Statistical Analysis Plan (SAP)

Study No. LMS-003

A Phase 3, Double-Blind, Placebo-Controlled, Randomized, Parallel-Group Study to Evaluate the Efficacy and Safety of Amifampridine Phosphate (3,4-Diaminopyridine Phosphate) in Patients with Lambert-Eaton Myasthenic Syndrome (LEMS)

Version 1.0

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Prepared for
Catalyst Pharmaceuticals, Inc.
355 Alhambra Circle, Suite 1250
Coral Gables, FL 33134

Prepared by
STATKING Clinical Services
759 Wessel Drive
Fairfield, OH 45014
513-858-2989
www.statkingclinical.com

STATKING Clinical Services

Approval Page

I agree to the format and content of this document.

Approved by:

Steven Miller, PhD **Date**
Chief Operating Officer and Chief Scientific Officer
Catalyst Pharmaceuticals, Inc.
355 Alhambra Circle, Suite 1250
Coral Gables, FL 33134
smiller@catalystpharma.com

Authored by:

Dennis Clason, PhD **Date**
Statistician
STATKING Clinical Services
759 Wessel Drive
Fairfield, OH 45014
513-858-2989 ext. 313
dclason@statkingclinical.com

Approved by (internal review):

Lori Christman, PhD **Date**
Statistician
STATKING Clinical Services
759 Wessel Drive
Fairfield, OH 45014
513-858-2989 ext. 317
lori@statkingclinical.com

Approved by:

Clare Geiger, RN **Date**
Project Manager
STATKING Clinical Services
759 Wessel Drive
Fairfield, OH 45014
513-858-2989 ext. 304
clare@statkingclinical.com

Revision History

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1.0 Synopsis of Study Design Procedures

This study is a prospective, randomized, placebo-controlled, multicenter study to evaluate the efficacy and safety of amifampridine phosphate in subjects diagnosed with Lambert Eaton Myasthenic Syndrome (LEMS). The endpoint assessments of this Phase 3 study are as follows:

- Primary:
  - To assess the clinical efficacy of amifampridine phosphate compared with placebo in adults with LEMS, based on change of the co-primary endpoints Quantitative Myasthenia Gravis (QMG) Score and Subject Global Impression (SGI).
- Secondary:
  - Clinical Global Impression of Improvement (CGI-I)
- Exploratory:
  - Greater than 20% increase in the average time of 3 repetitions for Timed Up and Go test (3TUG)
  - Subject rating on change of most bothersome symptom for them.

1.1 Design and Treatment

Subjects will be randomized on Day 0 to either treatment group in a 1:1 ratio. Investigational product (IP) will be administered under double-blind conditions such that neither the Investigator nor subject knows if they are taking placebo or amifampridine phosphate.

Amifampridine phosphate (at the subject's optimal dose established prior to enrollment into this trial) or placebo will be dispensed by the site pharmacist, according to the randomization schedule, to begin with the next dose after all Day 0 assessments are completed and continued for 4 days. Although subjects at the high (dose $\geq$ 60 mg/day) and low (dose < 60 mg/day) are randomized separately, the study is not powered to detect differences across dose levels and no inferential analyses are planned to compare dose levels (See Section 1.3). The amifampridine phosphate dose is 30 mg to 80 mg total daily dose (expressed as freebase form), given in 3 to 4 divided doses, with no single dose >20 mg.

1.2 Study Procedures

The planned duration of participation for each subject is 5 days (Day 0 through Day 4), excluding the screening period, which can last up to 7 days. Those subjects satisfying all inclusion and exclusion criteria will be randomized to a Treatment Group on the last day of the screening period (Day 0). The treatment period will take place on the remainder of Day 0 and Days 1 through 4. The following assessments will be performed at the beginning and end of the 4-day treatment period:

- Vital signs
- Urine sample for amifampridine level (collected at days 3, and 4)
- Clinical Global Impression of Improvement (CGI-I)
- Subject Global Impression (SGI)
- Quantitative Myasthenia Gravis (QMG)
- Triple Timed Up and Go (3TUG)
- Patient most bothersome symptom question
- Record concomitant medications
- Monitor for adverse events (throughout the 5-day treatment period)

1.3 Sample Size

The study is powered with respect to the co-primary efficacy endpoints of the study. For change from baseline (CFB) in QMG Scores, a between-treatment difference of -3.5 and a standard deviation of at most 3, a sample size of at least 24 subjects will provide power of at least 80% for a 0.05 level two-sided test. Similarly, for CFB in SGI Scores, a between-treatment difference of -2.1 and a standard deviation of at most 2, a sample size of at least 26 subjects will provide power of 80% for a 0.05 level two-sided test. Thus a total sample size of 26 subjects, equally randomized to two treatment sequences, will provide power of at least 80% for each of the two co-primary endpoints.

The randomization is stratified by high and low dose of the investigational product. Due to the fact that each dose strata has an insufficient number of observations to be adequately powered to do meaningful hypothesis testing, no statistical comparisons by treatment will be done for individual dose-strata subgroups.

2.0 Data Analysis Considerations

2.1 Types of Analyses

Analyses will consist of summarizing efficacy and safety data. Unless otherwise stated, two-sided P values <0.05 will be considered as statistically significant.

The following standards will be applied for the analyses unless otherwise specified. Simple summary statistics (descriptive statistics) for continuous data are: 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 by treatment. All data collected will be presented in the by-subject data listings, sorted by subject and by time point, where appropriate.

Additionally, the randomization is stratified by high and low dose of the investigational product. Therefore, descriptive statistics, grouped by both

treatment and dose level strata, will be presented using the same descriptive analyses described above for continuous and categorical data, as applicable.

2.2 Analysis Populations

The following analysis populations will be defined:

Safety: This population consists of all randomized subjects who receive at least 1 dose of IP (amifampridine or placebo).

Full Analysis Set (FAS), Intent to Treat Population: This population consists of all randomized subjects who receive at least 1 dose of IP (amifampridine or placebo) and have at least one post-treatment efficacy assessment.

Per Protocol (PP): This population is a subset of the FAS population, excluding subjects with major protocol deviations. The PP population will include all subjects who:

- Have no major protocol deviations or inclusion/exclusion criteria deviations that might potentially affect efficacy, and
- Subjects who took at least 80% of the required treatment doses and remained enrolled for at least 4 days.

