Other	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Pre-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Post-Synaptic	xx (xxx%)	xx (xxx%)	xx (xxx%)

The denominator for all percentages in the table is the number of enrolled patients in the pertinent treatment/dose group and overall.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table 2. Baseline Disease Characteristics by Age Group
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

	Mutation Type		
Age Group	Pre-Synaptic (N=xxx)	Post-Synaptic (N=xxx)	Overall (N=xxx)
Adult (≥17 years)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Pediatric	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
2- <7 years old	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
7- <13 years old	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
13- <17 years old	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Table 3. Demographics and Baseline Data Summary Statistics – Continuous Variables
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Variable	Treatment and Mutation Type	Mean	Std Dev	n	Min	Max	Median
Age (years)	Amifampridine/Placebo	xxx	xxx	xxx	xxx	xxx	xxx
	Pre-Synaptic	xxx	xxx	xxx	xxx	xxx	xxx
	Post-Synaptic	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo/Amifampridine	xxx	xxx	xxx	xxx	xxx	xxx
	Pre-Synaptic	xxx	xxx	xxx	xxx	xxx	xxx
	Post-Synaptic	xxx	xxx	xxx	xxx	xxx	xxx
Baseline Weight (kg)	Amifampridine/Placebo	xxx	xxx	xxx	xxx	xxx	xxx
	Pre-Synaptic	xxx	xxx	xxx	xxx	xxx	xxx
	Post-Synaptic	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo/Amifampridine	xxx	xxx	xxx	xxx	xxx	xxx
	Pre-Synaptic	xxx	xxx	xxx	xxx	xxx	xxx
	Post-Synaptic	xxx	xxx	xxx	xxx	xxx	xxx

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table 4. Demographics and Baseline Data Summary Statistics- Categorical Variables
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

		Pre-Synaptic		Post-Synaptic	
Variable	Category	Amifampridine/ Placebo (N=xxx)	Placebo/ Amifampridine (N=xxx)	Amifampridine/ Placebo (N=xxx)	Placebo/ Amifampridine (N=xxx)
Gender	Male	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Female	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Ethnicity	Hispanic or Latino	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Not Hispanic or Latino	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Unknown	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Decline/Refuse to Answer	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Race	White	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Black or African American	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Asian	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	American Indian or Alaska Native	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Native Hawaiian or Other Pacific Islander	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Other	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Decline/Refuse to Answer	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Age Group	Adult	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Pediatric	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	2- <7 years old	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	7- <12 years old	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	13- <17 years old	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table 5. SGI Score Summary Statistics by Treatment, Period and Time Point
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Treatment	Study Period	Time Point	Data Type[a]	Mean	Std Dev	n	Min	Max	Median
Amifampridine/Placebo	1	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	1	Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	1	Day 8	CFB	xxx	xxx	xxx	xxx	xxx	xxx
	2	Day 21 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	2	Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	2	Day 29	CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo/Amifampridine	1	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	1	Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	1	Day 8	CFB	xxx	xxx	xxx	xxx	xxx	xxx
	2	Day 21 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	2	Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	2	Day 29	CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] RAW = observed data entered in the database; CFB = change from baseline = Score at time point – baseline score.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population.

Table 7. SGI Score Summary Mann-Whitney Main Effects Test Results
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Variable	Test	n	Mean Difference[a]	Test Statistic	P-Value
SGI	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx

[a] The Period 1 mean-of-differences is the difference in mean CFB at the end of Study Period 1, corresponding to a parallel study design analysis. The Crossover mean-of-differences is the difference between the Period 1 CFB and Period 2 CFB.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population.

Table 9. SGI Score Mixed Model Analysis
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Statistic[a]	Amifampridine/ Placebo	Placebo/ Amifampridine
n	xxx	xxx
Least Squares (LS) Mean of Raw SGI Score	xxx	xxx
Between-Treatment Difference in LS Means (Amifampridine – Placebo)	xxx	
Denominator Degrees of freedom	x	
95% CI for Between-Treatment Difference in LS Means (Amifampridine – Placebo)	(xxx, xxx)	

[a] Raw SGI score was modeled as the response, with fixed effects terms for treatment, period, age group, mutation type and sequence*period (i.e., carryover). Subject was treated as a random effect.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population.

Table 11. MFM-32 Score Summary Statistics by Treatment, Period and Time Point
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Treatment	Variable[a]	Period	Time Point	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Amifampridine/Placebo	xxxxxxxx	1	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
				CFB	xxx	xxx	xxx	xxx	xxx	xxx
		2	Day 21 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
				CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo/Amifampridine	xxxxxxx	1	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
				CFB	xxx	xxx	xxx	xxx	xxx	xxx
		2	Day 21 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
				CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] This will correspond to one of the following: Dimension 1, 2, 3, the grand total, the corresponding percentages (X1, X2, X3, X), and the individual item scores.
[b] RAW = observed data entered in the database; CFB = Change from baseline = Score at time point – baseline score.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population.

