Cover Page for Protocol

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Statistical Analysis Plan (SAP)

Study No. MSK-003

Long Term Safety Study of Amifampridine Phosphate in Patients with MuSK Antibody Positive and AChR Antibody Positive Myasthenia Gravis Patients

Version 1.0

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Revision History

N/A

Table of Contents

1.0 Synopsis of Study Design Procedures

This is an open label observational study. The purpose of this study is to evaluate the long-term safety and tolerability of amifampridine phosphate in patients with muscle-specific receptor tyrosine kinase (MuSK) antibody positive and Acetylcholine receptor (AChR) antibody positive myasthenia gravis (MG) patients who participated in the MSK-002 study. The objectives of this Phase 3 study are as follows:

- Primary
  - To characterize the long-term safety and tolerability of amifampridine phosphate in patients with MG.

- Secondary
  - To assess the clinical efficacy of amifampridine phosphate over time in patients with MG based on change in Myasthenia Gravis Activities of Daily Living Score (MG-ADL).

1.1 Design and Treatment

The study will enroll patients who have completed the MSK-002 study and after all final evaluations for that study have been completed, or those who demonstrated benefit after completing the dose titration period but failed to meet the randomization criteria on Day 0 of MSK-002.

The duration of participation for each patient is expected to be at least 9 months as patients may continue in the study until amifampridine is approved by Regulatory Agencies or the clinical development of amifampridine is terminated for this indication. In addition to amifampridine, patients will continue to receive previous concomitant medications, as needed.

After a new informed consent is signed and inclusion / exclusion criteria for the current protocol are satisfied, eligible patients will be given the optimal dose and dosing schedule that was identified in the Run-in Period from Protocol MSK-002. The findings from the physical exam (including vital signs, weight); 12-lead ECG; clinical laboratory test results (including pregnancy testing) and any ongoing adverse events will be used as the baseline for the Long-Term safety evaluation.

The only efficacy measurement is the MG-ADL which will be completed every 3 or 6 months. Safety and tolerability assessments will be made every 3 or 6 months or more frequently at the discretion of the Investigator. The study will continue until amifampridine is approved by Regulatory Agencies or until development of the product for this indication is halted. The Investigator may alter the dose and dosing frequency during the Long-Term Study as well as schedule additional clinic visits for any reason.

Patients will be seen in the clinic at the end of Months 3, 6, 9, 12, 15, 21, 27, 33, 39. In between the 3-or 6-month visits, patients may also have telephone/video contact with the site, or unscheduled visits, if needed.

1.2 Study Procedures

- Start of Long Term Study

The following procedures are to be performed as scheduled Day 0 and can serve as baseline for the Long-Term study. Any procedure not performed for MSK-002 protocol needs to be completed for this trial.

  - Informed Consent for MSK-003;
  - Inclusion/Exclusion criteria for the current study must be verified;
  - Standard 12-lead ECG after 5 minutes in the supine position;
  - Complete physical examination including weight;
  - Vital signs (seated position), including SBP, DBP, heart rate, respiration, and body temperature;
  - Clinical laboratory tests including hematology, chemistry, and urinalysis;
  - Urine pregnancy test in females of childbearing potential only;
  - MG-ADL (use data from Day 0 of MSK-002 for all patients);
  - Adverse Events (AEs);
  - Concomitant medications.

- End of Months 3, 6, 9, 12, 15, 21, 27, 33 and 39

The following assessments and procedures will be determined at the end of Months 3, 6, 9, 12, 15 and 21, 27, 33 and 39. Each month has a clinic visit window of ± 1 week. Additional visits are allowed as necessary.

  - Assessment of AEs/ Serious Adverse Events (SAEs);
  - Complete physical exam with weight;
  - Vital signs (seated position), including SBP, DBP, heart rate, respiration, and body temperature;
  - Standard 12-lead ECG after 5 minutes in the supine position;
  - Clinical laboratory tests including hematology, chemistry, and urinalysis;
  - Urine pregnancy test in females of childbearing potential only;
  - Concomitant medications;
  - IP accountability of medication;
  - MG-ADL.

