Statistical Analysis Plan (SAP)

Study No. MSK-002

A Randomized, Placebo-Controlled, Parallel Group Study to Evaluate the Effect of Amifampridine Phosphate in Subjects with MuSK Antibody Positive Myasthenia Gravis, and a Sample of AChR Antibody Positive Myasthenia Gravis Subjects

Version 1.0

January 27, 2020

Prepared for
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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

This version (Version 1.0) is the first version of this Statistical Analysis Plan. However, on July 12, 2017, a draft version of this document was submitted to the FDA in sequence 0109 of IND 106,263 as part of a submission requesting review of a Special Protocol Assessment. The protocol (and indirectly the July 12, 2017 draft version of this SAP) was approved by the FDA on August 23, 2017. Subsequent to that, Catalyst evaluated the suitability of the proposed statistical methods and proposed revisions to them. Those proposed revisions are included in this document.

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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 MuSK-MG and a sample of AChR-MG subjects. The objectives of this Phase 3 study are as follows:

- Primary:
  - To characterize the overall safety and tolerability of amifampridine phosphate compared with placebo in subjects with MuSK-MG; and,
  - To assess the clinical efficacy of amifampridine phosphate compared with placebo in subjects with MuSK-MG, based on change of the Myasthenia Gravis-Activities of Daily Living (MG-ADL) score from Day 0 (baseline).
- Secondary:
  - To assess the clinical efficacy of amifampridine phosphate compared with placebo in subjects with MuSK-MG, based on change of the Quantitative Myasthenia Gravis (QMG) score from Day 0 (baseline); and,
  - To assess the safety and qualitative change in MG-ADL and QMG efficacy assessments of amifampridine phosphate compared with placebo in a sample of subjects with AChR-MG.

1.1 Design and Treatment

Subjects will be randomized on Day 0 to either treatment group in a 1:1 ratio. The 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 entering the randomization period of this trial) or placebo will be dispensed by the site pharmacist, according to the randomization schedule, to begin dosing after all Day 0 assessments are completed and continued for 11 days.

The amifampridine phosphate dose is 30 mg to 80 mg total daily dose (expressed in free base 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 1 to 14 days for screening, followed by a roughly 4-week run-in period. The run-in period will consist of open-label amifampridine dose adjustments every 3-4 days to achieve at least a 2 point improvement in MG-ADL (an inclusion requirement), observed during a subject's visit to the clinic during week 3 of run-in, and dose titration to

the limit of tolerability and no higher than a maximum of 80 mg per day. The subject must be on a constant dose for a minimum of 7 days to be eligible for randomization. Eligible subjects will be randomly assigned to either amifampridine or placebo tablets in a blinded fashion for treatment over 11 days (Day 0 through Day 10) provided all inclusion and exclusion criteria are met. The treatment period will commence on the remainder of Day 0 and continue to Day 10. The following assessments will be performed at the beginning and end of the 11-day treatment period:

- Vital signs;
- Urine sample collection on Day 10 for evaluation of IP compliance by testing for amifampridine and its metabolite in the urine;
- Complete Physical Examination;
- 12-lead Electrocardiogram (ECG);
- Myasthenia Gravis specific Activities of Daily Living (MG-ADL);
- Quantitative Myasthenia Gravis (QMG);
- Record concomitant medications;
- Monitor for adverse events (throughout the run-in and treatment periods).

1.3 Sample Size

The study is powered with respect to the primary endpoint of change in MG-ADL score from baseline. The sample size of the study has approximately 60% power to detect a 2 point difference, a minimally clinically significant difference, in the mean change from baseline between amifampridine and placebo treatment groups with a total sample size of n=60, assuming a standard deviation of the MG-ADL scores of 3.5. The 60 subjects do not include the 10 AChR-MG subjects. AChR-MG subjects are not expected to be respond robustly and positively and therefore no attempt will be made to adequately power the study for this group of study subjects.

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 descriptive statistics for continuous data are: n (number of non-missing observations), mean, median, standard deviation, minimum, and maximum. A frequency count and percentage will be used to summarize the 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.

2.2 Analysis Populations

The analysis populations are defined as follows:

Safety: The safety population will consist of all subjects who are enrolled in the study and have received at least one dose of study drug. (Subjects who begin the run-in period belong to the Safety Population whether they are randomized to a treatment or not.)

Full Analysis Set, Intent to Treat Population [FAS (ITT)]: This population consists of all randomized subjects who receive at least 1 dose of IP (amifampridine or placebo post randomization) and have at least one post-treatment efficacy assessment. Subjects will be compared for efficacy according to the treatment to which they were randomized, regardless of the treatment actually received.

Per Protocol (PP): This population is a subset of the FAS (ITT) 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 the validity of the efficacy analysis, and
- Subjects who took at least 80% of the required treatment doses.

Subjects who discontinue with no post-randomization data (no Day 0 and no Day 10 data) will be excluded from all efficacy analyses but will be included in the safety analyses. Decisions on exclusion from the FAS (ITT) and PP Populations will be finalized before unblinding prior to database lock.

The FAS (ITT) population will be the primary data set for all effectiveness analyses. The Safety population will be used to analyze all safety variables and baseline characteristics. The PP population will be used for selected effectiveness analyses.

2.2.1 Subgroup Definitions

Subgroup analyses for efficacy will be performed independently on the MuSK-MG and AChR-MG groups. No pooled analyses are planned.

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 10 due to treatment related disability ("Rescue"), the observations collected at the time of rescue will be analyzed with the other Day 10 observations. Evaluations obtained at the time of discontinuation will be included in the FAS (ITT) and PP analyses, as applicable.