Evaluations obtained at the time of discontinuation will be included in the FAS and PP analyses, as applicable. Subjects who discontinue with no post-randomization data (no Day 0 and no Day 4 data) will be excluded from all efficacy analyses but will be included in the safety analyses.

Exclusion from the FAS and PP Populations will be finalized prior to database lock and subsequent unblinding.

2.2.1 Subgroup Definitions

Descriptive statistics for each efficacy variable will be provided by low dose (less than 60 mg/day) and high dose (60 mg/day, and higher) subgroups for subjects receiving amifampridine or placebo. No hypotheses will be tested in these subgroup analyses. See Section 7.0 for a list of tables presenting results by dose group.

2.3 Missing Data Conventions

No missing value imputation will be used in this analysis. All analyses will be based on the observed data. For subjects that discontinue prior to Day 4 due to treatment related disability ("Rescue"), the observations collected at the time of rescue will be analyzed with the other Day 4 observations.

2.4 Interim Analyses

There are no interim analyses planned for this study.

2.5 Study Center Considerations in the Data Analysis

A study center is defined as a treatment administration site or group of treatment administration sites under the control and supervision of the same Principal Investigator (PI).

There will be no selective pooling of study centers in the analysis. All calculations will be made on the combined results of all centers.

2.6 Documentation and Other Considerations

The data 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 displayed via summary statistics (mean, median, sample size, standard deviation, minimum, and maximum). Gender and ethnicity will be summarized via counts and percentages.

A detailed listing of demographics data for each subject will also be provided as shown in Appendix B.

4.0 Analysis of Efficacy

4.1 Description of Efficacy Variables

4.1.1 Primary Efficacy Variables

The co-primary efficacy variables for the study are the following:

- Subject Global Impression (SGI) score
- Quantitative Myasthenia Gravis (QMG) score

The calculations and analyses pertaining to each of the above variables are shown in Section 4.2.1.

4.1.2 Secondary and Exploratory Efficacy Variables

The secondary and exploratory efficacy variables are the following:

- Clinical Global Impression of Improvement (CGI-I) score (secondary)
- Triple Timed Up and Go (3TUG) result (exploratory)
- Subject most bothersome symptom question (exploratory)
- QMG limb domains (exploratory)

The calculations and analyses pertaining to each of the above variables are shown in Section 4.2.2.

4.2 Analysis of Efficacy Variables

4.2.1 Primary Efficacy Analysis

Primary efficacy analyses will be conducted on the FAS and PP populations, with the FAS population serving as the primary analysis set. For both primary efficacy variables, change from baseline (CFB) will be computed as the post-treatment result (Day 4) minus the Baseline result (Day 0). The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.

Summary statistics for the QMG Day 0 assessment, QMG Day 4 assessment, and the corresponding CFB will be presented by treatment. The analysis of CFB for Total QMG Score is a co-primary efficacy endpoint and analysis will be performed by fitting a fixed effects linear model to the data with CFB as the response. The model will include terms for treatment and QMG at Baseline. The following test comparing the least squares (LS) means will be conducted to evaluate the treatment effect:

$$H_{A,0}: \text{LSMeanQMG(A)} = \text{LSMeanQMG(P)}$$
$$\text{vs.}$$
$$H_{A,1}: \text{LSMeanQMG(A)} \neq \text{LSMeanQMG(P)},$$

where LSMeanQMG(A) is the QMG LS mean of the amifampridine treatment group and LSMeanQMG(P) is the QMG LS mean of the placebo treatment group.

The raw individual QMG scores (each individual QMG domain) for Day 0, Day 4, and CFB will be summarized by treatment and dose within treatment. Additional between-treatment comparisons for several of the individual QMG domains, and the sum of these domains, will be performed using a fixed effects linear model as an exploratory endpoint. No inferential procedures will be performed on the other QMG domains. See section 4.2.2 for details.

Summary statistics for SGI score and the corresponding CFB will be presented by treatment. The analysis of CFB for SGI is a co-primary efficacy endpoint and analysis will be performed by fitting a fixed effects linear model to the data with

CFB as the response. The model will include terms for treatment and SGI at Baseline. The following test comparing the LS means will be conducted to evaluate the treatment effect:

$$H_{B,0}: \text{LSMeanSGI(A)} = \text{LSMeanSGI(P)}$$
$$\text{vs.}$$
$$H_{B,1}: \text{LSMeanSGI(A)} \neq \text{LSMeanSGI(P)},$$

where LSMeanSGI(A) is the SGI LS mean of the amifampridine treatment group and LSMeanSGI(P) is the SGI LS mean of the placebo treatment group.

A sensitivity analysis of the co-primary endpoints will be conducted to evaluate the patterns of early treatment discontinuation. For each co-primary endpoint, a randomization test will be conducted to determine if the results from the primary analysis are supported. The randomization test is an alternative to a full permutation, and will evaluate the fixed effects model specified above using permutations of the treatment group assignments. If early discontinuations are not associated with treatment, then it is expected that the p-value resulting from the randomization test will yield the same statistical interpretation as the p-value resulting from the primary analysis.

A data listing of the primary efficacy data will be constructed as shown in Appendix B.

4.2.2 Secondary and Exploratory Efficacy Analyses

Secondary and exploratory efficacy analyses will be conducted on the FAS and PP populations, with the FAS population serving as the primary analysis set.

Clinical Global Impression of Improvement (CGI-I)
CGI-I scores will be summarized by treatment group using descriptive statistics. A Wilcoxon Rank Sum Test will be conducted to assess for treatment group differences.

Triple Timed Up and Go (3TUG)
The number and proportion of subjects with at least a 20% increase in average time for 3TUG (i.e., a success) at Day 4 relative to Day 0 will be presented by treatment. For those subjects that are unable to complete the Day 4 assessment due to disability related to their disease, a success will be assigned to the subject by the site at the time of testing. The proportion will be computed in two ways: 1) using all subjects in the analysis population with Day 0 data as the denominator, regardless of whether or not they completed the Day 4 assessment; and 2) using only those subjects in the analysis population who completed both the Day 0 and Day 4 assessments. For each approach in calculating the proportion, a two-sided Fisher's Exact Test will be conducted to

test for a significant difference in the success proportions between treatment groups.

