STATISTICAL ANALYSIS PLAN

Protocol Number	CAP-1002-DMD-03
Trial Phase	Phase 2
Protocol Title	Open-Label Extension of the Halt cardiomyOPathy progrEssion in Duchenne (HOPE-Duchenne) Trial
Protocol Version	Amendment 1.0, 12 February 2018
Product Name or Number	CAP-1002 Allogeneic Cardiosphere-Derived Cells
Indication	Duchenne Muscular Dystrophy
Sponsor	Capricor, Inc. 8840 Wilshire Blvd., 2nd Floor Beverly Hills, CA 90211 USA
Document Type	Statistical Analysis Plan
Prepared by	[redacted]
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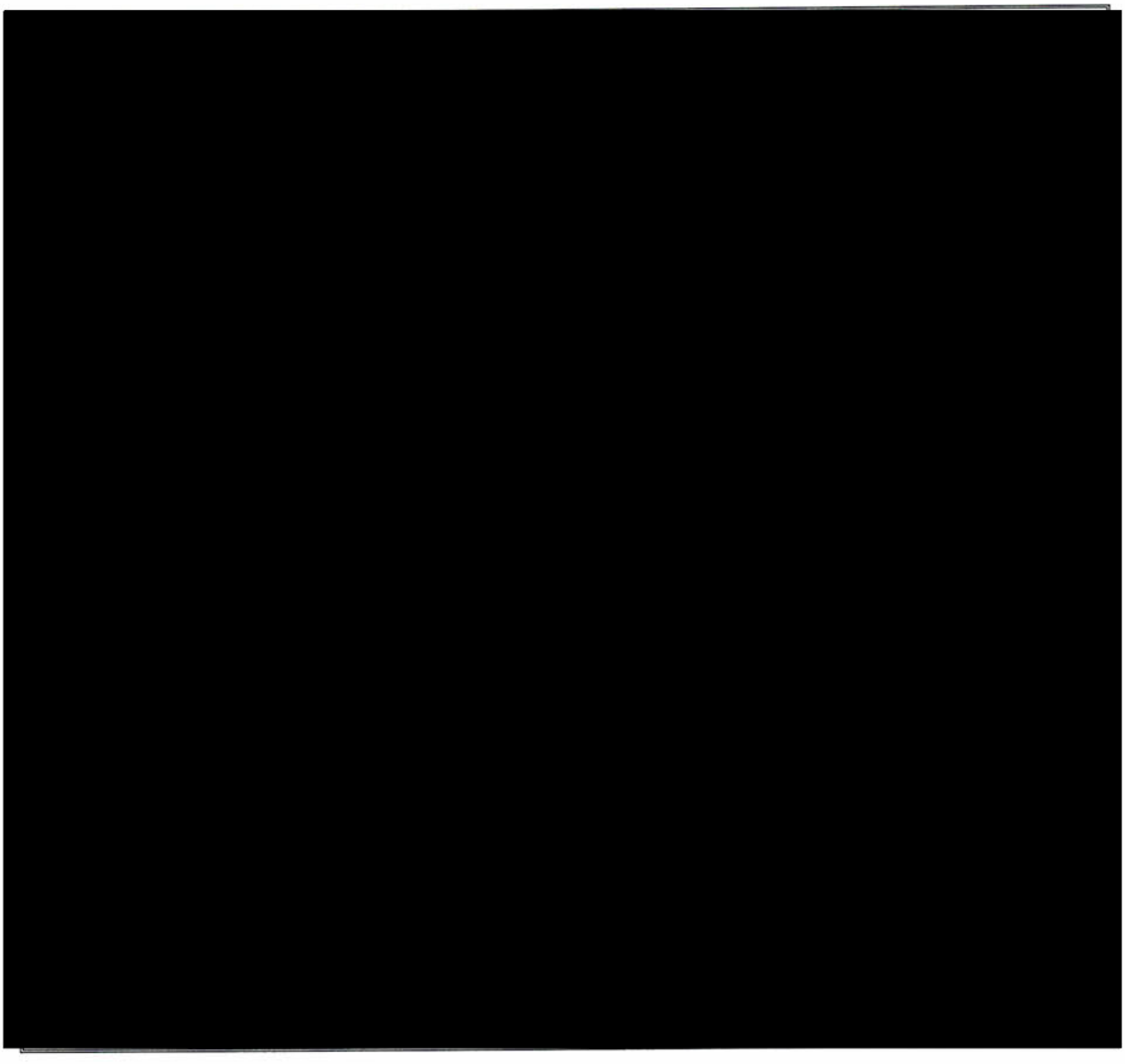
Final v. 1.0 (26 March 2019)