- End of Study Visit
  - Month 39 is the last subject visit in the study, and no IP should be dispensed at Month 39 visit. End of study assessments and procedures listed below should be performed for each subject along

with final IP accountability. This visit may be incorporated at the scheduled 3-or 6-month visit.
- Assessment of AEs/SAEs;
- Complete physical exam with weight;
- Vital signs (seated position), including SBP, DBP, heart rate, respiration, and body temperature;
- 12-Lead Electrocardiogram (ECG);
- Clinical laboratory tests;
- Urine pregnancy test in females of childbearing potential only;
- Concomitant medications;
- MG-ADL.

1.3 Sample Size

The study is not powered with respect to any endpoint but is an observational study to assess long term safety and lack of tolerance to the effects of amifampridine on MG-ADL.

2.0 Data Analysis Considerations

2.1 Types of Analyses

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.

2.2 Analysis Populations

The following analysis populations will be defined:

Safety Population: The safety population will consist of all subjects who are enrolled in the study and have received at least one dose of amifampridine.

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

The FAS 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.

2.2.1 Subgroup Definitions

Subgroup analyses for safety 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. That is, all analyses will be based on the observed data (i.e., complete case analysis).

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.

All SAS code used to generate SDTM and ADaM datasets and the final tables and listings will be provided.

3.0 Analysis of Baseline Subject Characteristics

Baseline and demographic characteristics will be summarized by MG type 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

4.1 Description of Efficacy Variables

For this study the efficacy endpoints are secondary endpoints. The efficacy variable is the MG-ADL score at months 3, 6, 9, 12, 15, 21, 27, 33 and 39.

4.2 Analysis of Efficacy Variables

4.2.1 Secondary Efficacy Analysis

Summary statistics (n, mean, standard deviation, minimum, median and maximum) for the MG-ADL score at Months 3, 6, 9, 12, 15, 21, 27, 33 39, and change from baseline (Day 0 of MSK-002 for all patients) will be presented

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

5.0 Analysis of Safety

For this study, the primary endpoint is safety and tolerability of amifampridine at Months 3, 6, 9, 12, 15 and 21, 27, 33 and 39.

The safety variables for this study are:

- AEs
- Vital signs
- Physical examination
- 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 Medical Dictionary for Regulatory Activities (MedDRA). Based on these coded terms, treatment emergent AEs (TEAEs) will be summarized using system organ class and preferred term by MG type 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 MG type. 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, or termination from the study will be listed.

Electrocardiogram
A table containing descriptive statistics for QTc values measured at screening, or termination from the study and CFB by MG type will be created. A shift table showing normal/normal, normal/abnormal, abnormal/normal and abnormal/abnormal shifts as counts and percentages 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), or termination from the study and Change From Screen Level (CFS) by MG type 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 MG type group and overall with medications summarized by anatomical therapeutic chemical (ATC) level 3 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 MG type. 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 MG type.

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 18 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	Number and Percent of Subjects with Treatment Emergent Adverse Events (Safety Population)	X	X
7	Summary of Treatment Emergent Adverse Events (Safety Population)	X	X
8	Number and Percent of Subjects with Treatment Emergent Serious Adverse Events (Safety Population)	X	X
9	Number and Percent of Subjects with Treatment Emergent Adverse Events by Relationship to MG Type (Safety Population)	X	X
10	Number and Percent of Subjects with Treatment Emergent Adverse Events by Severity Grade (Safety Population)	X	X
11	ECG Shift Summary Statistics by MG Type (Safety Population)	X	X
12	ECG QTc Interval Summary Statistics by Time Point and MG Type (Safety Population)	X	X
13	Serum Chemistry Clinical Laboratory Summary Statistics by Time Point and MG Type (Safety Population)	X	X
14	Hematology Clinical Laboratory Summary Statistics by Time Point and MG Type (Safety Population)	X	
15	Urinalysis Clinical Laboratory Summary Statistics by Time Point and MG Type (Safety Population)	X	
16	Serum Chemistry Shift Table by MG Type (Safety Population)	X	X
17	Hematology Shift Table by MG Type (Safety Population)	X	
18	Urinalysis Shift Table by MG Type (Safety Population)	X	
19	Vital Sign Parameters Summary Statistics by MG Type (Safety Population)	X	X
20	Vital Signs Shift Table by MG Type (Safety Population)	X	X
21	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	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-Activities of Daily Living Data Listing	X	X
DL18	Subject Data Profile	X	X