Patient Most Bothersome Symptom Question

For the Patient Most Bothersome Symptom (PMBS) question, change from baseline (CFB) will be computed as the post-treatment response (Day 4) minus the Baseline response (Day 0). The post-treatment response will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment response may be obtained at an earlier time point.

Summary statistics for the PMBS Day 0 response, PBMS Day 4 response, and the corresponding CFB will be presented by treatment. The analysis of CFB for PMBS Score will be performed using Wilcoxon's Rank Sum Test.

QMG Limb Domains

Descriptive statistics for Day 0, Day 4, and CFB for the sum of the QMG scores for Right arm outstretched, Left arm outstretched, Right leg outstretched, and Left leg outstretched will be presented by treatment. As was described in section 4.2.1, summary statistics will be presented for each of these for individual domains. The CFB for each of these four individual domains, and for the sum of these four domains, will be performed by fitting a fixed effects linear model to the data with CFB as the response. The model will include terms for treatment and QMG score of the respective individual domain, or sum of these four domains, as applicable, at Baseline. The following test comparing the least squares (LS) means will be conducted to evaluate the treatment effect:

$$H_{C,0}: \text{LSMeanQMG(A)} = \text{LSMeanQMG(P)}$$
$$\text{vs.}$$
$$H_{C,1}: \text{LSMeanQMG(A)} \neq \text{LSMeanQMG(P)},$$

where LSMeanQMG(A) is the QMG LS mean of the amifampridine treatment group and LSMeanQMG(P) is the QMG LS mean of the placebo treatment group for each individual QMG domain described above, or sum of these 4 domains. A sensitivity analysis by a randomization test for the QMG limb domain analyses is not required.

All secondary and exploratory efficacy data will be listed as shown in Appendix B.

5.0 Analysis of Safety

The safety variables for this study are:

- Adverse events (AE)
- Vital signs (screening, Days 1-4)

- Physical examination (at screening only)
- Concomitant medications

Adverse Events
All AEs will be observed for each subject from enrollment until termination from the study. Prior to analysis, all AEs will be coded using MedDRA. Based on these coded terms, treatment emergent AEs (TEAEs) will be summarized using system organ class and preferred term by treatment and overall for all subjects in the safety population. This analysis will be repeated for serious TEAEs (TESAEs).

TEAEs will also be summarized by severity and relationship to IP. An overall summary table will provide the highest relationship and maximum severity observed per subject, as well as the counts of subjects with at least one TESAE.

All AEs will be listed, regardless of whether or not they were treatment emergent. AEs having an end date prior to signing the informed consent for this study will not be displayed in the AE data listings.

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.

Physical Exam
The physical exam data collected at screening will be listed.

Concomitant Medications
A table of the WHO-coded medications will be constructed by treatment group and overall with medications summarized by level 3 anatomical therapeutic chemical (ATC) term and Preferred Term . The number and percent of subjects on each drug will be summarized. A data listing for all concomitant medications will be provided.

6.0 Other Relevant Data Analyses/Summaries

6.1 Subject Completion

A table will be constructed with counts of screen failures and enrolled subjects. Of those enrolled, counts and percentages of the number of subjects withdrawing from the study before study completion and the number completing the study will be displayed. For those subjects that withdraw before completion of the study, counts and percentages of the reasons for withdrawal will be tabulated. The

table will include summary counts and percentages by treatment. A data listing of all subject completion and withdrawal data will also be constructed.

6.2 Study Drug Administration and Compliance

Duration of treatment administration will be computed per subject as:

Duration (in days) = (Date of last dose) – (Date of first dose) + 1

Duration will be summarized using descriptive statistics by treatment group.

Compliance will be computed per subject as:

Compliance = 100%*(Number consumed)/(Number prescribed),

where number prescribed is defined as the duration times the number of tablets to have been taken daily. Compliance will be summarized using descriptive statistics by treatment group.

6.3 Patient Data Profiles

A Patient Data Profile listing will be provided. It will contain demographic information, randomization information, all endpoint assessments and laboratory measurements. See Appendix B, Data Listing 17 for full details. Some variation in the appearance of this table is acceptable to accommodate unformatted SAS® output provided that all information is present.

7.0 List of Analysis Tables, Figures and Listings

Table No.	Table Title	Included in Final Tables	Shown in Appendix B
1	Subject Disposition	X	X
2	Demographics and Baseline Data Summary Statistics – Continuous Variables (Safety Population)	X	X
3	Demographics and Baseline Data Summary Statistics – Categorical Variables (Safety Population)	X	X
4	Summary of Study Drug Administration and Compliance (Safety Population)	X	X
5	QMG Total Score Summary Statistics by Time Point (FAS Population)	X	X
6	QMG Total Score Summary Statistics by Time Point (PP Population)	X	
7	QMG Total Score Summary Statistics by Time Point and Dose Group (FAS Population)	X	X
8	QMG Total Score Summary Statistics by Time Point and Dose Group (PP Population)	X	
9	QMG Item Scores Summary Statistics by Time Point (FAS Population)	X	X
10	QMG Item Scores Summary Statistics by Time Point (PP Population)	X	
11	QMG Item Scores Summary Statistics by Time Point and Dose Group (FAS Population)	X	X
12	QMG Item Scores Summary Statistics by Time Point and Dose Group (PP Population)	X	
13	QMG Total Score CFB Analysis (FAS Population)	X	X
14	QMG Total Score CFB Analysis (PP Population)	X	
15	QMG Item Scores CFB Analysis (FAS Population)	X	X
16	QMG Item Scores CFB Analysis (PP Population)	X	
17	SGI Score Summary Statistics by Time Point (FAS Population)	X	X
18	SGI Score Summary Statistics by Time Point (PP Population)	X	
19	SGI Score Summary Statistics by Time Point and Dose Group (FAS Population)	X	X
20	SGI Score Summary Statistics by Time Point and Dose Group (PP Population)	X	
21	SGI Score CFB (FAS Population)	X	X
22	SGI Score CFB Analysis (PP Population)	X	
23	QMG Total Score and SGI Score Sensitivity Analysis (FAS Population)	X	X
24	QMG Total Score and SGI Score Sensitivity Analysis (PP Population)	X	
25	CGI-I Scores Summary Statistics (FAS Population)	X	X
26	CGI-I Scores Summary Statistics (PP Population)	X	
27	CGI-I Scores Summary Statistics by Dose Group (FAS Population)	X	X
28	CGI-I Scores Summary Statistics by Dose Group (PP Population)	X	