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 (sub-grouping) 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 height and 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

The primary and secondary efficacy analyses will be conducted on the FAS (ITT) and PP populations, with the FAS (ITT) population serving as the primary efficacy analysis set for the primary and secondary efficacy variables. For the efficacy variables, change from baseline (CFB) will be computed as the post-treatment result (usually Day 10) minus the Baseline result (Day 0). The post-treatment result for subjects who discontinued treatment early is the CFB computed using the primary variable obtained at the time of dropout.

The analysis of efficacy will employ a closed testing procedure. Statistical significance in terms of the family-wise error rate for secondary endpoints will be

controlled at an alpha level of 0.05. Control will be accomplished based on a hierarchical closed testing procedure[1,2]. A secondary endpoint will be declared to have a statistically significant treatment effect if it and all previous endpoints in the hierarchy (including the primary) have reached statistical significance. The procedure stops at the first test that does not produce a two-sided p-value less than 0.05. The hierarchy of endpoint testing is MG-ADL (Primary), QMG (first secondary), MG-ADL Binary Response (second secondary), and QMG Binary Response (third secondary).

4.1 Description of Efficacy Variables

4.1.1 Primary Efficacy Variables

The primary efficacy variable for the study is the change in MG-ADL score from Day 0 (baseline) to Day 10 for MuSK-MG subjects treated with amifampridine and placebo.

The calculations and analyses are shown in Section 4.2.1.

4.1.2 Secondary Efficacy Variables

The secondary and exploratory efficacy variables are the following:

- The change in QMG score from Day 0 (baseline) to Day 10 for MuSK-MG subjects treated with amifampridine and placebo.
- A binary indicator (0 or 1) of response defined as a change of 2 or more in MG-ADL score from baseline to Day 10 for MuSK-MG subjects treated with amifampridine and placebo.
- A binary indicator (0 or 1) of response defined as a change of 3 or more in QMG score from baseline to Day 10 for MuSK-MG subjects treated with amifampridine and placebo.

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

4.1.3 Exploratory Analysis of the Response of AChR-MG Subjects to Treatment

As described in Section 1.3, AChR-MG subjects are not expected to respond robustly and positively to the treatment with amifampridine. Those subjects showing an improvement of 2, or more, in MG-ADL from treatment with amifampridine during run-in will be enrolled, randomized, and qualitatively evaluated for the change from Day 0 (baseline) in the MG-ADL score and QMG. The endpoints for this evaluation are:

- Summary statistics by treatment for the MG-ADL score for Day 0, Day 10, and change from Day 0 (baseline).
- Summary statistics by treatment for the QMG score for Day 0, Day 10, and change from Day 0 (baseline).

4.2 Analysis of Efficacy Variables

4.2.1 Primary Efficacy Analysis

Summary statistics for the MG-ADL Day 0 assessment, MG-ADL Day 10 assessment, and the corresponding change from baseline for MG-ADL (CFB-MGADL) will be presented by treatment. The mean MG-ADL Total Score by time will be plotted by treatment and MG type.

The primary analysis of CFB-MGADL 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 MG-ADL at Baseline. The test comparing the least squares (LS) means will be conducted to evaluate the treatment effect:

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

where LSMeanCFB-MGADL(A) is the MG-ADL change from baseline LS mean of the amifampridine treatment group and LSMeanCFB-MGADL(P) is the MG-ADL change from baseline LS mean of the placebo treatment group.

A sensitivity analysis of the results of the test of the primary endpoint (MG-ADL CFB) score will be conducted using a nonparametric randomization test. The randomization test will repeat the fixed effects model analysis specified above after permutations of the treatment group assignments have been randomly performed. The procedure will be repeated a minimum of 1,000 times. If the observed test statistic is found to be in either the upper or lower 0.025 tail of the randomization p-value distribution, the null hypothesis of no treatment difference is rejected. A histogram of the p-values resulting from the 1000 permutation ANOVAs will be displayed together with the location of the p-value derived from the observed test statistic.

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

4.2.2 Secondary Efficacy Analysis

Summary statistics for the Quantitative Myasthenia Gravis (QMG) Day 0 assessment, QMG Day 10 assessment, and the corresponding change from baseline (CFB-QMG) will be presented by treatment.

The secondary analysis of CFB-QMG will be performed by fitting a fixed effects linear model to the data with CFB-QMG as the response. The model will include terms for treatment and QMG at Baseline. The test comparing the least squares (LS) means will be conducted to evaluate the treatment effect:

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

where LSMeanCFB-QMG(A) is the QMG change from baseline LS mean of the amifampridine treatment group and LSMeanCFB-QMG(P) is the QMG change from baseline LS mean of the placebo treatment group.

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

MG-ADL Binary Response
The number and proportion of MuSK-MG subjects with at least a 2 point change in MG-ADL score at Day 10 relative to Day 0 will be presented by treatment. The proportion for each treatment group will be computed with the number of subjects at Day 0 for each treatment group as the denominator, regardless of whether or not they completed the Day 10 assessment, and the number of subjects with at least a 2 point change in MG-ADL score at Day 10 as the numerator. A logistic regression model will be fitted to the binary variable indicating at least 2 points change in MG-ADL score as a function of the treatment assignment (Amifampridine or placebo) and the baseline MG-ADL score. The point estimate and 95% confidence interval on the point estimate will be displayed.

QMG Binary Response
The number and proportion of MuSK-MG subjects with at least a 3-point change in QMG score at Day 10 relative to Day 0 will be presented by treatment. The proportion for each treatment group will be computed with the number of subjects at Day 0 for each treatment group as the denominator, regardless of whether or not they completed the Day 10 assessment, and the number of subjects with at least a 3 point change in QMG score at Day 10 as the numerator. A logistic regression model will be fitted to the binary variable indicating at least 3 points change in QMG score as a function of treatment assignment (Amifampridine or placebo) and the baseline QMG score. The point estimate and 95% confidence interval on the point estimate will be displayed.