Table 13. MFM-32 Score Summary Mann-Whitney Main Effects Test Results
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Scale	Test	n	Mean of Differences[a]	Test Statistic	P-Value
Total	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
D-1	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
D-2	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
D-3	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
Item xx	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
Item yy	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
Item zz	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx

[a] The Period 1 mean-of-differences is the difference in mean CFB at the end of Study Period 1, corresponding to a parallel study design analysis. The Crossover mean-of-differences is the difference between the Period 1 CFB mean and Period 2 CFB mean. Individual items are sorted by the value of the test statistic associated with the crossover test.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Individual items will be ordered in increasing order of the p-values for the Crossover test.

Table 15. MFM-32 Score Mixed Model Analysis
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Variable	Statistic[a]	Amifampridine/ Placebo	Placebo/ Amifampridine
Overall	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D1 Domain	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline)	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D2 Domain	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D3 Domain	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
Item xx	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	

[a] Raw MFM score was modeled as the response, with fixed effects terms for treatment, period, age group, mutation type and sequence*period (i.e., carryover). Subject was treated as a random effect.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Individual items will be ordered in increasing order of the p-values for the Crossover test.

Table 17. MFM-20 Score Mixed Model Analysis
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Variable	Statistic[a]	Amifampridine/ Placebo	Placebo/ Amifampridine
Overall	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D1 Domain	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline)	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D2 Domain	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D3 Domain	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
Item xx	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	

[a] Raw MFM score was modeled as the response, with fixed effects terms for treatment, period, age group, mutation type and sequence*period (i.e., carryover). Subject was treated as a random effect.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Individual items will be ordered in increasing order of the p-values for the Crossover test.

Table 19. MFM-20 Score Summary Statistics by Treatment, Period and Time Point
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Variable[a]	Treatment Sequence	Period	Time Point	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
xxxxxxxxxx	xxxxxxxx	1	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
				CFB	xxx	xxx	xxx	xxx	xxx	xxx
		2	Day 21 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
				CFB	xxx	xxx	xxx	xxx	xxx	xxx
	xxxxxxx	1	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
				CFB	xxx	xxx	xxx	xxx	xxx	xxx
		2	Day 21 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
				CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] This will correspond to one of the following: Dimension 1, 2, 3, the grand total, the corresponding percentages (X1, X2, X3, X), and the individual items.
[b] RAW = observed data entered in the database; CFB = change from baseline.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population.

Table 21. MFM-20 Score Summary Mann-Whitney Main Effects Test Results
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Scale	Test	n	Mean of Differences[a]	Test Statistic	P-Value
Total	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
D-1	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
D-2	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
D-3	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
Item xx	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
Item yy	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx
Item zz	Period 1	xx	xxx	xxx	xxx
	Crossover	xx	xxx	xxx	xxx

[a] The Period 1 mean-of-differences is the difference in mean CFB at the end of Study Period 1, corresponding to a parallel study design analysis. The Crossover mean-of-differences is the difference between the Period 1 CFB mean and Period 2 CFB mean. Individual items are sorted by the value of the test statistic associated with the crossover test.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Individual items will be ordered in increasing order of the p-values for the Crossover test.

Table 23. MFM-20 Score Mixed Model Analysis
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Variable	Statistic[a]	Amifampridine/ Placebo	Placebo/ Amifampridine
Overall	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D1 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline)	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D2 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D3 Subscale	N	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
Item xxx	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	

[a] Raw MFM score was modeled as the response, with fixed effects terms for treatment, period, sequence*period (i.e., carryover) and mutation type at Baseline.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Individual items will be ordered in increasing order of the p-values for the Crossover test.

Table 25. MFM-32 Score Mixed Model Analysis by Age Group
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Age Group	Statistic[a]	Amifampridine/ Placebo	Placebo/ Amifampridine
Adult	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
Pediatric, 7- < 12 years	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
Pediatric, 13- < 17 years	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	

[a] Raw MFM score was modeled as the response, with fixed effects terms for treatment, period, mutation type and sequence*period (i.e., carryover). Subject was treated as a random effect.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Individual items will be ordered in increasing order of the p-values for the Crossover test.

Table 27. MFM Percent-of-Possible Score Mixed Model Analysis
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Variable	Statistic[a]	Amifampridine/ Placebo	Placebo/ Amifampridine
Overall	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D1 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline)	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D2 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D3 Subscale	N	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
Item xxx	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	

[a] MFM score expressed as a percentage of the maximum possible was modeled as the response, with fixed effects terms for treatment, period, sequence*period (i.e., carryover) and mutation type at Baseline. Individuals measured with the MFM-20 are excluded from the analyses for items in the MFM-32 excluded from the MFM-20.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Individual items will be ordered in increasing order of the p-values for the Crossover test.

Table 29. MFM Percent-of-Possible Score Mixed Model Analysis by Age Group
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Part 1 of 2: Ages 2- < 17

Variable	Statistic[a]	Amifampridine/ Placebo	Placebo/ Amifampridine
Overall	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D1 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline)	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D2 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D3 Subscale	N	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
Item xxx	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	

[a] MFM score expressed as a percentage of the maximum possible was modeled as the response, with fixed effects terms for treatment, period, sequence*period (i.e., carryover) and mutation type at Baseline. Individuals measured with the MFM-20 (i.e., aged 2- 6 years) are excluded from the analyses for items in the MFM-32 excluded from the MFM-20.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table 29. MFM Percent-of-Possible Score Mixed Model Analysis by Age Group
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Part 2 of 2: Ages 17 and above

Variable	Statistic[a]	Amifampridine/ Placebo	Placebo/ Amifampridine
Overall	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D1 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline)	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D2 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D3 Subscale	N	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
Item xxx	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	

[a] MFM score expressed as a percentage of the maximum possible was modeled as the response, with fixed effects terms for treatment, period, sequence*period (i.e., carryover) and mutation type at Baseline. Individuals measured with the MFM-20 (i.e., aged 2- 6 years) are excluded from the analyses for items in the MFM-32 excluded from the MFM-20.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Individual items will be ordered in increasing order of the p-values for the Crossover test.