Table No.	Table Title	Included in Final Tables	Shown in Appendix B
29	Number and Proportion of Subjects with ≥ 20% Increase in 3TUG Average Time (FAS Population)	X	X
30	Number and Proportion of Subjects with ≥ 20% Increase in 3TUG Average Time (PP Population)	X	
31	Number and Proportion of Subjects with ≥ 20% Increase in 3TUG Average Time by Dose Group (FAS Population)	X	X
32	Number and Proportion of Subjects with ≥ 20% Increase in 3TUG Average Time by Dose Group (PP Population)	X	
33	Summary of Patient Most Bothersome Symptom (FAS Population)	X	X
34	Summary of Patient Most Bothersome Symptom (PP Population)	X	
35	Summary of Patient Most Bothersome Symptom by Dose Group (FAS Population)	X	X
36	Summary of Patient Most Bothersome Symptom by Dose Group (PP Population)	X	
37	QMG Total Score Summary Statistics by Limb Domain and Time Point (FAS Population)	X	X
38	QMG Total Score Summary Statistics by Limb Domain and Time Point (PP Population)	X	
39	Limb Domain Specific QMG Total Score CFB Analysis (FAS Population)	X	X
40	Limb Domain Specific QMG Total Score CFB Analysis (PP Population)	X	
41	Number and Percent of Subjects with Treatment Emergent Adverse Events (Safety Population)	X	X
42	Number and Percent of Subjects with Treatment Emergent Adverse Events by Dose Level (Safety Population)	X	X
43	Summary of Treatment Emergent Adverse Events (Safety Population)	X	X
44	Number and Percent of Subjects with Serious Treatment Emergent Adverse Events (Safety Population)	X	X
45	Number and Percent of Subjects with Treatment Emergent Adverse Events by Relationship to Treatment (Safety Population)	X	X
46	Number and Percent of Subjects with Treatment Emergent Adverse Events by Severity Grade (Safety Population)	X	X
47	Vital Sign Parameters Summary Statistics (Safety Population)	X	X
48	Number and Percent of Subjects Taking Concomitant Medications by ATC Level 3 and Preferred Term (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	Protocol Deviations Data Listing	X	X
DL3	Demographics Data Listing	X	X
DL4	Subjects Excluded from FAS Population Data Listing	X	X
DL5	Subjects Excluded from PP Population Data Listing	X	X
DL6	Medical History Data Listing	X	X
DL7	Prior and Concomitant Medications Data Listing	X	X
DL8	Adverse Events Data Listing	X	X
DL9	Physical Exam Data Listing	X	X
DL10	Vital Signs Data Listing	X	X
DL11	Study Drug Administration Data Listing	X	X
DL12	SGI Data Listing	X	X
DL13	QMG Data Listing	X	X
DL14	CGI-I Data Listing	X	X
DL15	3TUG Data Listing	X	X
DL16	Patient Most Bothersome Symptom Data Listing	X	X
DL17	Patient Data Profile	X	X

8.0 References

Cassell, David. A Randomization-test Wrapper for SAS® PROCs. Paper 251-27, (2002) SUGI 27 Proceedings. http://www2.sas.com/proceedings/sugi27/p251-27.pdf, doi:2017-06-22.

Appendix A – Tables, Figures and Listing Specifications

Orientation

Tables, figures, and listings will be displayed in landscape.

Margins

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

Font

Courier New, 8 point.

Headers

The table number will be on the second line of the title area. 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.
- 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.

Computation Values for Study Dates

The date format to be used is dd-mmm-yyyy. Missing parts of dates are not shown (e.g., for a missing day value, the value displayed is in mmm-yyyy format). When date computations are necessary, the following table indicates the substitutions used in order to make those computations.

Scenario	Value Used for Computations
Start date – Missing month and day values	January 1 of the indicated year
Start date – Missing day values	The first day of the indicated month
Stop date – Missing month and day values	December 31 of the indicated year
Stop date – Missing day values	The last day of the indicated month

Appendix B – Table Shells

Table 1. Subject Disposition
Catalyst Pharmaceuticals, Inc. - LMS-003

		Amifampridine	Placebo	Overall
Screen Failures				xx
Enrolled		xx	xx	xx
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
Completed		xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
Withdrawn		xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
Reason for Withdrawal	Adverse Event	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Lost To Follow-Up	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Death	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Physician Decision	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Protocol Deviation	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Study Terminated by Sponsor	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Withdrawal by Subject	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Other	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)

The denominator for all percentages in the table is the number of enrolled subjects in the pertinent treatment/dose group and overall.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 1

Table 2. Demographics and Baseline Data Summary Statistics – Continuous Variables
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Variable	Treatment Group	Mean	Std Dev	n	Min	Max	Median
Age (years)	Amifampridine	xxx	xxx	xxx	xxx	xxx	xxx
	Low Dose (< 60 mg/day)	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (≥ 60 mg/day)	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	xxx	xxx	xxx	xxx	xxx	xxx
	Low Dose (< 60 mg/day)	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (≥ 60 mg/day)	xxx	xxx	xxx	xxx	xxx	xxx
	Overall	xxx	xxx	xxx	xxx	xxx	xxx
Baseline Weight (kg)	Amifampridine	xxx	xxx	xxx	xxx	xxx	xxx
	Low Dose (< 60 mg/day)	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (≥ 60 mg/day)	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	xxx	xxx	xxx	xxx	xxx	xxx
	Low Dose (< 60 mg/day)	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (≥ 60 mg/day)	xxx	xxx	xxx	xxx	xxx	xxx
	Overall	xxx	xxx	xxx	xxx	xxx	xxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 3

Table 3. Demographics and Baseline Data Summary Statistics – Categorical Variables
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Demographics Variable	Category	Amifampridine (N=xxx)	Placebo (N=xxx)	Overall (N=xxx)
Gender	Male	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%[a])	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Female	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
Ethnicity	Hispanic or Latino	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Not Hispanic or Latino	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Low Dose (< 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)
	High Dose (≥ 60 mg/day)	xx (xxx%)	xx (xxx%)	xx (xxx%)

[a] Denominator for treatment/class/dose percentages is the treatment/class/dose level total.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 3

Table 4. Summary of Study Drug Administration and Compliance
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

	Statistic	Amifampridine (N=xxx)	Placebo (N=xxx)
Duration (days)	n	xxx	xxx
	Mean (SD)	xxx (xxx)	xxx (xxx)
	Median	xxx	xxx
	Minimum, Maximum	xxx, xxx	xxx, xxx
Compliance (%)	n	xxx	xxx
	Mean (SD)	xxx (xxx)	xxx (xxx)
	Median	xxx	xxx
	Minimum, Maximum	xxx, xxx	xxx, xxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 11

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Table 5. QMG Total Score Summary Statistics by Time Point
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Treatment	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.
[b] RAW = observed data entered in the database; CFB = change from baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 14

Table format will be repeated for the PP Population.