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

4.2.3 Exploratory Efficacy Analysis

As noted above, AChR-MG subjects are not expected to respond positively to amifampridine treatment. No inferential statistics are planned for this subgroup.

Summary statistics (n, mean, standard deviation, minimum, median and maximum) by treatment for the MG-ADL score for Day 0, Day 10, and change from Day 0 (baseline) will be presented.

Summary statistics (n, mean, standard deviation, minimum, median and maximum) by treatment for the QMG score for Day 0, Day 10, and change from Day 0 (baseline) will be presented.

All 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 and 10)
- Physical examination (screening, Day 1, and Day 10 or termination from study)
- Electrocardiogram (ECG)
- Clinical laboratory results
- 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.

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
A shift table of physical exam results will be created showing the shifts in results by parameter relative to the normal ranges. The number and percentage of subjects with the following shifts will be presented: normal/normal, normal/low, normal/high, low/low, low/normal, low/high, high/low, high/normal, and high/high. The physical exam data collected at screening, Day 0 and Day 10 (or termination from the study) will be listed.

Electrocardiogram
A table containing descriptive statistics for QTc values measured at screening, Day 0 and Day 10 (or termination from the study) and CFB by treatment will be created. A shift table showing normal/normal, normal/abnormal, abnormal/normal and abnormal/abnormal shifts as counts and percentages for Days 0 and 10 will be created. The ECG data collected will be listed.

Clinical Laboratory Results
Tables containing descriptive statistics for serum chemistry, hematology and urinalysis values measured at screening (pre-treatment level, the baseline value), Day 0 and Day 10 (or termination from the study) and Change From Screen Level (CFS) for both Day 0 and Day 10 by treatment will be created. In addition, a shift table will be constructed to show the shifts in laboratory results by parameter relative to the normal ranges. The number and percentage of subjects with the following shifts will be presented: normal/normal, normal/low, normal/high, low/low, low/normal, low/high, high/low, high/normal, and high/high.

Concomitant Medications
A table of the WHO-coded medications will be constructed by treatment group and overall with medications summarized by anatomical therapeutic chemical (ATC) level 4 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 randomization) + 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 Subject Data Profiles

A Subject Data Profile listing will be provided. It will contain demographic information, randomization information, all endpoint assessments and laboratory measurements. See Appendix B, Data Listing 19 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 (All subjects)	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	MG-ADL Total Score Summary Statistics by Time Point and MG Type (FAS Population)	X	X
6	MG-ADL Total Score Summary Statistics by Time Point and MG Type (PP Population)	X	
7	MG-ADL CFB Analysis (FAS Population)	X	X
8	MG-ADL CFB Analysis (PP Population)	X	
9	MG-ADL CFB ANOVA Table (FAS Population)	X	X
10	MG-ADL CFB ANOVA Table (PP Population)	X	
11	QMG Total Score Summary Statistics by Time Point and MG Type (FAS Population)	X	X
12	QMG Total Score Summary Statistics by Time Point and MG Type (PP Population)	X	
13	QMG Total CFB Analysis (FAS Population)	X	X
14	QMG Total CFB Analysis (PP Population)	X	
15	QMG Total CFB ANOVA Table (FAS Population)	X	X
16	QMG Total CFB ANOVA Table (PP Population)	X	
17	MG-ADL Sensitivity Analysis (FAS Population)	X	X
18	MG-ADL Sensitivity Analysis (PP Population)	X	
19	MG-ADL Score Shift of At Least 2 Points, MuSK MG Subjects (FAS Population)	X	X
20	MG-ADL Score Shift of At Least 2 Points, MuSK MG Subjects (PP Population)	X	
21	QMG Score Shift of At Least 3 Points, MuSK MG Subjects (FAS Population)	X	X
22	QMG Score Shift of At Least 3 Points, MuSK MG Subjects (PP Population)	X	
23	Number and Percent of Subjects with Treatment Emergent Adverse Events (Safety Population)	X	X
24	Summary of Treatment Emergent Adverse Events (Safety Population)	X	X
25	Number and Percent of Subjects with Serious Treatment Emergent Adverse Events (Safety Population)	X	X
26	Number and Percent of Subjects with Treatment Emergent Adverse Events by Relationship to Treatment (Safety Population)	X	X
27	Number and Percent of Subjects with Treatment Emergent Adverse Events by Severity Grade (Safety Population)	X	X
28	ECG Shift Summary Statistics by Treatment (Safety Population)	X	X

Table No.	Table Title	Included in Final Tables	Shown in Appendix B
29	ECG QTc Interval Summary Statistics by Time Point and Treatment (Safety Population)	X	X
30	Serum Chemistry Clinical Laboratory Summary Statistics by Time Point and Treatment (Safety Population)	X	X
31	Hematology Clinical Laboratory Summary Statistics by Time Point and Treatment (Safety Population)	X	
32	Urinalysis Clinical Laboratory Summary Statistics by Time Point and Treatment (Safety Population)	X	
33	Serum Chemistry Shift Table by Treatment (Safety Population)	X	X
34	Hematology Shift Table by Treatment (Safety Population)	X	
35	Urinalysis Shift Table by Treatment (Safety Population)	X	
36	Vital Sign Parameters Summary Statistics (Safety Population)	X	X
37	Vital Signs Shift Table by Treatment (Safety Population)	X	X
38	Number and Percent of Subjects Taking Concomitant Medications by ATC Level 3 and Preferred Term (Safety Population)	X	X