Table 31. MFM Percent-of-Possible Score Mixed Model Analysis by Mutation Type
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Part x of y: XXXXXX Mutation

Variable	Statistic[a]	Amifampridine/ Placebo	Placebo/ Amifampridine
Overall	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D1 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline)	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D2 Subscale	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
D3 Subscale	N	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	
Item xxx	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline	xxx	xxx
	Between-Treatment Difference in LS Means (A-B)	xxx	
	Denominator degrees of freedom	x	
	95% CI for Between-Treatment Difference in LS Means (A-B)	(xxx, xxx)	

[a] MFM score expressed as a percentage of the maximum possible was modeled as the response, with fixed effects terms for treatment, period, sequence*period (i.e., carryover) and mutation type at Baseline. Individuals measured with the MFM-20 (i.e., aged 2- <7 years) are excluded from the analyses for items in the MFM-32 excluded from the MFM-20.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Parts will be included for all subjects, pre- and post- synaptic mutations and each mutation type. Individual items will be ordered in increasing order of the p-values for the Crossover test.

Page x of y

Table 33. Clinical Global Impression Summary by Mutation Type
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Part x of y: xxxxxxxxxx Mutation

Variable	Treatment Sequence	Time Point	Type[a]	1	2	3	4	5	6	7
CGI-Severity	Amifampridine/Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo/Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
CGI-Improvement	Amifampridine/Placebo	Day 0 (Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo/Amifampridine	Day 0 (Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx
		Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxx

[a] RAW = observed data entered in the database; CFB = change from baseline = Value at timepoint − Baseline value.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population. Parts will be included for all subjects, pre- and post- synaptic mutations and each mutation type.

Page x of y

Table 35. Clinical Global Impression Score Mann-Whitney Main Effects Analysis
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Subgroup	Variable	Time Point	Hodges-Lehmann Estimate	90% Confidence Limits	Mann-Whitney Test P-value
All Subjects	CGI-Severity	Day 8	xxxx	(xxxx, xxxx)	xxxxx
		Day 21	xxxx	(xxxx, xxxx)	xxxxx
	CGI-Improvement	Day 8	xxxx	(xxxx, xxxx)	xxxxx
		Day 21	xxxx	(xxxx, xxxx)	xxxxx
xxxxxxxxxx	CGI-Severity	Day 8	xxxx	(xxxx, xxxx)	xxxxx
		Day 21	xxxx	(xxxx, xxxx)	xxxxx
	CGI-Improvement	Day 8	xxxx	(xxxx, xxxx)	xxxxx
		Day 21	xxxx	(xxxx, xxxx)	xxxxx
xxxxxxxxx	CGI-Severity	Day 8	xxxx	(xxxx, xxxx)	xxxxx
		Day 21	xxxx	(xxxx, xxxx)	xxxxx
	CGI-Improvement	Day 8	xxxx	(xxxx, xxxx)	xxxxx
		Day 21	xxxx	(xxxx, xxxx)	xxxxx

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population.
Subgroups will be included for all subjects, pre- and post-synaptic mutations and each mutation type.

Table 37. Summary Statistics for Single Fiber Electromyogram Measurements
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Treatment Sequence	Variable	Time Point	Data Type[a]	Mean	Std Dev	n	Min	Max	Median
Amifampridine/Placebo	xxxxxxxx	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo/Amifampridine	xxxxxxx	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] RAW = observed data entered in the database; CFB = change from baseline = Value at timepoint – Baseline value.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population.
Variable will correspond to one of the following: Mean Jitter, Median Jitter, and Percent Block

Table 39. Summary Statistics for Slurp Test Measurement by Treatment, Period and Time Point
Catalyst Pharmaceuticals, Inc. - CMS-001
FAS Population (N=xxx)

Treatment	Time Point	Data Type[a]	Mean	Std Dev	n	Min	Max	Median
Amifampridine/Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Day 8	CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Day 21 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Day 29	CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo/Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Day 8	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Day 8	CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Day 21 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Day 29	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Day 29	CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] RAW = observed data entered in the database; CFB = change from baseline = Value at timepoint – Baseline value.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table format repeats for PP population

Table 41. Summary of Treatment Emergent Adverse Events
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxxx)