Open-Label Extension of the Halt cardiomyOPathy progrEssion in Duchenne (HOPE-Duchenne) Trial



REVISION HISTORY

Version	Date	Summary of Revision(s)
1.0	26 March 2019	New Document

LIST OF ABBREVIATIONS AND DEFINITION OF TERMS

Term	Explanation
6MWT	6-Minute Walk Test
AE	Adverse Event
ALT	Alanine Aminotransferase
AST	Aspartate Aminotransferase
ATC	Anatomic Therapeutic Chemical
BMI	Body Mass Index
BUN	Blood Urea Nitrogen
BSA	Body Surface Area
CBC	Complete Blood Count
CDC	Cardiosphere-Derived Cells
CEC	Clinical Events Committee
CI	Confidence Interval
CK-MB	Creatine kinase MB Isoenzyme
DMD	Duchenne Muscular Dystrophy
DSA	Donor-Specific Antibody
DSMB	Data Safety Monitoring Board
ECG	Electrocardiogram
EOS	End-of-Study
FEV_1	Forced Expiratory Volume in 1 Second
FVC	Force Vital Capacity
HLA	Human Leukocyte Antigen
IFR	Inspiratory Flow Reserve
IP	Investigational Product
ITT	Intent-to-Treat
IV	Intravenous
LS	Least Square

Term	Explanation
LV	Left Ventricle / Left Ventricular
LVEDV	Left Ventricular End-Diastolic Volume
LVEF	Left Ventricular Ejection Fraction
LVESV	Left Ventricular End-Systolic Volume
M cells	Million cells
MedDRA	Medical Dictionary for Regulatory Activities
MEP	Mean Expiratory Pressure
MFI	Mean Fluorescence Intensity
MIP	Mean Inspiratory Pressure
mL	Milliliter
MRI	Magnetic Resonance Image
OLE	Open-Label Extension
PCF	Peak Cough Flow
PEF	Peak Expiratory Flow
PFT	Pulmonary Function Test
PI	Primary Investigator
PP	Per Protocol
PT	Preferred Term
PUL	Performance of the Upper Limb
QTc	Corrected QT
RBC	Red Blood Cell
SAE	Serious Adverse Event
SD	Standard Deviation
SOC	System Organ Class
SOP	Standard Operating Procedures
SpO_2	Peripheral Capillary Hemoglobin Oxygen Saturation

Term	Explanation
SAP	Statistical Analysis Plan
TEAE	Treatment-Emergent Adverse Events
TLF	Tables, Listings and Figures

Term	Explanation
SVC	Slow Vital Capacity
WBC	White Blood Cell
WHODrug Global	World Health Organization Drug Global Dictionary

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1 STUDY OVERVIEW

Duchenne muscular dystrophy (DMD) is a severe, X-linked, progressive disease affecting approximately one in 3,600 to 9,200 male births caused by mutations in the dystrophin gene resulting in the absence of or non-functional dystrophin protein.

DMD occurs in early childhood, with degeneration occurring progressively in the skeletal musculature and ultimately in the heart and respiratory muscles, resulting in premature death. Progressive weakness and muscle atrophy caused by degenerating muscle fibers begins in the lower extremities and pelvis before spreading into the upper extremities. Other symptoms include loss of some reflexes, a waddling gait, frequent falls, difficulty when rising from a sitting or lying position or when climbing stairs, changes to overall posture, and impaired breathing. Many children precipitously lose the ability to run or jump. The atrophied muscles, in particular the calf muscles, and less commonly, muscles in the buttocks, shoulders, and arms, may be enlarged by an accumulation of fat and connective tissue, causing them to look larger and healthier than they actually are ("pseudohypertrophy"). Bone thinning and scoliosis are common. Ultimately, a wheelchair becomes necessary, in most cases between 12 to 15 years of age.