Figure No.	Figure Title	Included in Final Figures	Shown in Appendix B
Fig1	Mean MG-ADL Total Score by Time Point and MG Type (FAS Population)	X	X
Fig2	Mean MG-ADL Total Score by Time Point and MG Type (PP Population)	X	
Fig3	Mean QMG Total Score by Time Point and MG Type (FAS Population)	X	
Fig4	Mean QMG Total Score by Time Point and MG Type (PP Population)	X	
Fig5	Randomization Test Histogram for MG-ADL Total Score (FAS Population)	X	X
Fig6	Randomization Test Histogram for MG-ADL Total Score (PP Population)	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

Listing No.	Data Listing Title	Included in Final Listings	Shown in Appendix B
DL9	Physical Exam Data Listing	X	X
DL10	Vital Signs Data Listing	X	X
DL11	ECG Data Listing	X	X
DL12	Study Drug Administration Data Listing	X	X
DL13	Serum Chemistry Data Listing	X	X
DL14	Hematology Data Listing	X	X
DL15	Urinalysis Data Listing	X	X
DL16	Amifampridine Level Data Listing	X	X
DL17	MG-ADL Data Listing	X	X
DL18	QMG Data Listing	X	X
DL19	Subject Data Profile	X	X

8.0 References

1. Jennison, C; Turnbull, B.W.; "Group Sequential methods with Applications to Clinical Trials"; Chapman & Hall/CRC Press; 1999

2. Marcus, R.; Peritz, E.; Gabriel, K.R.; "On closed Testing Procedures with Special Reference to ordered Analysis of Variance"; Biometrica **63**, 655-660 (1976)

Appendix A – Tables, Figures and Listing Specifications

Orientation

Tables, figures, and listings will be displayed in landscape with the exception of the Subject Data Profile Listing (DL19), which will be in portrait layout.

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.
- 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.

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 (All subjects)
Catalyst Pharmaceuticals, Inc. – MSK-002

		Amifampridine (N=xx)	Placebo (N=xx)	Overall
Screen Failures				xx
Enrolled		xx	xx	xx
Completed		xx (xxx%)	xx (xxx%)	xx (xxx%)
Withdrawn		xx (xxx%)	xx (xxx%)	xx (xxx%)
Reason for Withdrawal	Adverse Event	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Lost To Follow-Up	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Death	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Physician Decision	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Protocol Deviation	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Study Terminated by Sponsor	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Withdrawal by Subject	xx (xxx%)	xx (xxx%)	xx (xxx%)
	Other	xx (xxx%)	xx (xxx%)	xx (xxx%)

The denominator for all percentages in the table is the number of enrolled subjects in the respective treatment group and overall.
STATKING Clinical Services (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. - MSK-002
Safety Population (N=xxx)

Variable	Treatment Group	Mean	Std Dev	n	Min	Max	Median
Age (years)	Amifampridine	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	xxx	xxx	xxx	xxx	xxx	xxx
	Overall	xxx	xxx	xxx	xxx	xxx	xxx
Baseline Weight (kg)	Amifampridine	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	xxx	xxx	xxx	xxx	xxx	xxx
	Overall	xxx	xxx	xxx	xxx	xxx	xxx
Baseline Height (cm)	Amifampridine	xxx	xxx	xxx	xxx	xxx	xxx
	Placebo	xxx	xxx	xxx	xxx	xxx	xxx
	Overall	xxx	xxx	xxx	xxx	xxx	xxx

STATKING Clinical Services (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. - MSK-002
Safety Population (N=xxx)

Demographics Variable	Category	Amifampridine (N=xxx)	Placebo (N=xxx)	Overall (N=xxx)
Gender	Male	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Female	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Ethnicity	Hispanic or Latino	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
	Not Hispanic or Latino	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 3

Table 4. Summary of Study Drug Administration and Compliance
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

Myasthenia Gravis		Statistic	Amifampridine (N=xxx)	Placebo (N=xxx)
MuSK	Duration (days)	n	xxx	xxx
		Mean (Std Dev)	xxx (xxx)	xxx (xxx)
		Median	xxx	xxx
		Minimum, Maximum	xxx, xxx	xxx, xxx
	Compliance (%)	n	xxx	xxx
		Mean (Std Dev)	xxx (xxx)	xxx (xxx)
		Median	xxx	xxx
		Minimum, Maximum	xxx, xxx	xxx, xxx
AChR	Duration (days)	n	xxx	xxx
		Mean (Std Dev)	xxx (xxx)	xxx (xxx)
		Median	xxx	xxx
		Minimum, Maximum	xxx, xxx	xxx, xxx
	Compliance (%)	n	xxx	xxx
		Mean (Std Dev)	xxx (xxx)	xxx (xxx)
		Median	xxx	xxx
		Minimum, Maximum	xxx, xxx	xxx, xxx

STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 12

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Table 5. MG-ADL Total Score Summary Statistics by Time Point and MG type
Catalyst Pharmaceuticals, Inc. - MSK-002
FAS Population (N=xxx)

Myasthenia Gravis Type	Treatment	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
MuSK	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
AChR	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 10, 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 = Value at time point – Baseline value.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Table format will be repeated for the PP Population.

Table 7. MG-ADL CFB Analysis
Catalyst Pharmaceuticals, Inc. – MSK-002
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 MG-ADL total score was modeled as the response, with fixed effects terms for treatment and MG-ADL at Baseline.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Table format will be repeated for the PP Population.