	Amifampradine[a]	Placebo[a]
Patients With ≥ 1 Treatment Emergent Adverse Event (TEAE)	xxx (xxx%)	xxx (xxx%)
Maximum TEAE Severity		
Mild	xxx (xxx%)	xxx (xxx%)
Moderate	xxx (xxx%)	xxx (xxx%)
Severe	xxx (xxx%)	xxx (xxx%)
Highest Relationship of TEAE to Treatment		
Not Related [n(%)]	xxx (xxx%)	xxx (xxx%)
Unlikely [n(%)]	xxx (xxx%)	xxx (xxx%)
Possible [n(%)]	xxx (xxx%)	xxx (xxx%)
Probable [n(%)]	xxx (xxx%)	xxx (xxx%)
Definite [n(%)]	xxx (xxx%)	xxx (xxx%)
Patients Who Discontinued Dosing Prematurely due to AE	xxx (xxx%)	xxx (xxx%)
Patients with ≥ 1 Serious TEAE	xxx (xxx%)	xxx (xxx%)
Deaths	xxx (xxx%)	xxx (xxx%)

[a] The denominator for all percentages will be the number of patients in the safety population.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Table 42. Number and Percent of Patients with Treatment Emergent Adverse Events by System Organ Class and Preferred Term
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Adverse Event Category[b]	Amifampridine[a]	Placebo[a]	Overall[a]
Total Number of TEAEs	xxx	xxx	xxx
Patients with ≥ 1 TEAE	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] The denominator for all percentages will be the number of patients in the safety population.
[b] Adverse events coded with MedDRA Coding Dictionary Version XXX.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table 43. Number and Percent of Patients with Treatment Emergent Adverse Events
by Severity and System Organ Class and Preferred Term
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Part 1 of 3
Treatment at Time of Onset = Amifampridine

Adverse Event Category[a]:	Mild[b]	Moderate[b]	Severe[b]
Total Number of TEAEs	xxx	xxx	xxx
Patients with ≥ 1 TEAE	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] Adverse events coded with MedDRA Coding Dictionary Version XXX.
[b] The denominator for all percentages will be the number of patients in the safety population.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Format repeats for Treatment at Time of Onset for Amifampridine, Placebo, and Overall.

Table 44. Number and Percent of Patients with Treatment Emergent Adverse Events
by Highest Relationship to Treatment and System Organ Class and Preferred Term
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Part x of 3
Treatment at Time of Onset = Amifampridine

Adverse Event Category[a, b]:	Not Related	Unlikely	Possible	Probable	Definitely Related
Total Number of TEAEs	xxx	xxx	xxx	xxx	xxx
Patients with ≥ 1 TEAE	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] The denominator for all percentages will be the number of patients in the safety population.
[b] Adverse events coded with MedDRA Coding Dictionary Version XXX.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Format repeats for Treatment at Time of Onset for Amifampridine, Placebo, and Overall.

Table 45. Number and Percent of Patients Taking Concomitant Medications
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Medication[a, b]:	Amifampridine	Placebo	Overall
Total Number of Concomitant Medications	xxx	xxx	xxx
Patients Taking ≥ 1 Concomitant Medication	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
ATC Level 4 Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
ATC Level 4 Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] The denominator for all percentages will be the number of patients in the safety population.
[b] Medications coded with WHO Coding Dictionary xxxxxxxx.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table 46. Clinical Laboratory Summary Statistics by Treatment and Time Point
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Laboratory Parameter (units)	Treatment Sequence	Time Point	Data Type[a]	Mean	Std Dev	n	Min	Max	Median
xxxxxxxxxxxxxx (xxxx)	Amifampridine/Placebo	Screening	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day X	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFP	xxx	xxx	xxx	xxx	xxx	xxx
		Day X	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFP	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo/Amifampridine	Screening	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day X	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFP	xxx	xxx	xxx	xxx	xxx	xxx
		Day X	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFP	xxx	xxx	xxx	xxx	xxx	xxx

[a] RAW = observed data recorded in database; CFP = change from previous assessment = Value at Timepoint – Value at Previous Assessment.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table 47. Number and Percent of Patients with Abnormal Laboratory Results by Treatment and Time Point
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Laboratory Parameter (units)	Time Point	Treatment at Time of Assessment: Amifampridine	Treatment at Time of Assessment: Placebo
xxxxxxxxxxxx	Screening	xxx (xxxx%)	xxx (xxxx%)
	Day X	xxx (xxxx%)	xxx (xxxx%)
	Screening	xxx (xxxx%)	xxx (xxxx%)
	Day X	xxx (xxxx%)	xxx (xxxx%)

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table 48. Vital Sign Parameters Summary Statistics
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Vital Sign Parameter (units)	Visit	Data Type[a]	Mean	Std Dev	n	Min	Max	Median
Amifampridine/Placebo	xxxxxxxxxx (xxx)	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day X	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFP	xxx	xxx	xxx	xxx	xxx	xxx
Placebo/Amifampridine	xxxxxxxxxx (xxx)	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day X	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFP	xxx	xxx	xxx	xxx	xxx	xxx

[a] RAW = observed data recorded in database; CFP = change from previous assessment = Value at Timepoint – Value at Previous Assessment.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Table 49. Electrocardiogram Parameters Summary Statistics
By Treatment and Time Point
Catalyst Pharmaceuticals, Inc. CMS-001
Safety Population (N=xxx)