No pharmacologic or biological therapies proven to stop or reverse the progression of DMD have been approved in the US. Disease management consists primarily of preventive measures as well as active interventions to address the primary and secondary aspects of the disorder.

The proposed open-label extension (OLE) trial will enroll subjects randomized to the Usual Care Treatment Group in the HOPE-Duchenne trial (CAP-1002-DMD-01) and who completed the 12-month follow-up period. All subjects enrolled in the OLE will receive CAP-1002.

CAP-1002 is intended to be used as a therapeutic to improve the morbidities associated with DMD and thus improve or prevent decline in muscle function through its composite immune-modulatory, anti-fibrotic, and regenerative mechanisms of action. Preliminary clinical evidence from the HOPE-Duchenne trial in subjects with cardiomyopathy secondary to DMD suggests that CAP-1002 can have systemic effects; including benefits on skeletal function (as assessed by mid-level PUL) compared to usual care control subjects. This clinical evidence suggests that CAP-1002 has the potential to address unmet medical needs for patients with DMD.

2 STUDY OBJECTIVES AND ENDPOINTS

2.1 Study Objectives

2.1.1 Primary

To provide CAP-1002 to subjects who were randomized to the Usual Care Treatment Group of the HOPE-Duchenne trial and completed the 12-month follow-up period.

2.1.2 Secondary

To explore the safety and efficacy of intravenous administration of CAP-1002 with repeat dosing at Month 3.

2.2 Study Endpoints

2.2.1 Primary Safety Endpoint

The ***primary safety endpoints*** include the incidence of the following from baseline through the 6-month timepoint:

- Acute respiratory decompensation within 2 hours following Investigational Product (IP) administration
- Hypersensitivity reaction
  - Hypersensitivity reaction is defined as a clinical syndrome including, but not limited to, fever, leukocytosis, or rash with onset ≤ 2 hours post infusion and lasting < 24 hours, in the absence of clinical signs of concomitant infection.
- All-cause mortality
- Serious adverse events (SAEs)
- Treatment-emergent adverse events (TEAEs) related to IP or administration procedure
- Immune sensitization syndrome
  - Immune sensitization syndrome shall be defined as:
    (a) clinical signs and symptoms consistent with systemic inflammation (e.g., fever, leukocytosis, rash, or arthralgia) with onset ≥ 24 hours post infusion and the absence of clinical signs of concomitant infection,
    AND
    (b) elevation of anti-human leukocyte antigen (HLA) antibodies against the donor cells (i.e., DSAs), detected ≤ 30 days following onset of syndrome, of
    (i) ≥ 2000 mean fluorescent intensity (MFI) if baseline MFI ≤ 1000,
    or
    (ii) ≥ 2 times baseline otherwise

2.2.2 Exploratory Efficacy Endpoints

The ***exploratory endpoints*** include the change from baseline to each assessment timepoint for the following assessments:

- All subjects
  - Mid-level (elbow) dimension of the Performance of the Upper Limb (PUL) 1.2
  - Mid- and distal-level (elbow) dimension of the PUL 1.2
  - High-level (shoulder) dimension of the PUL 1.2
  - Left ventricular structure and function, as assessed by cardiac MRI, including regional systolic left ventricular wall thickening, ejection fraction, end-diastolic volume, end-systolic volume, stroke volume, regional wall thickness, and circumferential strain
  - Slow vital capacity (SVC), forced expiratory volume in one second (FEV_1), forced vital capacity (FVC), peak expiratory flow (PEF), maximum inspiratory pressure (MIP), maximum expiratory pressure (MEP), peak cough flow (PCF), and inspiratory flow reserve (IFR)

- Ambulatory subjects only
  - 6-Minute Walk Test (6MWT)

3 STUDY DESIGN

This Phase 2, multi-center, open label extension trial will provide CAP-1002 to subjects who were randomized to the Usual Care Treatment Group and completed 12 months of follow-up in the HOPE-Duchenne trial. The trial will assess the safety of CAP-1002, administered as two IV infusions, each separated by 3 months. All subjects will undergo targeted tests and procedures within 30 days prior to first infusion, unless otherwise stated, to confirm eligibility based on protocol inclusion and exclusion criteria.