Table 9. MG-ADL CFB ANOVA Table
Catalyst Pharmaceuticals, Inc. – MSK-002
FAS Population (N=xxx)

Source	Degrees of Freedom	Sum of Squares	Mean Sum of Squares	F Statistic	p-Value
Treatment	xxx	xxxxx	xxxxx	xxxx	xxxx
Baseline MG-ADL	xxx	xxxxx	xxxxx	xxxx	xxxx
Error	xxx	xxxxx	xxxxx		
Total	xxx	xxxxx			

[a] CFB for MG-ADL total score was modeled as the response, with fixed effects terms for treatment and MG-ADL at Baseline.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Table format will be repeated for the PP Population.

Table 11. QMG Total Score Summary Statistics by Time Point and MG Type
Catalyst Pharmaceuticals, Inc. – MSK-002
FAS Population (N=xxx)

Myasthenia Gravis Type	Treatment	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
MuSK	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
AChR	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 10, 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 = Value at time point – Baseline value.
[c] Observed significance level (p-value) for Wilcoxon-Mann-Whitney test of equality of change from baseline distributions.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 18

Table format will be repeated for the PP Population.

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Table 13. QMG Total CFB Analysis
Catalyst Pharmaceuticals, Inc. – MSK-002
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 18

Table format will be repeated for the PP Population.

Table 15. QMG CFB ANOVA Table
Catalyst Pharmaceuticals, Inc. – MSK-002
FAS Population (N=xxx)

Source	Degrees of Freedom	Sum of Squares	Mean Sum of Squares	F Statistic	p-Value
Treatment	xxx	xxxxx	xxxxx	xxxx	xxxx
Baseline QMG	xxx	xxxxx	xxxxx	xxxx	xxxx
Error	xxx	xxxxx	xxxxx		
Total	xxx	xxxxx			

[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 17

Table format will be repeated for the PP Population.

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Table 17. MG-ADL Sensitivity Analysis
Catalyst Pharmaceuticals, Inc. – MSK-002
FAS Population (N=xxx)

Statistic[a]	MG-ADL Score
P-value for Between-Treatment Difference in LS Means	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 17, Data Listing 18

Table format will be repeated for the PP Population.

Table 19. MG-ADL Score Shift Of At Least 2 Points, MuSK MG Subjects
Catalyst Pharmaceuticals, Inc. - MSK-002
FAS Population (N=xxx)

	Score change						
Treatment	Less Than 2 points	2 or more Points	Total	Logistic Regression Parameter	Point Estimate	95% Confidence Interval of Estimate	p-value[a]
Amifampridine	xxx	xxx	xxx	Treatment	xxxx	(xxxx,xxxx)	xxxx
Conditional	(xx.x%)	(xx.x%)		Baseline Score	xxxx	(xxxx,xxxx)	xxxx
Placebo	xxx	xxx	xxx				
Conditional	(xx.x%)	(xx.x%)					
Total	xxx	xxx	xxx				
	(xx.x%)	(xx.x%)					

[a] P-value for the test of the null hypothesis $\beta = 0$.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Table format will be repeated for the PP Population.

Table 21. QMG Score Shift Of At Least 3 Points, MuSK MG Subjects
Catalyst Pharmaceuticals, Inc. - MSK-002
FAS Population (N=xxx)

	Score change						
Treatment	Less Than 3 points	3 or more Points	Total	Logistic Regression Parameter	Point Estimate	95% Confidence Interval of Estimate	p-value[a]
Amifampridine	xxx	xxx	xxx	Treatment	xxxx	(xxxx,xxxx)	xxxx
Conditional	(xxx%)	(xxx%)		Baseline Score	xxxx	(xxxx,xxxx)	xxxx
Placebo	xxx	xxx	xxx				
Conditional	(xxx%)	(xxx%)					
Total	xxx	xxx	xxx				
	(xxx%)	(xxx%)					

[a] P-value for the test of the null hypothesis $\beta = 0$.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 18

Table format will be repeated for the PP Population.

Table 23. Number and Percent of Subjects with Treatment Emergent Adverse Events
Catalyst Pharmaceuticals, Inc. - MSK-002
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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

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Table 24. Summary of Treatment Emergent Adverse Events
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxxx)

	Amifampridine (N=xxx)	Placebo (N=xxx)	Overall (N=xxx)
Subjects with at Least One 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%)

STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 25. Number and Percent of Subjects with Serious Treatment Emergent Adverse Events
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

Adverse Event Category[a]:	Amifampridine (N=xxx)	Placebo (N=xxx)	Overall (N=xxx)
Total Number of Serious TEAEs	xxx	xxx	xxx
Subjects with at Least One Serious 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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 26. Number and Percent of Subjects with Treatment Emergent Adverse Events
by Relationship to Treatment
Catalyst Pharmaceuticals, Inc. - MSK-002
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 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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 27. Number and Percent of Subjects with Treatment Emergent Adverse Events by Severity Grade
Catalyst Pharmaceuticals, Inc. - MSK-002
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 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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 27. Number and Percent of Subjects with Treatment Emergent Adverse Events by Severity Grade
Catalyst Pharmaceuticals, Inc. - MSK-002
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 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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 28. ECG Shift Summary Statistics by Treatment
Catalyst Pharmaceuticals, Inc. MSK-002
Safety Population (N=xxx)

Treatment Groups	Day 0 (Baseline) Normal/ End of Study[a] Normal	Day 0 (Baseline) Normal/ End of Study[a] Abnormal	Day 0 (Baseline) Abnormal/ End of Study[a] Normal	Day 0 (Baseline) Normal/ End of Study[a] Abnormal
Amifampridine (N=xxx)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
Placebo (N=xxx)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] End of study is Day 10 or day of withdrawal from study, whichever is earlier.
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 11