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Table 7. QMG Total Score Summary Statistics by Time Point and Dose Group
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Treatment	Dose Group	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Amifampridine									
	Low Dose (< 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (≥ 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo									
	Low Dose (< 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (≥ 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.
[b] RAW = observed data entered in the database; CFB = change from baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 14

Table format will be repeated for the PP Population.

Table 9. QMG Item Scores Summary Statistics by Time Point
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Treatment	QMG Item[a]	Time Point[b]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Amifampridine	xxxxxxxxxx	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo	xxxxxxxxxx	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] Table excludes limb domain items which are summarized on separate tables.
[b] The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.
[b] RAW = observed data entered in the database; CFB = change from baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 14

Table format will be repeated for the PP Population.

Table 11. QMG Item Scores Summary Statistics by Time Point and Dose Group
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

QMG Parameter = xxxxxxxxxxxxxx

Treatment	Dose Group	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Amifampridine									
	Low Dose (< 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (> 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo									
	Low Dose (< 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (> 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

Table repeats per QMG Parameter

[a] The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.
[b] RAW = observed data entered in the database; CFB = change from baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 14

Table format will be repeated for the PP Population.

Table 13. QMG Total Score CFB Analysis
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Statistic[a]	Amifampridine	Placebo
n	xxx	xxx
Least Squares (LS) Mean of Change from Baseline (CFB)	xxx	xxx
Between-Treatment Difference in LS Means	xxx	
95% CI for Between-Treatment Difference in LS Means	(xxx, xxx)	
P-value for Between-Treatment Difference in LS Means	xxx	

[a] CFB for QMG total score was modeled as the response, with fixed effects terms for treatment and QMG at Baseline.
Table Creation Date: (DD-MMM-YYYY)
xxxxxxx.sas
Source Listing: Data Listing 14

Table format will be repeated for the PP Population.

Table 15. QMG Item Scores CFB Analysis
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

QMG Item	Statistic[a]	Amifampridine	Placebo
xxxxxxxxx	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline (CFB)	xxx	xxx
	Between-Treatment Difference in LS Means	xxx	
	95% CI for Between-Treatment Difference in LS Means	(xxx, xxx)	
	P-value for Between-Treatment Difference in LS Means	xxx	

[a] CFB for QMG item score was modeled as the response, with fixed effects terms for treatment and QMG at Baseline. A separate model was run for each QMG item.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 14

Table format will be repeated for the PP Population.

Table 17. SGI Score Summary Statistics by Time Point
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Treatment	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.
[b] RAW = observed data entered in the database; CFB = change from baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 13

Table format will be repeated for the PP Population.

Table 19. SGI Score Summary Statistics by Time Point and Dose Group
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Treatment	Dose Group	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Amifampridine									
	Low Dose (< 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (> 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo									
	Low Dose (< 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (> 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.
[b] RAW = observed data entered in the database; CFB = change from baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 13

Table format will be repeated for the PP Population.

Table 21. SGI Score CFB Analysis
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Statistic[a]	Amifampridine	Placebo
n	xxx	xxx
Least Squares (LS) Mean of Change from Baseline (CFB)	xxx	xxx
Between-Treatment Difference in LS Means	xxx	
95% CI for Between-Treatment Difference in LS Means	(xxx, xxx)	
P-value for Between-Treatment Difference in LS Means	xxx	

[a] CFB for SGI score was modeled as the response, with fixed effects terms for treatment and QMG at Baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 13

Table format will be repeated for the PP Population.

Table 23. QMG Total Score and SGI Score Sensitivity Analysis
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Statistic[a]	QMG Total Score	SGI Score
P-value for Between-Treatment Difference in LS Means	xxx	xxx

[a] P-value based on conducting a randomization test by running the fixed effects linear model analysis on permuted treatment assignments. For each of the xxxx permutations, CFB was modeled as the response for each endpoint, with fixed effects terms for treatment and score at Baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 13, Data Listing 14

Table format will be repeated for the PP Population.

Table 25. CGI-I Scores Summary Statistics
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Treatment	Time Point	Mean	Std Dev	n	Min	Max	Median	P-value[a]
Amifampridine	Day 4	xxx	xxx	xxx	xxx	xxx	xxx	xxx
Placebo	Day 4	xxx	xxx	xxx	xxx	xxx	xxx	

[a] P-value based on the Wilcoxon Rank Sum Test for treatment differences.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 15

Table format will be repeated for the PP Population.

Table 27. CGI-I Scores Summary Statistics by Dose Group
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Treatment	Dose Group	Time Point	Data Type	Mean	Std Dev	n	Min	Max	Median
Amifampridine									
	Low Dose (< 60mg/day)	Day 4	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (≥ 60mg/day)	Day 4	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 4	RAW	xxx	xxx	xxx	xxx	xxx	xxx
Placebo									
	Low Dose (< 60mg/day)	Day 4	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (≥ 60mg/day)	Day 4	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 4	RAW	xxx	xxx	xxx	xxx	xxx	xxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 15

Table format will be repeated for the PP Population.

Table 29. Number and Proportion of Subjects with ≥ 20% Increase in 3TUG Average Time
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Calculation	Amifampridine (N=xxx)	Placebo (N=xxx)	P-value[a]
Denominator Based on Subjects Who Completed Day 0 3TUG	xxx/xxx (xxx)	xxx/xxx (xxx)	xxx
Denominator Based on Subjects Who Completed Day 0 and Day 4 3TUG	xxx/xxx (xxx)	xxx/xxx (xxx)	xxx

[a] P-value based on a two-sided Fisher's Exact Test of difference in treatment proportions.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 16

Table format will be repeated for the PP Population.