Eligible subjects will undergo baseline safety and efficacy assessments (APPENDIX B) prior to the first infusion of CAP-1002. Administration of CAP-1002 (Day 1) should occur within a maximum of 14 days following confirmation of eligibility; if a delay of more than 14 days between enrollment and CAP-1002 administration is unavoidable, a conversation between the Investigator and Medical Monitor should occur to determine the need for repeat assessments prior to infusion. For the purposes of this trial, enrollment is defined as the time consent is signed.

All intravenous infusions will be conducted in an outpatient setting at the investigative site on Day 1 and at Month 3. Subjects will be observed in the outpatient setting for at least two hours post infusion and then discharged the same day if medically cleared by the site Investigator.

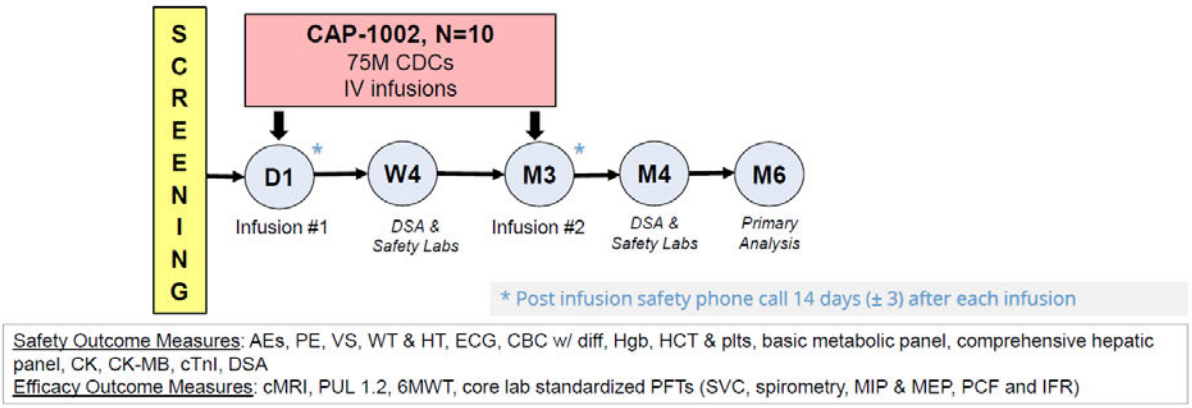
A safety phone call will be performed 14 days (±3 days) after each CAP-1002 infusion, and if clinically indicated, an unscheduled in-person visit will be performed at the investigative site with targeted assessments based on presentation of signs and symptoms.

Blood samples will be collected at Week 4 and Month 4 (±7 days, respectively) for DSA testing and safety laboratory assessments. Collection will occur either at the investigative site or remotely at a designated central laboratory patient service center.

Subsequent trial visits at the investigative site will occur at Months 3 and 6 (±14 days, respectively). Subjects will complete safety and efficacy assessments at each of these visits and prior to CAP-1002 administration in the case of the Month 3 trial visit.

A schematic of the study design is displayed in Figure 1.

Figure 1 Trial Design



3.1 Data Safety Monitoring Board

The Data Safety Monitoring Board (DSMB) will act in a senior advisory capacity to Capricor regarding data and safety matters throughout the duration of the trial. The objectives, roles and responsibilities of the DSMB, as well as the format and frequency of their meetings are documented in the DSMB charter.

4 GENERAL STRATEGIES FOR DATA PRESENTATION

Data with qualifiers (e.g. "<") will be listed with the qualifier but summarized without the qualifier. Subject data listings will include data collected for all subjects. Listings will be sorted by treatment, site, subject number, nominal visit, date and time (as applicable), unless otherwise indicated.

Categorical analyses will be summarized using counts and percentages. Percentages will be based on the number of subjects in the analysis set for whom data are available, unless otherwise specified. Continuous variables, including change from baseline, will be summarized using descriptive statistics (n, mean, standard deviation [SD], median, minimum, maximum).