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Table 29. ECG QTc Interval Summary Statistics
By Time Point and Treatment
Catalyst Pharmaceuticals, Inc. MSK-002
Safety Population (N=xxx)

Treatment	Visit	Data Type[a]	n	Mean (msec)	Std Dev	Min	Median	Max
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
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

[a] CFB refers to Change From Baseline CFB = Value at time point – Screening value.
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 11

Table 30. Serum Chemistry Clinical Laboratory Summary Statistics
by Time Point and Treatment
Catalyst Pharmaceuticals, Inc. MSK-002
Safety Population (N=xxx)

Laboratory Panel: Serum Chemistry

Parameter	Treatment	Visit	Data Type[a]	n	Mean	Std Dev	Min	Median	Max
xxxxxxxxxxxxxxxxxx	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
	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
xxxxxxxxxxxxxxxxxx	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
	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

[a] CFB refers to Change From Baseline CFB = Value at time point – Screening value.
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 13

The format of this table is repeated for hematology and urinalysis panels.

Table 33. Serum Chemistry Shift Table by Treatment
Catalyst Pharmaceuticals, Inc. MSK-002
Safety Population (N=xxx)

		Baseline Low[a]			Baseline Normal			Baseline High		
Lab Parameter	Treatment	EoS[b] Low	EoS Normal	EoS High	EoS Low	EoS Normal	EoS High	EoS Low	EoS Normal	EoS High
xxxxxxxxxx	Amifampridine	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
xxxxxxxxxx	Placebo	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] Shifts represent screening/end of study (EoS), where EoS is Day 10 or day of withdrawal from study, whichever is earlier.
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 13

Table repeats for hematology and urinalysis panels.

Table 36. Vital Signs Parameters Summary Statistics
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

Treatment	Vital Sign Parameter (units)	Visit	Data Type[a]	n	Mean	Std Dev	Min	Max	Median
Amifampridine	xxxxxxxxxx (xxx)	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Day 0	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		End of Study[b]	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 0	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		End of Study[b]	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] CFB refers to Change From Baseline CFB = Value at time point – Screening value.
[b] End of study is Day 10 or day of withdrawal from study, whichever is earlier.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 10

Table 37. Vital Signs Shift Table by Treatment
Catalyst Pharmaceuticals, Inc. MSK-002
Safety Population (N=xxx)

		Baseline Low[a]			Baseline Normal			Baseline High		
Lab Parameter	Treatment	EoS Low	EoS Normal	EoS High	EoS Low	EoS Normal	EoS High	EoS Low	EoS Normal	EoS High
xxxxxxxxxx	Amifampridine	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
xxxxxxxxxx	Placebo	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] End of study (EoS) is Day 10 or day of withdrawal from study, whichever is earlier.
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 10

Table 38. Number and Percent of Subjects Taking Concomitant Medications
by ATC Level 3 and Preferred Term
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

Concomitant Medication Category[a,b]	Amifampridine (N=xxx)	Placebo (N=xxx)	Overall (N=xxx)
ATC Level 4 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 4 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 4 term followed by preferred term.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 7