Parameter (Units)	Treatment Sequence	Visit	Data Type[a]	n	Mean (msec)	Std Dev	Min	Median	Max
xxxxxxxxxxxxxx (xxx)	Amifampridine/Placebo	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day 0 (Randomization)	RAW	xxx	xxx	xxx	xxx	xxx	Xxx
			CFB	xxx	xxx	xxx	xxx	xxx	Xxx
		End of study	RAW	xxx	xxx	xxx	xxx	xxx	Xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo/Amifampridine	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day 0 (Randomization)	RAW	xxx	xxx	xxx	xxx	xxx	Xxx
			CFB	xxx	xxx	xxx	xxx	xxx	Xxx
		End of study	RAW	xxx	xxx	xxx	xxx	xxx	Xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] RAW = observed data recorded in database; CFB = Change from Baseline (Screening ECG) = Time point value – Baseline Value.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Table 50. Electrocardiogram Shift Table
Catalyst Pharmaceuticals, Inc. CMS-001
Safety Population (N=xxx)

Time Point	Treatment Groups	Screening Normal/ Time Point[a] Normal	Screening Normal/ Time Point[a] Abnormal	Screening Abnormal/ Time Point[a] Normal	Screening Abnormal/ Time Point[a] Abnormal
End of Period 2	Amifampridine/Placebo	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Placebo/Amifampridine	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
End of Study	Amifampridine/Placebo	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Placebo/Amifampridine	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] End of study is Day 29 or day of withdrawal from study, whichever is earlier.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Data Listing 1. Subject Disposition Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001

Treatment Sequence	Subject No.	Disposition Status	Date of Disposition	Withdrawal Reason
x/x	xxxx	xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxx
x/x	xxxx	xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxx
x/x	xxxx	xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxx
x/x	xxxx	xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxx

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Data Listing 2. Demographics and Baseline Characteristics Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Subject No.	Informed Consent Date	Date of Birth	Age (yrs)	Mutation Type	Gender	Ethnicity	Screening Weight (kg)
x/x	xxxx	xxxxxx	xxxxxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxxxxx
x/x	xxxx	xxxxxx	xxxxxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxxxxx
x/x	xxxx	xxxxxx	xxxxxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxxxxx
x/x	xxxx	xxxxxx	xxxxxx	xxx	xxxxxx	xxxxxx	xxxxxx	xxxxxx

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Data Listing 3. Adverse Events Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Subject No.	Treatment at AE Onset	Start Date/ Stop Date	Duration (Days)	MedDRA System Organ Class[a]/ MedDRA Preferred Term/ CRF Verbatim Event Name	Relationship to Study Treatment	Severity	Serious?	Action Taken	Outcome
x/x	xxxxxx	xxxxxx	xxxxxx/ xxxxxx	xxxxx	xxxxxxxxxxxxxxxxxxxx/ xxxxxxxxxxxxxxxxxxxx/ xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxx	xxx	xxxxxx	xxxxxx
x/x	xxxxxx	xxxxxx	xxxxxx/ xxxxxx	xxxxx	xxxxxxxxxxxxxxxxxxxx/ xxxxxxxxxxxxxxxxxxxx/ xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxx	xxx	xxxxxx	xxxxxx

[a] Adverse events coded with MedDRA Coding Dictionary Version xxx.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Data Listing 4. Clinical Laboratory Tests Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Subject No.	Sample Date/Time	Treatment at Time of Sample Collection	Lab Panel/ Lab Test	Result (Units)	Normal Range Low	Normal Range High	Findings
x/x	xxxxxx	xxxxxx	xxxxxx	xxxxxxxxxxxx/ xxxxxxxxxxx	xxxxxx	xxx	xxx	xxx
x/x	xxxxxx	xxxxxx	xxxxxx	xxxxxxxxxxxx/ xxxxxxxxxxx	xxxxxx	xxx	xxx	xxx

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Data Listing 5. Vital Signs Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Treatment At Time of Measurement	Subject No.	Visit	Date	Time	Temp. (°F)	Systolic Blood Pressure (mmHg)	Diastolic Blood Pressure (mmHg)	Heart Rate (bpm)	Respiratory Rate (breaths/min)
x/x	xxxxxx	xxxx	xxxxxxx	xxxxxxx	xxxxx	xxx	xxx	xxx	xxx	xxx
					xxxxx	xxx	xxx	xxx	xxx	xxx
					xxxxx	xxx	xxx	xxx	xxx	xxx
x/x	xxxxxx	xxxx	xxxxxxx	xxxxxxx	xxxxx	xxx	xxx	xxx	xxx	xxx
					xxxxx	xxx	xxx	xxx	xxx	xxx
					xxxxx	xxx	xxx	xxx	xxx	xxx

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Data Listing 6. SGI Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Treatment At Time of Assessment	Subject No.	Visit	Impression of Effects of Study Medication During Preceding Week on Physical Well Being[a]
x/x	xxxxxx	xxxx	xxxxxxxx	xxxx
x/x	xxxxxx	xxxx	xxxxxxxx	xxxx

[a] 1=Terrible; 2=Mostly Dissatisfied; 3=Mixed; 4=Partially Satisfied; 5=Mostly Satisfied; 6=Pleased; 7=Delighted.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Data Listing 7. MFM-32 Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Treatment At Time of Assessment	Subject No.	Age	Visit	Dimension[a]/ Item	Motor Function Measure	Response[b]
x/x	xxxxxx	xxxx	xxxx	xxxxxxxx	x/xx	xxxxxxxxxxxx	xxxx
x/x	xxxxxx	xxxx	xxxx	xxxxxxxx	x/xx	xxxxxxxxxxxx	xxxx