All analyses will be performed using SAS v 9.3 or higher (SAS Institute, Inc, Cary, North Carolina, USA). Validation and quality control of the tables, listings and figures (TLFs) will follow the appropriate Innovative Analytics standard operating procedures (SOPs).

4.1 Treatment Group

The treatment group to be presented will be composed of those subjects who receive CAP-1002.

4.2 Study Day

The day of the first IP administration is defined as Day 1. All other study days will be labeled relative to Day 1. Thus, the study day for a particular event date on or after Day 1 is calculated as: *(Date of event – Date of first IP administration + 1)*. An event that occurs prior to Day 1 is calculated as: *(Date of event – Date of IP administration)*.

The duration of an event will be calculated as *(Event end date – Event start date + 1)*. Day 0 will not be used.

The baseline value for each subject is the last non-missing value obtained prior to the time of the first IP administration on Day 1.

4.3 Handling of Dropouts or Missing Data

Subject-level listings will present data as reported. Missing or partially missing dates that are required for date-dependent definitions (e.g., treatment-emergent adverse events, concomitant medications) will be assumed to be the most conservative date possible.

An adverse event (AE) with a completely missing start date will be considered treatment-emergent; similarly, an AE that started the same month and year as IP administration but with a missing start day will be considered treatment-emergent.

AEs with missing seriousness will be counted as "serious" in tables and missing in listings; likewise, AEs with missing severity will be counted as "severe" in tables and missing in listings, and AEs with missing relatedness to the IP and/or IP administration procedure will be counted as "possible" and missing in listings.

Medical history events with missing stop dates will be considered ongoing. Medications with missing stop dates will be considered in concurrent use during the study and counted in the summary table of concomitant medications unless the start date is after the last IP administration.

4.4 Analysis Populations

The following analysis populations will be defined.

Intent-to-Treat (ITT) Population: The Intent-to-Treat (ITT) population will be defined as all subjects who meet the enrollment criteria and are enrolled in the study.

Safety Population: The Safety population will be defined as all subjects who receive any amount of IP.

Per Protocol (PP) Population: The PP population will include subjects who have no protocol deviations/violations that could significantly impact the completeness, accuracy and/or reliability of the trial data. The list of subjects in the per protocol population will be compiled prior to database lock.

A summary of the number and percentage of subjects in each analysis population will be provided. A listing of subject assignment into each analysis population will also be displayed.

5 STUDY POPULATION PARAMETERS

The study population parameters to be listed and summarized are described below. The ITT population will be used for the listings and summaries. Some of the summaries may also be done for other analysis populations defined in Section 4.4.

5.1 Eligibility and Informed Consent

Eligibility and informed consent parameters will be provided in a listing, which will include the following: date of informed consent/assent, participation in HOPE-Duchenne protocol, protocol version, inclusion and/or exclusion criteria that were not met, if applicable, and subject infused with IP and reason subject was not infused, if applicable.

The number of subjects meeting all screening eligibility criteria and reasons for screen failure will be summarized.

5.2 Missed Visits and Assessments

A listing of missed efficacy visits and assessments will include visit and/or assessment missed.

5.3 Protocol Deviations

Protocol deviations will be listed and may include, but are not limited to:

- Visit not done or out-of-window
- Assessment not completed or out-of-window
- Inclusion/Exclusion
- Informed Consent/Assent
- IP Administration

The number and percentage of subjects with a protocol deviation will be summarized by protocol deviation category.

5.4 Demographics and Baseline Characteristics

Demographic and baseline characteristics will be listed and summarized. Demographic characteristics consist of age, sex, ethnicity, and race. A subject's age in years is calculated using the date of the informed consent/assent and date of birth using the following formula:

Age = [(Informed Consent/Assent Date – Birth Date + 1)/365.25].

Baseline characteristics to be summarized include ulna length, height, weight, and body mass index (BMI). In addition, subjects using wheelchair or scooter for part-time and for full-time will be summarized. For ambulatory subjects, the number of subjects able to perform the 6-Minute Walk Test and total distance walked at baseline will be summarized. Subject's height is either measured (i.e., standing height) or calculated from the subject's ulna length.