8.0 References

NA

Appendix A – Tables, Figures and Listing Specifications

Orientation

Tables, figures, and listings will be displayed in landscape with the exception of the Patient Data Profile Listing (DL18), 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-003

		MuSK	AChR	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 MG 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-003
Safety Population (N=xxx)

Variable	MG Type	Mean	Std Dev	n	Min	Max	Median
Age (years)	MuSK	xxx	xxx	xxx	xxx	xxx	xxx
	AChR	xxx	xxx	xxx	xxx	xxx	xxx
	Overall	xxx	xxx	xxx	xxx	xxx	xxx
Baseline Weight (kg)	MuSK	xxx	xxx	xxx	xxx	xxx	xxx
	AChR	xxx	xxx	xxx	xxx	xxx	xxx
	Overall	xxx	xxx	xxx	xxx	xxx	xxx
Baseline Height (cm)	MuSK	xxx	xxx	xxx	xxx	xxx	xxx
	AChR	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-003
Safety Population (N=xxx)

Demographics Variable	Category	MuSK (N=xxx)	AChR (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-003
Safety Population (N=xxx)

	Statistic	MuSK (N=xxx)	AChR (N=xxx)
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

Table 5. MG-ADL Total Score Summary Statistics by Time Point and MG Type
Catalyst Pharmaceuticals, Inc. - MSK-003
FAS Population (N=xxx)

Part 1 of 2

MG Type[a]	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
MuSK	Day 0 of MSK-002 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Month 3	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 6	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 9	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 12	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 15	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 27	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 33	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 39	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] The treatment of MG result will be the result obtained at Months 3, 6, 9, 12, 15, 21, 27, 33 and 39, unless the subject discontinued treatment of MG 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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Table 5. MG-ADL Total Score Summary Statistics by Time Point and MG Type
Catalyst Pharmaceuticals, Inc. - MSK-003
FAS Population (N=xxx)

Part 2 of 2

MG Type[a]	Time Point[a]	Data Type[b]	Mean	Std Dev	n	Min	Max	Median
AChR	Day 0 of MSK-002 (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Month 3	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 6	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 9	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 12	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 15	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 27	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 33	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 39	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] The treatment of MG result will be the result obtained at Months 3, 6, 9, 12, 15, 21, 27, 33 and 39, unless the subject discontinued treatment of MG 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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 17

Table 6. Number and Percent of Subjects with Treatment Emergent Adverse Events
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

Adverse Event Category[a]:	MuSK (N=xxx)	AChR (N=xxx)
Total Number of Treatment Emergent Adverse Events (TEAEs)	xxx	xxx
Subjects with at Least One TEAE	xxx (xxx%)	xxx (xxx%)
System Organ Class 1	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)
System Organ Class 2	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	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 7. Summary of Treatment Emergent Adverse Events
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxxx)

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

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

Table 8. Number and Percent of Subjects with Treatment Emergent Serious Adverse Events
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

Adverse Event Category[a]:	MuSK N=xxx)	AChR (N=xxx)
Total Number of Serious Treatment Emergent Adverse Events (TESAEs)	xxx	xxx
Subjects with at Least One TESAE	xxx (xxx%)	xxx (xxx%)
System Organ Class 1	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	xxx (xxx%)	xxx (xxx%)
System Organ Class 2	xxx (xxx%)	xxx (xxx%)
Preferred Term 1	xxx (xxx%)	xxx (xxx%)
Preferred Term 2	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 9. Number and Percent of Subjects with Treatment Emergent Adverse Events
by Relationship to MG Type
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

	MuSK (N=xxx)			AChR (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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 8