[a] 1=Standing and Transfer; 2=Axial and Proximal Motor Function; 3=Distal Motor Function.
[b] 0=Cannot initiate task; 1=partially performs task; 2=performs the movement incompletely, or completely but imperfectly; 3=performs the task fully and "normally".
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Data Listing 8. MFM-20 Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Treatment at Time of Assessment	Subject No.	Age	Visit	Dimension[a]/ Item	Motor Function Measure	Response[b]
xxxxxx	x/x	xxxx	xxxx	xxxxxxxx	x/xx	xxxxxxxxxxx	xxxx
xxxxxx	x/x	xxxx	xxxx	xxxxxxxx	x/xx	xxxxxxxxxxx	xxxx

[a] 1=Standing and Transfer; 2=Axial and Proximal Motor Function; 3=Distal Motor Function.
[b] 0=Cannot initiate task; 1=partially performs task; 2=performs the movement incompletely, or completely but imperfectly; 3=performs the task fully and "normally".
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Data Listing 9. MFM-32 Summary Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Treatment at Time of Assessment	Subject No.	Visit	Dimension[a]	Value	Percent[b]
x/x	xxxxxx	xxxx	xxxxxxxx	T1	xxxx	xxxx
				T2	xxxx	xxxx
				T3	xxxx	xxxx
				T (T1+T2+T3)	xxxx	xxxx
				1	xxx	
				2	xxx	
				3	xxx	
				x	xxx	
				32	xxx	
x/x	xxxxxx	xxxx	xxxxxxxx	T1	xxxx	xxxx
				T2	xxxx	xxxx
				T3	xxxx	xxxx
				T (T1+T2+T3)	xxxx	xxxx
				1	xxx	
				2	xxx	
				3	xxx	
				x	xxx	
				32	xxx	

[a] Corresponds to the subtotal score for the given dimension. T1=Standing and Transfer; T2=Axial and Proximal Motor Function; T3=Distal Motor Function or the item number.
[b] Percentage of the corresponding maximum possible score.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Data Listing 10. MFM-20 Summary Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Treatment At Time of Assessment	Subject No.	Visit	Dimension[a]	Value	Percent[b]
x/x	xxxxxx	xxxx	xxxxxxxx	T1	xxxx	xxxx
				T2	xxxx	xxxx
				T3	xxxx	xxxx
				T (T1+T2+T3)	xxxx	xxxx
				1	xxx	
				2	xxx	
				3	xxx	
				x	xxx	
				20	xxx	
x/x	xxxxxx	xxxx	xxxxxxxx	T1	xxxx	xxxx
				T2	xxxx	xxxx
				T3	xxxx	xxxx
				T (T1+T2+T3)	xxxx	xxxx
				1	xxx	
				2	xxx	
				3	xxx	
				x	xxx	
				20	xxx	

[a] Corresponds to the subtotal score for the given dimension. T1=Standing and Transfer; T2=Axial and Proximal Motor Function; T3=Distal Motor Function or the item number.
[b] Percentage of the corresponding maximum possible score.
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Data Listing 11. Physical Examination Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Subject No.	Visit	Date Conducted	Body System	Result	Abnormality
x/x	xxxx	xxxxxxx	xxxxxxx	xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
				xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
				xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
				xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
				xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
				xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
				xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
				xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
				xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
				xxxxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Data Listing 12. Medical History Data Listing
Catalyst Pharmaceuticals, Inc. – CMS-001
Safety Population (N=xxx)

Treatment Sequence	Subject No.	MedDRA System Organ Class[a]/ MedDRA Preferred Term/ CRF Verbatim Term	Start Date	Ongoing?
xxxxxxxxx/xxxxxxxxxxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxx	xxx
		xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxx	xxx
		xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxx	xxx

[a] Medical history terms coded with MedDRA Coding Dictionary Version xxx.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 13. Medication Accountability Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Subject No.	Treatment Start Date	Treatment End Date	Treatment Duration (Days)	Tablets Dispensed	Dose (mg/day)	Tablets Consumed	Compliance (%)[a]
xxxxxxxxx/xxxxxxxxxxx	xxxxxx	xxxxxx	xxxxxx	xxx	xxx	xxx	xxx	xxx
xxxxxxxxx/xxxxxxxxxx	xxxxxx	xxxxxx	xxxxxx	xxx	xxx	xxx	xxx	xxx

[a] Compliance is computed as 100*(number of tablets consumed)/(number of tables dispensed).
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Data Listing 14. Single Fiber Electromyelogram and Slurp Test Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Subject No.	Date	Visit	Mean Jitter	Median Jitter	Percent Blockage	Slurp Test
x/x	xxxxxxxx	xxxxxxxx	xxxxxxx	xxxxx	xxxxx	xxxxx	xxxxxxxx
x/x	xxxxxxxx	xxxxxxxx	xxxxxxx	xxxxx	xxxxx	xxxxx	xxxxxxxx
x/x	xxxxxxxx	xxxxxxxx	xxxxxxx	xxxxx	xxxxx	xxxxx	xxxxxxxx
x/x	xxxxxxxx	xxxxxxxx	xxxxxxx	xxxxx	xxxxx	xxxxx	xxxxxxxx
x/x	xxxxxxxx	xxxxxxxx	xxxxxxx	xxxxx	xxxxx	xxxxx	xxxxxxxx