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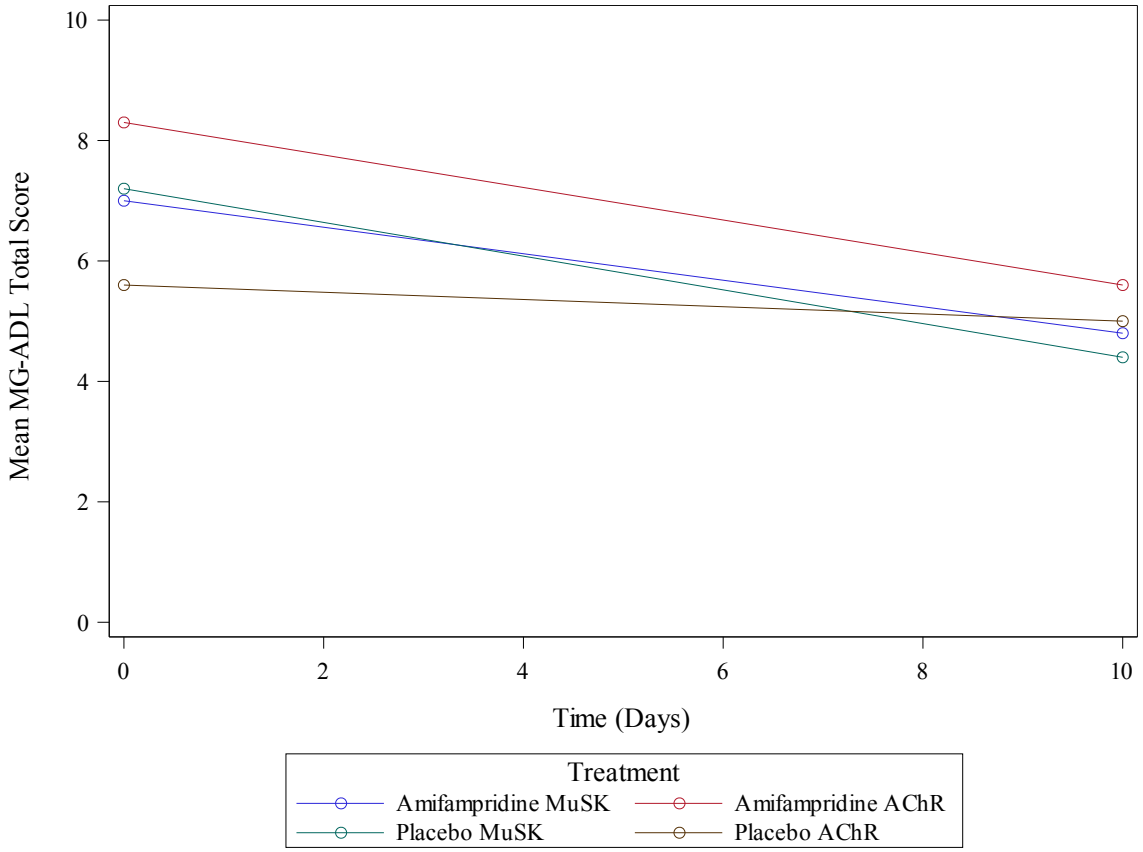
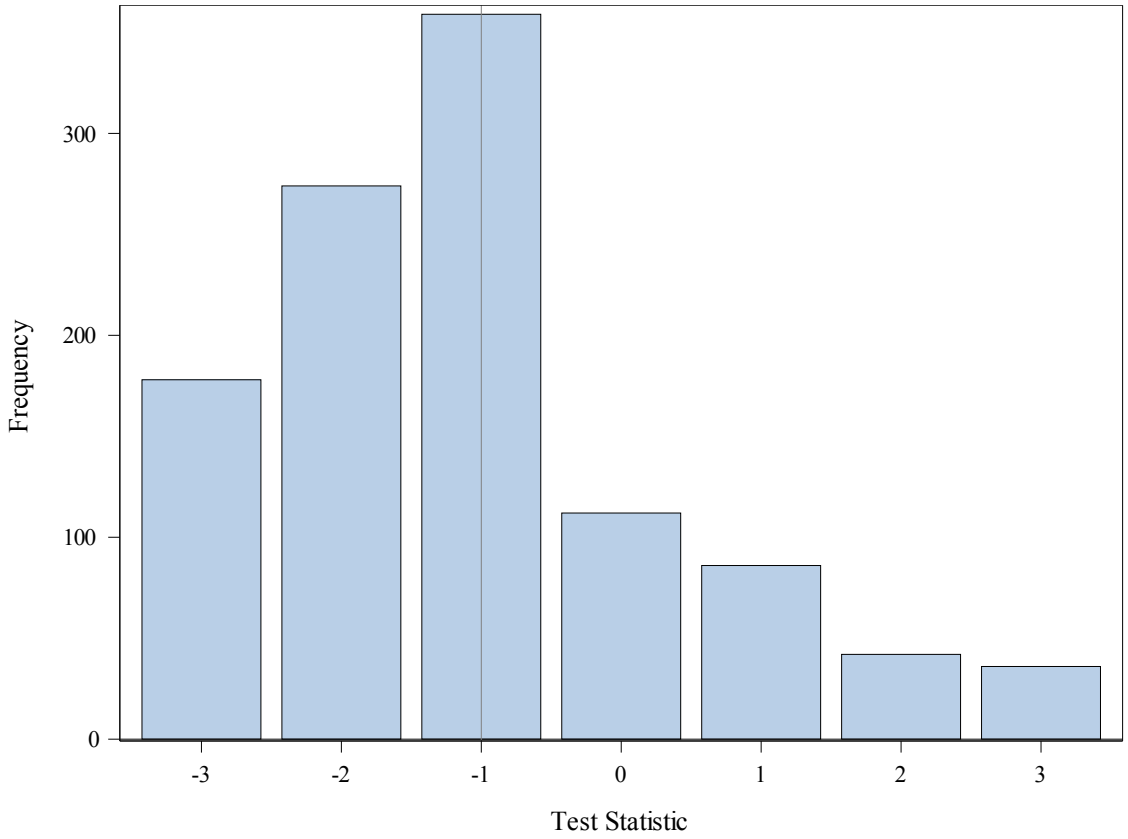
Figure 1. Mean MG-ADL Total Score by Time Point and MG Type
Catalyst Pharmaceuticals, Inc. - MSK-002
FAS Population (N=xxx)



STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Figure format will be repeated for the PP Population and for Mean QMG Total Score.

Figure 5. Randomization Test Histogram for MG-ADL Total Score
Catalyst Pharmaceuticals, Inc. - MSK-002
FAS Population (N=xxx)



STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Figure format will be repeated for the PP Population.

Data Listing 1. Subject Disposition Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002

Treatment	Subject No.	MG-Type	Disposition Status	Date of Disposition	Withdrawal Reason
xxxxxx	xxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxxxx

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

Data Listing 2. Protocol Deviations Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
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

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

Data Listing 3. Demographics Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

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

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

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Data Listing 4. Subjects Excluded from FAS Population Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

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

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

Data Listing 5. Subjects Excluded from PP Population Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
All Enrolled Subjects (N=xxx)

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

Data Listing 6. Medical History Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

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Data Listing 7. Prior and Concomitant Medications Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

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

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

Data Listing 8. Adverse Events Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

Treatment	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	xxxxxxxxx	xxxxxxx xxxxxxx/ xxxxxxx xxxxxxx	xxxxxxx xxxxxxx	xxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxx	xxxxxxxx	xxxxxxxx	xxx	xxxxxxxx
xxxxxx	xxxxxxxxx	xxxxxxx xxxxxxx/ xxxxxxx xxxxxxx	xxxxxxx xxxxxxx	xxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxx xxxxxxxxxxxxxxxxx	xxxxxxxx	xxxxxxxx	xxx	xxxxxxxx

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

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Data Listing 9. Physical Exam Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
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

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

Data Listing 10. Vital Signs Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
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

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

Data Listing 11. ECG Data Listing
Catalyst Pharmaceuticals – MSK-002
Safety Population (N=xxx)