Table 31. Number and Proportion of Subjects with ≥ 20% Increase in 3TUG Average Time by Dose Group
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

	Low Dose(< 60mg/day)		High Dose(≥ 60mg/day)	
Calculation	Amifampridine (N=xxx)	Placebo (N=xxx)	Amifampridine (N=xxx)	Placebo (N=xxx)
Denominator Based on Subjects Who Completed Day 0 3TUG	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Denominator Based on Subjects Who Completed Day 0 and Day 4 3TUG	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 16

Table format will be repeated for the PP Population.

Table 33. Summary of Patient Most Bothersome Symptom
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Treatment	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median	P-value[c]
Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx	
	Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx	
		CFB	xxx	xxx	xxx	xxx	xxx	xxx	xxxx
Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx	
	Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx	
		CFB	xxx	xxx	xxx	xxx	xxx	xxx	

[a] The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.
[b] RAW = observed data entered in the database; CFB = change from baseline.
[c] P-value based on the Wilcoxon Rank Sum Test for treatment differences in CFB results.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Table format will be repeated for the PP Population.

Table 35. Summary of Patient Most Bothersome Symptom by Dose Group
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Treatment	Dose Group	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Amifampridine									
	Low Dose (< 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (> 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo									
	Low Dose (< 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	High Dose (> 60mg/day)	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	All Doses	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.
[b] RAW = observed data entered in the database; CFB = change from baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Table format will be repeated for the PP Population.

Table 37. QMG Total Score Summary Statistics by Limb Domain and Time Point
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Limb Domain	Treatment	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Left Arm	Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Right Arm	Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Left Leg	Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

Table 37 (cont.). QMG Total Score Summary Statistics by Limb Domain and Time Point
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Limb Domain	Treatment	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
Right Leg	Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Total of Region Scores	Amifampridine	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	Day 0 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Post-Baseline	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] The post-treatment result will be the result obtained on Day 4, unless the subject discontinued treatment early, in which case the post-treatment result may be obtained at an earlier time point.
[b] RAW = observed data entered in the database; CFB = change from baseline.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 14

Table format will be repeated for the PP Population.

Table 39. Limb Domain Specific QMG Total Score CFB Analysis
Catalyst Pharmaceuticals, Inc. - LMS-003
FAS Population (N=xxx)

Limb Domain	Statistic[a]	Amifampridine	Placebo
Left Arm Outstretched	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline (CFB)	xxx	xxx
	Between-Treatment Difference in LS Means	xxx	
	95% CI for Between-Treatment Difference in LS Means	(xxx, xxx)	
	P-value for Between-Treatment Difference in LS Means	xxx	
Right Arm Outstretched	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline (CFB)	xxx	xxx
	Between-Treatment Difference in LS Means	xxx	
	95% CI for Between-Treatment Difference in LS Means	(xxx, xxx)	
	P-value for Between-Treatment Difference in LS Means	xxx	
Left Leg Outstretched	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline (CFB)	xxx	xxx
	Between-Treatment Difference in LS Means	xxx	
	95% CI for Between-Treatment Difference in LS Means	(xxx, xxx)	
	P-value for Between-Treatment Difference in LS Means	xxx	
Right Leg Outstretched	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline (CFB)	xxx	xxx
	Between-Treatment Difference in LS Means	xxx	
	95% CI for Between-Treatment Difference in LS Means	(xxx, xxx)	
	P-value for Between-Treatment Difference in LS Means	xxx	
Total of Regions[b]	n	xxx	xxx
	Least Squares (LS) Mean of Change from Baseline (CFB)	xxx	xxx
	Between-Treatment Difference in LS Means	xxx	
	95% CI for Between-Treatment Difference in LS Means	(xxx, xxx)	
	P-value for Between-Treatment Difference in LS Means	xxx	

[a] CFB for QMG score was modeled as the response, with fixed effects terms for treatment and QMG at Baseline.
[b] Total is the CFB of the sum of the four regions listed above.
Table Creation Date: (DD-MMM-YYYY)
xxxxxxx.sas
Source Listing: Data Listing 14

Table format will be repeated for the PP Population.

Table 41. Number and Percent of Subjects with Treatment Emergent Adverse Events
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Adverse Event Category[a]:	Amifampridine (N=xxx)	Placebo (N=xxx)	Overall (N=xxx)
Total Number of Treatment Emergent Adverse Events (TEAEs)	xxx	xxx	xxx
Subjects with at Least One 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.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 42. Number and Percent of Subjects with Treatment Emergent Adverse Events by Dose Level
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

	Amifampridine			Placebo		
Adverse Event Category[a]:	Low Dose (N=xxx)	High Dose (N=xxx)	All Doses (N=xxx)	Low Dose (N=xxx)	High Dose (N=xxx)	All Doses (N=xxx)
Total Number of Treatment Emergent Adverse Events (TEAEs)	xxx	xxx	xxx	xxx	xxx	xxx
Subjects with at Least One TEAE	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] Adverse events coded with MedDRA Coding Dictionary Version XXX.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 43. Summary of Treatment Emergent Adverse Events
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxxx)

	Amifampridine (N=xxx)	Placebo (N=xxx)	Overall (N=xxx)
Subjects with at Least One Treatment Emergent Adverse Event (TEAE)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Maximum TEAE Severity Grade			
Mild (Grade 1)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Moderate (Grade 2)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Severe (Grade 3)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Life-threatening (Grade 4)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Death (Grade 5)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Highest Relationship of TEAE to Treatment			
Not Related	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Possibly	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Probably	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Subjects with at Least One Serious TEAE	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 44. Number and Percent of Subjects with Serious Treatment Emergent Adverse Events
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Adverse Event Category[a]:	Amifampridine (N=xxx)	Placebo (N=xxx)	Overall (N=xxx)
Total Number of Serious Treatment Emergent Adverse Events (TESAEs)	xxx	xxx	xxx
Subjects with at Least One TESAE	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.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 45. Number and Percent of Subjects with Treatment Emergent Adverse Events
by Relationship to Treatment
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