5.5 Medical Status Questionnaire

Subjects medical status will be displayed in subject listings, which will include date of completion, frequency of walker and manual/power wheelchair or scooter use, age permanently transitioned to a wheelchair full time, ventilatory support, supplemental O2 use, and frequency of falls.

5.6 General Medical History

General medical history will be coded using MedDRA version 21.0. Medical history will be listed and summarized. The summary will be presented by MedDRA System Organ Class (SOC) and Preferred Term (PT). At each level of subject summarization, a subject will be counted only once if that subject experienced the event more than once.

5.7 DMD Medical History

The following DMD medical history will be listed and summarized:

- Age at diagnosis (years),
- Age at first chronic glucocorticoid (years),
- Loss of stair climb and age at loss of stair climb (years),
- Loss of stair descend and age at loss of stair descend (years),
- Use of a walker and age at first walker use (years),

- Part-time use of a manual/power wheelchair or scooter and age at first part-time use of a manual/power wheelchair or scooter (years),
- Transition to full-time use of a manual/power wheelchair or scooter and age at first full-time use of a manual/power wheelchair or scooter (years),
- Start of non-invasive ventilatory support and age non-invasive ventilatory support started (years),
- Use of a medical cough assist device and age at first mechanical cough assist device use (years),
- History of spine surgery,
- History of symptomatic heart failure and age at first symptoms of heart failure (years), and
- History of medical (non-surgical) hospitalizations.

5.8 Donor-Specific Antibodies

Donor, donor profiles, allele, HLA, HLA Class, and MFI will be listed by subject and visit. Subject HLA profiles will be provided in a separate listing.

DSA data will be analyzed in the context of immune sensitization syndrome should there be an occurrence.

5.9 Physical Examination

Physical examination results will be listed and summarized. Observed status (e.g., normal, abnormal, not done) and changes from baseline in body system-specific physical examination findings will be summarized by visit and at end-of-study (EOS) within each body system.

5.10 Prior and Concomitant Medications

Prescription, over-the-counter, and alternative medication use will be coded to drug class, preferred drug name, and generic/trade drug name using the World Health Organization Drug Global dictionary (WHODrug Global) version September 1, 2017. Medications that were stopped before the start of the IP administration procedure will be considered “pre-treatment.” All other medications will be considered “concomitant.” Medications that were started or ongoing at the time the IP administration procedure was started will be considered “baseline” (a subset of “concomitant”). All reported medications will be listed.

Frequencies and percentages of subjects reporting or receiving each medication will be summarized by WHODrug Global Anatomic Therapeutic Chemical (ATC) Level 2 and Preferred Term. Pre-treatment and baseline medications will be summarized separately.

At each level of subject summarization, subjects who reported one or more medications within that level are only counted once for that level.

5.11 Planned Medical/Surgical Procedures

All reported elective medical and/or surgical procedures (e.g., wisdom tooth extraction) that were not the result of an adverse event will be displayed in subject listings. For each procedure, the type of procedure, indication, and start date/time will be reported.

Any administered medication(s) related to the planned medical/surgical procedure(s) will be captured as a concomitant medication.

5.12 IP Administration

The number of subjects that completed IP infusion at each visit will be summarized. For each visit, the number of patients who completed IP syringe administration, reason IP syringe was not completed, duration of IP administration (minutes), and calculated total dose of IP syringe delivered (M cells) will be summarized.

For the total dose of IP syringe delivered, 50mL of IP is equivalent to [redacted] cells of total dose of IP.

Details of IP administration will be displayed in subject listings.

5.13 Subject Study Progress

A listing of subject study progress will show dates of screening, informed consent, IP administration, study visits, and end-of-study (EOS). The number of subjects who completed each visit will be summarized.

5.14 Subject Disposition

All screen failures will be listed along with the primary reason for not receiving IP infusion.

Subject disposition will be summarized by treatment group for the ITT population overall and by site. The number of subjects who received IP, the reason for not receiving IP, the number of subjects who completed the study, the number of subjects who were discontinued from the study, and the primary reason for discontinuation will be summarized.

6 EFFICACY ANALYSES

Listings and summaries of the efficacy parameters will be done for both the ITT and PP populations, unless indicated otherwise. Efficacy parameters are described below.