Subject No.	MG Type	Age	Time Point	Date	Time	Heart Rate	PR Interval	QRS Duration	QT Interval	ECG Assessment/ If Abnormal, Specify
xxxxxx	xxxx	xxxx	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 12. Study Drug Administration Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

Treatment	Subject No.	Treatment Start Date	Treatment End Date	Treatment Duration (Days)	Tablets Consumed	Dose (mg/day)	Tablets Prescribed[a]	Compliance (%)[b]
xxxxxx	xxxx	xxxxxx	xxxxxx	xxx	xxx	xxx	xxx	xxx
xxxxxx	xxxx	xxxxxx	xxxxxx	xxx	xxx	xxx	xxx	xxx

[a] Number of tablets prescribed is computed as the duration times the number of tablets to have been taken daily.
[b] Compliance is computed as 100%*(number of tablets consumed)/(number of tables prescribed).
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas

Data Listing 13. Serum Chemistry Data Listing
Catalyst Pharmaceuticals – MSK-002
Safety Population (N=xxx)

Subject No.	Time Point	Date	Parameter (Units)	Value	Assessment/ If Abnormal, Specify
xxxxxx	xxxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxxx
			xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx

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

Data Listing 14. Hematology Data Listing
Catalyst Pharmaceuticals – MSK-002
Safety Population (N=xxx)

Subject No.	Time Point	Date	Parameter (Units)	Value	Assessment/ If Abnormal, Specify
xxxxxx	xxxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxxx
			xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx

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

Data Listing 15. Urinalysis Laboratory Data Listing
Catalyst Pharmaceuticals – MSK-002
Safety Population (N=xxx)

Subject No.	Time Point	Date	Parameter (Units)	Value	Assessment/ If Abnormal, Specify
xxxxxx	xxxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxxx
			xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx

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

Data Listing 16. Amifampridine Levels Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

Treatment	Subject No.	Visit	Date of Sample	Time of Sample	Amifampridine Level (Units)
xxxxxx	xxxx	xxxxxxxx	xxxxxxxx	xx:xx	xxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxx	xxxxxxxx	xx:xx	xxxxxxxxxxxxxxxxxxxx

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

Data Listing 17. MG-ADL Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

Treatment	Subject No.	Visit	Myasthenia Gravis ADL Score	Baseline Value	Change from Baseline	Best-Case Imputed Value	Worst-Case Imputed Value
xxxxxx	xxxx	xxxxxxxx	xxxx	xxxx	xxxx	xxxx	xxxx
xxxxxx	xxxx	xxxxxxxx	xxxx	xxxx	xxxx	xxxx	xxxx

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

Data Listing 18. QMG Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-002
Safety Population (N=xxx)

Treatment	Subject No.	Visit	Item	Score
xxxxxx	xxxx	xxxxxxxx	Double Vision (Lateral Gaze) Sec.	xxxx
			Bothersome 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

Data Listing 19. Subject Data Profile
Catalyst Pharmaceuticals, Inc. – MSK-002
Safety Population (N=xxx)

Study Number: MSK-002 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 MG Type: xxxxxxxx

Endpoint Measurements

Myasthenia Gravis – Activities of Daily Living Scores

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

Quantitative Myasthenia Gravis Scores

Visit	Date	Item	Score	CFB
Baseline	xx-xxx-xxxxx	Double Vision Sec.	xxxxx	--
		Bothersome 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 (kg)	xxxxx	--
		Left Hand Grip (kg)	xxxxx	--
		Head, Lifted	xxxxx	--
		Right Leg Outstretched	xxxxx	--
		Left Leg Outstretched	xxxxx	--
		Limb Total	xxxxx	--
		Total	xxxxx	--
Day x	xx-xxx-xxxxx	Double Vision Sec.	xxxxx	xxxxx
		Bothersome 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

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

Data Listing 19. Subject Data Profile
Catalyst Pharmaceuticals, Inc. – MSK-002
Safety Population (N=xxx)

Study Number: MSK-002 Site: xxxxxxxxx Subject ID: xxxxx

Quantitative Myasthenia Gravis Scores

Visit	Date	Item	Score	CFB
Day x	xx-xxx-xxxxx	Right Hand Grip (kg)	xxxxx	xxxxx
		Left Hand Grip (kg)	xxxxx	xxxxx
		Head, Lifted	xxxxx	xxxxx
		Right Leg Outstretched	xxxxx	xxxxx
		Limb Total	xxxxx	xxxxx
		Total	xxxxx	xxxxx

Safety Measurements

Laboratory Values

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

Electrocardiogram Values

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

Vital Sign Values

Visit	Date	Parameter (units)	Result	Abnormal Criterion
Baseline	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxx	xxxx	

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

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Data Listing 19. Subject Data Profile
Catalyst Pharmaceuticals, Inc. – MSK-002
Safety Population (N=xxx)

Study Number: MSK-002 Site: xxxxxxxxx Subject ID: xxxxx

Vital Sign Values

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

Adverse Events

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

Concomitant Medications

Preferred Term	Dose (units, freq)	Start Date	Stop Date
xxxxxxx	xxxxx (xxxx, xxxx)	xx-xxx-xxxx	xx-xxx-xxxx
xxxxxxx	xxxxx (xxxx, xxxx)	xx-xxx-xxxx	xx-xxx-xxxx
xxxxxxx	xxxxx (xxxx, xxxx)	xx-xxx-xxxx	xx-xxx-xxxx
xxxxxxx	xxxxx (xxxx, xxxx)	xx-xxx-xxxx	xx-xxx-xxxx
xxxxxxx	xxxxx (xxxx, xxxx)	xx-xxx-xxxx	xx-xxx-xxxx

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

Table repeats per subject beginning on a new page.