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Data Listing 15. Electrocardiogram Data Listing
Catalyst Pharmaceuticals – CMS-001
Safety Population (N=xxx)

Treatment Sequence	Subject No.	Time Point	Date	Time	Heart Rate	PR Interval	QRS Duration	QT Interval	ECG Assessment/ If Abnormal, Specify
x/x	xxxxxxx	Screen	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
		Day 0	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
		Day 10	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx

STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 16. Prior and Concomitant Medications Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001
Safety Population (N=xxx)

Treatment Sequence	Treatment at Medication Start Date	Subject No.	WHO Preferred Term[a]/ Verbatim Drug Name/ ATC Level 4 Term	Indication	Start Date	Stop Date	Route	Ongoing?
x/x	xxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxx	xxxxxxx	xxxxxxx	xxxxx	xxxxx
x/x	xxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxx	xxxxxxx	xxxxxxx	xxxxx	xxxxx

[a] Concomitant medications coded with WHO Coding Dictionary xxxxxxxxx
STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Data Listing 17. Population Exclusions Data Listing
Catalyst Pharmaceuticals, Inc. - CMS-001

Subject No.	Exclusion	Reason for Exclusion
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxxxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx

STATKING Clinical Services (yyyy-mm-dd)
Source Program: xxxxxxx.sas

Page x of y

Data Listing 18. Subject Data Profile
Catalyst Pharmaceuticals, Inc. – CMS-001
Safety Population (N=xxx)

Subject No.: xxxx

Study Number: CMS-001 Site: xxxxx

Randomization Code: xxxx Sequence: xxxxxx Dose: xxxxxx Dose Group: xxxx
Age (yrs): xxxx Gender: xxxxxx Ethnicity: xxxxxxx

Screening Weight (kg): xxxx Mutation: xxxxxxxx Mutation Type: xxxxxx

Endpoint Measurements

Subject Global Impression Scores

Visit	Date		Score	CFB
Screening	xx-xxx-xxxx	Total	xxxxx	--
Day x	xx-xxx-xxxx	Total	xxxxx	xxxxx

Clinical Global Impression-Severity Scores

Visit	Date		Score	CFB
Screening	xx-xxx-xxxx	Total	xxxxx	--
Day x	xx-xxx-xxxx	Total	xxxxx	xxxxx

Clinical Global Impression-Improvement Scores

Visit	Date		Score	CFB
Screening	xx-xxx-xxxx	Total	xxxxx	--
Day x	xx-xxx-xxxx	Total	xxxxx	xxxxx

Stimulated Single Fiber Electromyelogram

Visit	Date	Parameter	Score
Day 8	xx-xxx-xxxx	xxxxxxxxxx	xxxxx
	xx-xxx-xxxx	xxxxxxxxxx	xxxxx
	xx-xxx-xxxx	xxxxxxxxxx	xxxxx

Slurp Test

Visit	Date		Score	CFB
Screening	xx-xxx-xxxx	Time to slurp sound	xxxxx	--
Day x	xx-xxx-xxxx	Time to slurp sound	xxxxx	xxxxx

STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Listing continues on next page

Page x of y

Data Listing 18. Subject Data Profile
Catalyst Pharmaceuticals, Inc. – CMS-001
Safety Population (N=xxx)

Subject No.: xxxx

Study Number: CMS-001 Site: xxxxxxxxx

Motor Function Measure Scores
Form: xxx-xx

Visit	Date	Item	Score	CFB[a]	Treatment Main Effect Difference[b]
Day 0	xx-xxx-xxxxx	D1: Standing and Transfer	xxxxx		
		D2: Axial and Proximal Motor Function	xxxxx		
		D3: Distal Motor Function	xxxxx		
		Total	xxxxx		
		Item xx	xxxxx		
Day 8	xx-xxx-xxxx	D1: Standing and Transfer	xxxxx	xxxxx	
		D2: Axial and Proximal Motor Function	xxxxx	xxxxx	
		D3: Distal Motor Function	xxxxx	xxxxx	
		Total	xxxxx	xxxxx	
		Item xx	xxxxx	xxxxx	
Day 21	xx-xxx-xxxxx	D1: Standing and Transfer	xxxxx		
		D2: Axial and Proximal Motor Function	xxxxx		
		D3: Distal Motor Function	xxxxx		
		Total	xxxxx		
		Item xx	xxxxx		
Day 29	xx-xxx-xxxx	D1: Standing and Transfer	xxxxx	xxxxx	xxxxx
		D2: Axial and Proximal Motor Function	xxxxx	xxxxx	xxxxx
		D3: Distal Motor Function	xxxxx	xxxxx	xxxxx
		Total	xxxxx	xxxxx	xxxxx
		Item xx	xxxxx	xxxxx	xxxxx