	Amifampridine (N=xxx)			Placebo (N=xxx)		
Adverse Event Category[a]:	Not Related	Possibly	Probably	Not Related	Possibly	Probably
Total Number of Treatment Emergent Adverse Events (TEAEs)	xxx	xxx	xxx	xxx	xxx	xxx
Subjects with at Least One TEAE	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
System Organ Class 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] Adverse events coded with MedDRA Coding Dictionary Version XXX.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 46. Number and Percent of Subjects with Treatment Emergent Adverse Events by Severity Grade
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Part 1 of 2

	Amifampridine (N=xxx)				
Adverse Event Category[a]:	Grade 1	Grade 2	Grade 3	Grade 4	Grade 5
Total Number of Treatment Emergent Adverse Events (TEAEs)	xxx	xxx	xxx	xxx	xxx
Subjects with at Least One 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] Adverse events coded with MedDRA Coding Dictionary Version XXX.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 46 (cont.). Number and Percent of Subjects with Treatment Emergent Adverse Events by Severity Grade
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Part 2 of 2

	Placebo (N=xxx)				
Adverse Event Category[a]:	Grade 1	Grade 2	Grade 3	Grade 4	Grade 5
Total Number of Treatment Emergent Adverse Events (TEAEs)	xxx	xxx	xxx	xxx	xxx
Subjects with at Least One 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] Adverse events coded with MedDRA Coding Dictionary Version XXX.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 47. Vital Signs Summary Statistics
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Vital Sign Parameter (units)	Visit	Data Type[a]	Mean	Std Dev	n	Min	Max	Median
Amifampridine	xxxxxxxxxx (xxx)	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day X	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
Placebo	xxxxxxxxxx (xxx)	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day X	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
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 10

Table 48. Number and Percent of Subjects Taking Concomitant Medications by ATC Level 3 and Preferred Term
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Concomitant Medication Category[a,b]	Amifampridine (N=xxx)	Placebo (N=xxx)	Overall (N=xxx)
ATC Level 3 Term	xxxx (xxxx%)	xxxx (xxxx%)	xxxx (xxxx%)
WHO Preferred Term	xxxx (xxxx%)	xxxx (xxxx%)	xxxx (xxxx%)
WHO Preferred Term	xxxx (xxxx%)	xxxx (xxxx%)	xxxx (xxxx%)
ATC Level 3 Term	xxxx (xxxx%)	xxxx (xxxx%)	xxxx (xxxx%)
WHO Preferred Term	xxxx (xxxx%)	xxxx (xxxx%)	xxxx (xxxx%)
WHO Preferred Term	xxxx (xxxx%)	xxxx (xxxx%)	xxxx (xxxx%)

[a] Concomitant medications coded with WHO Coding Dictionary xxxxxxxxxxxx.
[b] Concomitant medication categories will include anatomical therapeutic chemical (ATC) level 3 term followed by preferred term.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 7

Data Listing 1. Subject Disposition Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003

Treatment	Subject No.	Disposition Status	Date of Disposition	Withdrawal Reason
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 2. Protocol Deviations Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Date of Deviation	Deviation Description	Deviation Major or Minor
xxxxxx	xxxx	xxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxxxxx
xxxxxx	xxxx	xxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxxxxx
xxxxxx	xxxx	xxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxxxxx
xxxxxx	xxxx	xxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxxxxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 3. Demographics Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

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

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 4. Subjects Excluded from FAS Population Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Reason for Exclusion
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 5. Subjects Excluded from PP Population Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
All Enrolled Subjects (N=xxx)

Treatment	Subject No.	Reason for Exclusion
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 6. Medical History Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

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

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

Data Listing 7. Prior and Concomitant Medications Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	WHO Preferred Term[a]/ Verbatim Drug Name/ Indication/ ATC Level 3 Term	Start Date	Stop Date	Route	Ongoing?
xxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxx	xxxxxxx	xxxxx	xxxxx
xxxxxx	xxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxxxxxxxxxxxxxxx	xxxxxxx	xxxxxxx	xxxxx	xxxxx

[a] Concomitant medications coded with WHO Coding Dictionary xxxxxxxxx
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

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Data Listing 8. Adverse Events Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Dose	Subject No.	Start Date and Time/ End Date and Time	Treatment Start Date	MedDRA System Organ Class[a]/ MedDRA Preferred Term/ CRF Verbatim Term	Severity Grade	Relation to Treatment	Serious?	Outcome
xxxxxx	xxxxx	xxxxxxxxx	xxxxxxx xxxxxxx/ xxxxxxx xxxxxxx	xxxxxxx xxxxxxx	xxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxx	xxxxxxxx	xxxxxxxx	xxx	xxxxxxxx
xxxxxx	xxxxx	xxxxxxxxx	xxxxxxx xxxxxxx/ xxxxxxx xxxxxxx	xxxxxxx xxxxxxx	xxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxx	xxxxxxxx	xxxxxxxx	xxx	xxxxxxxx

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

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Data Listing 9. Physical Exam Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Visit	Date Conducted	Body System	Result	Abnormality
xxxxxx	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

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 10. Vital Signs Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Visit	Date	Time	Temp. (°F)	Systolic Blood Pressure (mmHg)	Diastolic Blood Pressure (mmHg)	Heart Rate (bpm)
xxxxxx	xxxx	xxxxxxx	xxxxxxx	xxxxx	xxx	xxx	xxx	xxx
				xxxxx	xxx	xxx	xxx	xxx
				xxxxx	xxx	xxx	xxx	xxx
xxxxxx	xxxx	xxxxxxx	xxxxxxx	xxxxx	xxx	xxx	xxx	xxx
				xxxxx	xxx	xxx	xxx	xxx
				xxxxx	xxx	xxx	xxx	xxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 11. Study Drug Administration Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Treatment Start Date	Treatment End Date	Treatment Duration (Days)	Tablets Consumed	Dose Group (Low or High)[a]	Tablets Prescribed[b]	Compliance (%)[c]
xxxxxx	xxxx	xxxxxx	xxxxxx	xxx	xxx	xxx	xxx	xxx
xxxxxx	xxxx	xxxxxx	xxxxxx	xxx	xxx	xxx	xxx	xxx