Table 10. Number and Percent of Subjects with Treatment Emergent Adverse Events by Severity Grade
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

Part 1 of 2

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

Table 10. 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

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

Table 11. ECG Shift Summary Statistics by MG Type
Catalyst Pharmaceuticals, Inc. MuSK-003
Safety Population (N=xxx)

MG Type	Screening (Baseline) Normal/ End of Study[a] Normal	Screening (Baseline) Normal/ End of Study[a] Abnormal	Screening (Baseline) Abnormal/ End of Study[a] Normal	Screening (Baseline) Normal/ End of Study[a] Abnormal
MuSK (N=xxx)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
AChR (N=xxx)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] End of study is at least 9 months (i.e. until amifampridine is approved by Regulatory Agencies for the treatment of MG or the development program is discontinued for this indication).
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 11

Table 12. ECG QTc Interval Summary Statistics
By Time Point and MG Type
Catalyst Pharmaceuticals, Inc. MuSK-003
Safety Population (N=xxx)

Part 1 of 2

MG Type	Visit	Data Type[a]	n	Mean (msec)	Std Dev	Min	Median	Max
MuSK	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Month 3	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 6	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 9	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 12	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 15	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 27	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 33	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 39	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] CFB refers to Change from Baseline (Screening ECG).
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 11

Table 19. ECG QTc Interval Summary Statistics
By Time Point and MG Type
Catalyst Pharmaceuticals, Inc. MuSK-003
Safety Population (N=xxx)

Part 2 of 2

MG Type	Visit	Data Type[a]	n	Mean (msec)	Std Dev	Min	Median	Max
AChR	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
	Month 3	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 6	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 9	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 12	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 15	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 27	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 33	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx
	Month 39	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		CFB	xxx	xxx	xxx	xxx	xxx	xxx

[a] CFB refers to Change from Baseline (Screening ECG).
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 11

Table 13. Serum Chemistry Clinical Laboratory Summary Statistics
by Time Point and MG Type
Catalyst Pharmaceuticals, Inc. MuSK-003
Safety Population (N=xxx)

Laboratory Panel: Serum Chemistry

Parameter	MG Type	Visit	Data Type[a]	n	Mean	Std Dev	Min	Median	Max
xxxxxxxxxxxxxxxxxx	MuSK	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		End of study	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
xxxxxxxxxxxxxxxxxx	AChR	Screening (Baseline)	RAW	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 (Screening sample).
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 16. Serum Chemistry Shift Table by MG Type
Catalyst Pharmaceuticals, Inc. MSK-003
Safety Population (N=xxx)

Lab Parameter	MG Type	Low/ Low	Low/ Normal	Low/ High	Normal/ Low	Normal/ Normal	Normal/ High	High/ Low	High/ Normal	High/ High
xxxxxxxxxx	MuSK	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
xxxxxxxxxx	AChR	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, where end of study is least 9 months (i.e. until amifampridine is approved by Regulatory Agencies for the treatment of MG or the development program is discontinued for this indication).
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 13

Table repeats for hematology and urinalysis panels.

Table 19. Vital Signs Parameters Summary Statistics by MG Type
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

Part 1 of 2

Vital Sign Parameter (units)	MG Type	Visit	Data Type[a]	n	Mean	Std Dev	Min	Max	Median
xxxxxxxxxx (xxx)	MuSK	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Month 3	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 6	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 9	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 12	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 15	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 27	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 33	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 39	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
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 10

Table 19. Vital Signs Parameters Summary Statistics by MG Type
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

Part 2 of 2

Vital Sign Parameter (units)	MG Type	Visit	Data Type[a]	n	Mean	Std Dev	Min	Max	Median
xxxxxxxxxx (xxx)	AChR	Screening (Baseline)	RAW	xxx	xxx	xxx	xxx	xxx	xxx
		Month 3	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 6	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 9	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 12	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 15	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 21	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 27	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 33	RAW	xxx	xxx	xxx	xxx	xxx	xxx
			CFB	xxx	xxx	xxx	xxx	xxx	xxx
		Month 39	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
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 10