[a] CFB = Change from Baseline = Value at time point – Baseline value. Baseline scores are Day 0 for Period 1 and Day 21 for Period 2.
[b] The Treatment Main Effect Difference is the difference in CFB for Day 8 and Day 29.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Listing continues on next page

Page x of y

Data Listing 18. Subject Data Profile
Catalyst Pharmaceuticals, Inc. – CMS-001
Safety Population (N=xxx)

Subject No.: xxxx

Study Number: CMS-001 Site: xxxxxxxxx

Laboratory Values

Visit	Date	Parameter (units)	Result	Abnormal Criterion
Baseline	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
Day x	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	

Electrocardiogram Value

Visit	Date	Parameter (units)	Result	Abnormal Criterion
Baseline	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
Day x	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	

[a] CFB = Change from Baseline = Value at time point – Baseline value. Baseline scores are Day 0 for Period 1 and Day 21 for Period 2.
[b] The Treatment Main Effect Difference is the difference in CFB for Day 8 and Day 29.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Listing continues on next page

Data Listing 18 – Subject Data Profile
Catalyst Pharmaceuticals, Inc – CMS-001
Safety Population (N=xxx)

Subject No.: xxxx

Study Number: CMS-001 Site: xxxxxxxxx

Vital Signs

Visit	Date	Parameter (units)	Result	Abnormal Criterion
Baseline	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
Day x	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxxxx	xxxx	

Adverse Event[a]

Preferred Term	Date	System Organ Class	Severity	Treatment Related?
Xxxxxxxxxx	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	
Xxxxxxxxxx	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	xxxxxxxxxx
Xxxxxxxxxx	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	xxxxxxxxxx
Xxxxxxxxxx	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	xxxxxxxxxx

[a] Adverse events coded with MedDRA Coding Dictionary Version xxx.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Listing continues on next page

Page x of y

Data Listing 18 – Subject Data Profile
Catalyst Pharmaceuticals, Inc – CMS-001
Safety Population (N=xxx)

Subject No.: xxxx

Study Number: CMS-001 Site: xxxxxxxxx

Prior and Concomitant Medication[b]

Preferred Term	Dose	Start Date	Stop Date
xxxxxxx	xxxxx	xx-xxx-xxxx	xx-xxx-xxxx
xxxxxxx	xxxxx	xx-xxx-xxxx	xx-xxx-xxxx
xxxxxxx	xxxxx	xx-xxx-xxxx	xx-xxx-xxxx
xxxxxxx	xxxxx	xx-xxx-xxxx	xx-xxx-xxxx
xxxxxxx	xxxxx	xx-xxx-xxxx	xx-xxx-xxxx

Physical Exam Findings

Date	System	Findings
xx-xxx-xxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxxxx

[b] Concomitant medications coded with WHO Coding Dictionary xxxxxxxxxxxxx.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Listing repeats per Subject beginning on a new page.
MFM Item entries in this listing will be sorted in item order (i.e., Item 1, Item 2, …, Item 32)

Page x of y

Data Listing 19. Subject Motor Function Measure Data Profile
Catalyst Pharmaceuticals, Inc. – CMS-001
Safety Population (N=xxx)

Subject No.: xxxx

Study Number: CMS-001 Site: xxxxxxxxx

Randomization Code: xxxx Sequence: xxxxxx Dose: xxxxxx Dose Group: xxxx
Age (yrs): xxxx Gender: xxxxxx Ethnicity: xxxxx

Screening Weight (kg): xxxx Mutation: xxxxxxxx Mutation Type: xxxxxxxxxxxxx

Motor Function Measure Scores
Form: xxx-xx

Visit	Date	Item	Score	CFB[a]	Treatment Main Effect Difference[b]
Day 0	xx-xxx-xxxxx	D1: Standing and Transfer	xxxxx		
		D2: Axial and Proximal Motor Function	xxxxx		
		D3: Distal Motor Function	xxxxx		
		Total	xxxxx		
		Item xx	xxxxx		
Day 8	xx-xxx-xxxx	D1: Standing and Transfer	xxxxx	xxxxx	
		D2: Axial and Proximal Motor Function	xxxxx	xxxxx	
		D3: Distal Motor Function	xxxxx	xxxxx	
		Total	xxxxx	xxxxx	
		Item xx	xxxxx	xxxxx	
Day 21	xx-xxx-xxxxx	D1: Standing and Transfer	xxxxx		
		D2: Axial and Proximal Motor Function	xxxxx		
		D3: Distal Motor Function	xxxxx		
		Total	xxxxx		
		Item xx	xxxxx		
Day 29	xx-xxx-xxxx	D1: Standing and Transfer	xxxxx	xxxxx	xxxxx
		D2: Axial and Proximal Motor Function	xxxxx	xxxxx	xxxxx
		D3: Distal Motor Function	xxxxx	xxxxx	xxxxx
		Total	xxxxx	xxxxx	xxxxx
		Item xx	xxxxx	xxxxx	xxxxx

[a] CFB = Change from Baseline = Value at time point – Baseline value. Baseline scores are Day 0 for Period 1 and Day 21 for Period 2.
[b] The Treatment Main Effect Difference is the difference in CFB for Day 8 and Day 29.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Item entries in this listing will be sorted in decreasing order of the Treatment Main Effect Difference.