[a] Low dose group is < 60mg/day and high dose group is ≥ 60mg/day, based on prescribed tablets.
[b] Number of tablets prescribed is computed as the duration times the number of tablets to have been taken daily.
[c] Compliance is computed as 100%*(number of tablets consumed)/(number of tables prescribed).
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 12. SGI Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Visit	Impression of Effects of Study Medication During Preceding 3 Days on Physical Well Being[a]
xxxxxx	xxxx	xxxxxxxx	xxxx
xxxxxx	xxxx	xxxxxxxx	xxxx

[a] 1=Terrible; 2=Mostly Dissatisfied; 3=Mixed; 4=Partially Satisfied; 5=Mostly Satisfied; 6=Pleased; 7=Delighted.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 13. QMG Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Visit	Item	Score
xxxxxx	xxxx	xxxxxxxx	Double Vision (Lateral Gaze) Sec.	xxxx
			Ptosis (Upward Gaze) Sec.	xxxx
			Facial Muscles	xxxx
			Swallowing 4oz. Water (1/2 cup)	xxxx
			Speech Following Counting Aloud From 1-50 (Onset of Dysarthria)	xxxx
			Right Arm Outstretched (90°, sitting) Sec.	xxxx
			Left Arm Outstretched (90°, sitting) Sec.	xxxx
			Forced Vital Capacity	xxxx
			Right Hand Grip (kg)	xxxx
			Left Hand Grip (kg)	xxxx
			Head, Lifted (45%, supine) Sec.	xxxx
			Right Leg Outstretched (45-50%, supine) Sec.	xxxx
			Left Leg Outstretched (45-50%, supine) Sec.	xxxx
			TOTAL	xxxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 14. CGI-I Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Visit	How Much Has Subject's Condition Changed Since Day 0?[a]
xxxxxx	xxxx	xxxxxxxx	xxxx
xxxxxx	xxxx	xxxxxxxx	xxxx

[a] 1=Very Much Improved; 2=Much Improved; 3=Minimally Improved; 4=No Change; 5=Minimally Worse; 6=Much Worse; 7=Very Much Worse.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 15. 3TUG Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Visit	Time (Sec)	% Increase from Baseline
xxxxxx	xxxx	Day 0 (Baseline)	xxxx	
xxxxxx	xxxx	xxxxxxxx	xxxx	xxxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 16. Patient Most Bothersome Symptom Data Listing
Catalyst Pharmaceuticals, Inc. - LMS-003
Safety Population (N=xxx)

Treatment	Subject No.	Visit	Rating of Most Bothersome Symptom Before Treatment[a]	Rating of Most Bothersome Symptom During the Last 24 Hours After Treatment with Study Medication[b]
xxxxxx	xxxx	xxxxxxxx	xxxx	xxxx
xxxxxx	xxxx	xxxxxxxx	xxxx	xxxx

[a] 0=Not At All Bothersome; 1=Bothered Me a Little; 2=Bothered Me Some; 3=Bothered Me a Lot.
[a] 0=Not At All Bothersome; 1=Bothers Me a Little; 2=Bothers Me Some; 3=Bothers Me a Lot.
Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

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Data Listing 17 – Subject Data Profile
Catalyst Pharmaceuticals, Inc.
Safety Population (N=xxx)

Study Number: LMS-003 Site: xxxxxxxxx Subject ID: xxxxx

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

Screening Weight (kg): xxxx

Endpoint Measurements

Subject General Impression:

Visit	Date	Score	CFB
Baseline	xx-xxx-xxxx	xxxxxx	--
Day 4	xx-xxx-xxxx	xxxxxx	xxxxxx

Quantitative Myasthenia Gravis Scores

Visit	Date	Item	Score	CFB
Baseline	xx-xxx-xxxxx	Double vision	xxxxx	--
		Ptosis	xxxxx	--
		Facial Muscles	xxxxx	--
		Swallowing	xxxxx	--
		Speech following counting from 1-50	xxxxx	--
		Right arm outstretched	xxxxx	--
		Left arm outstretched	xxxxx	--
		Forced vital capacity	xxxxx	--
		Right hand grip	xxxxx	--
		Left hand grip	xxxxx	--
		Head, lifted	xxxxx	--
		Right leg outstretched	xxxxx	--
		Left leg outstretched	xxxxx	--
		Limb total	xxxxx	--
		Total Score	xxxxx	--
Day x	xx-xxx-xxxx	Double vision	xxxxx	xxxxx
		Ptosis	xxxxx	xxxxx
		Facial Muscles	xxxxx	xxxxx
		Swallowing	xxxxx	xxxxx
		Speech following counting from 1-50	xxxxx	xxxxx
		Right arm outstretched	xxxxx	xxxxx
		Left arm outstretched	xxxxx	xxxxx
		Forced vital capacity	xxxxx	xxxxx
		Right hand grip	xxxxx	xxxxx
		Left hand grip	xxxxx	xxxxx
		Head, lifted	xxxxx	xxxxx
		Right leg outstretched	xxxxx	xxxxx
		Left leg outstretched	xxxxx	xxxxx
		Limb total	xxxxx	xxxxx
		Total Score	xxxxx	xxxxx

Clinical Global Impression xxxxxxxx

Triple Timed Up and Go (3TUG) Scores

Time	Date	Score	CFB
Baseline	xx-xxx-xxxx	xxxx	--
Day xxx	xx-xxx-xxxx	xxxx	xxxx

Patient Most Bothersome Symptom

Time	Date	Score	CFB
Baseline	xx-xxx-xxxx	xxxx	--
Day xxx	xx-xxx-xxxx	xxxx	xxxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

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Data Listing 17 – Subject Data Profile
Catalyst Pharmaceuticals, Inc.
Safety Population (N=xxx)

Study Number: LMS-003 Site: xxxxxxxxx Subject ID: xxxxx

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	

Vital Signs

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

Adverse Events[a]

Event (Preferred Term)	Date	System Organ Class	Severity	Treatment Related?
xxxxxxxxxxx	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	xxxxxxxxxxx
xxxxxxxxxxx	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	xxxxxxxxxxx
xxxxxxxxxxx	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	xxxxxxxxxxx
xxxxxxxxxxx	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxxxx	xxxx	xxxxxxxxxxx

Prior and Concomitant Medications[b]

Medication (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

[a] Adverse events coded with MedDRA Coding Dictionary Version xxx.
[b] Concomitant medications coded with WHO Coding Dictionary xxxxxxxxx

Table Creation Date: (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Table repeats per Subject beginning on a new page.