Table 20. Vital Signs Shift Table by MG Type
Catalyst Pharmaceuticals, Inc. MSK-003
Safety Population (N=xxx)

Lab Parameter	MG Type[a]	Low/ Low	Low/ Normal	Low/ High	Normal/ Low	Normal/ Normal	Normal/ High	High/ Low	High/ Normal	High/ High
xxxxxxxxxx	MuSK	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)
xxxxxxxxxx	AChR	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)	xxx (xxx%)

[a] End of study is least 9 months (i.e. until amifampridine is approved by Regulatory Agencies for the treatment of MG or the development program is discontinued for this indication). withdrawal from study, whichever is earlier.
STATKING Clinical Services (month day, year)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 10

Table 21. Number and Percent of Subjects Taking Concomitant Medications
by ATC Level 3 and Preferred Term
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

Concomitant Medication Category[a,b]	MuSK (N=xxx)	AChR (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.
STATKING Clinical Services (DD-MMM-YYYY)
Source Program: xxxxxxx.sas
Source Listing: Data Listing 7

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

Subject No.	MG Type	Disposition Status	Date of Disposition	Withdrawal Reason
xxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxx
xxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxx
xxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxx
xxxx	xxxxxxx	xxxxxxxxxxxxxxxxxxxx	xxxxxxxxx	xxxxxxxxxxxxxxxxxxxxxxxx

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

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

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

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

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

MG Type	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

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

MG Type	Subject No.	Reason for Exclusion
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxx

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

MG Type	Subject No.	Reason for Exclusion
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxx
xxxxxx	xxxx	xxxxxxxxxxxxxxxxxxxxxxxx

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

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

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

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

MG Type	Subject No.	WHO Preferred Term[a]/ Verbatim Drug Name/ Indication/ ATC Level 1 Term/ ATC Level 3 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-003
Safety Population (N=xxx)

MG Type	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

Page x of y

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

MG Type	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-003
Safety Population (N=xxx)

MG Type	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 – MuSK-003
Safety Population (N=xxx)

MG Type	Subject No.	Age	Time Point	Date	Time	Heart Rate	PR Interval	QRS Duration	QT Interval	ECG Assessment/ If Abnormal, Specify
xxxx	xxxxxx	xxxx	Screen	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
			Month 3	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
			Month 9	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
			Month 12	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
			Month 15	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
			Month 21	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
			Month 27	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
			Month 33	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxx	xxxxxxx/ xxxxxxxxxxxxxxxxxx
			Month 36	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-003
Safety Population (N=xxx)

MG Type	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 – MuSK-003
Safety Population (N=xxx)

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

Data Listing 14. Hematology Data Listing
Catalyst Pharmaceuticals – MuSK-003
Safety Population (N=xxx)

MG Type	Subject No.	Time Point	Date	Parameter (Units)	Value	Assessment/ If Abnormal, Specify
xxxx	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 Data Listing
Catalyst Pharmaceuticals – MuSK-003
Safety Population (N=xxx)

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

Data Listing 16. Amifampridine Levels Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

MG Type	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-Activities of Daily Living Data Listing
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

MG Type	Subject No.	Visit	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. Subject Data Profile
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

Study Number: MSK-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 MG Type: xxxxxxxx

Endpoint Measurements

Myasthenia Gravis – Activities of Daily Living Scores

Visit	Date		Score	CFB
Baseline	xx-xxx-xxxxx	Total	xxxxx	--
Month 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	--
Month 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
		Right Hand Grip (kg)	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 18. Subject Data Profile
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

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

Quantitative Myasthenia Gravis Scores

Visit	Date	Item	Score	CFB
Month x	xx-xxx-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	xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
Month x	xx-xxx-xxxx	xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	
		xxxxxxxxxxxxxxxxxxx	xxxx	

Electrocardiogram Values

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

Vital Sign Values

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

Data Listing 18. Subject Data Profile
Catalyst Pharmaceuticals, Inc. - MSK-003
Safety Population (N=xxx)

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

Vital Sign Values

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