6.1 PUL Scale

All subjects, regardless of ambulatory status, will complete testing the 1.2 module in the same preferred arm. Observed values and changes from baseline, mid- and distal-levels combined and for each dimension (high-level, shoulder; mid-level, elbow; distal-level, wrist and hand) separately, will be summarized. The 95% confidence intervals (CI) for the mean estimates will also be displayed.

The observed values will be displayed in subject listings.

6.2 Pulmonary Function Testing

Pulmonary function tests (PFTs) will measure SVC, FVC, FEV_1, PEF, MIP, MEP, PCF, and IFR. Observed best values (absolute and percent predicted, if it applies) and changes from baseline will be summarized. The 95% confidence intervals for the mean estimates will also be displayed.

The observed values (absolute, best value and percent predicted, if it applies) will be displayed in subject listings.

6.3 Cardiac MRI

LV structure and function as assessed by cardiac MRI include LV (myocardium) mass, , LV end-systolic volume, LV end-systolic volume index, LV end-diastolic volume, LV end-diastolic volume index, , LV stroke volume, LV ejection fraction, systolic wall thickening (anterior LV, lateral LV, inferior LV, septal LV), regional wall motion [dyssynchrony (CURE)], circumferential strain (global, anterior LV, lateral LV, inferior LV, septal LV), LV wall thickness that includes end-systolic wall thickness average (anterior, lateral, inferior, septal) and end-diastolic wall thickness average (anterior, lateral, inferior, septal).

In additional, the LV mass index (g/m^2) will be calculated as

$$\text{LV Mass Index } (g/m^2) = \text{LV Mass } (g)/\text{BSA } (m^2)$$

where Body Surface Area (BSA) = $0.007184 * \text{Height (cm)}^{0.725} * \text{Weight (kg)}^{0.425}$. Height and weight measurements closest to the MRI scan will be used. The standing height will be used, if provided, otherwise, the height from ulna length will be used.

Observed and changes from baseline will be summarized. The 95% confidence intervals for the mean estimates will also be displayed.

The observed values will be displayed in subject listings.

6.4 6-Minute Walk Test

Observed values and changes from baseline in the total distance walked during the 6-minute walk test will be summarized for ambulatory subjects only, if applicable.

The observed values for ambulatory subjects will be displayed in subject listings.

7 SAFETY ANALYSES

Listings and summaries of the safety parameters will be done for the Safety population. An overall summary table will be presented that summarizes the primary safety endpoints. Safety parameters are described below.

7.1 Adverse Events

Adverse events will be coded using the Medical Dictionary for Regulatory Activities (MedDRA) coding system (Version 21.0). Potential primary safety endpoints, described in Section 2.2.1, will be adjudicated by an independent Clinical Events Committee (CEC).

The following AEs are defined as treatment-emergent AEs (TEAEs):

- AEs occurring after the initiation of the IV catheter placement for the initial dose of IP; or
- AEs with a completely missing start date, or similarly, AEs that started the same month and year as IP administration but with missing start day.

Subject listings of all adverse events as reported will be provided. A separate listing will be done for TEAEs as adjudicated by the CEC. All summary tables will be reported using as adjudicated by the CEC.

An overall summary table will be presented by treatment group that summarizes subject with the following:

- Any TEAEs
- TEAEs by maximum severity
- TEAEs and serious TEAEs by greatest degree of relationship to IP or IP administration
- TEAEs and serious TEAEs by greatest degree of relationship to IP only
- TEAEs and serious TEAEs by greatest degree of relationship to IP administration only
- Serious TEAEs
- TEAEs leading to study discontinuation, and
- TEAEs leading to death

In addition, TEAEs will be summarized overall by system organ class and preferred term and by severity and by relationship to the IP and IP administration procedure.

Deaths, AEs that resulted in study discontinuation, serious adverse events (SAEs), serious TEAEs and AEs within 24 hours of IP administration will be listed and summarized.

Summaries of 'related' TEAEs, will include TEAEs with a reported relationship to IP and/or IP administration of "Probable" or "Possible". Adverse events will be displayed by MedDRA System Organ Class and Preferred Terms, with subjects who have the same adverse event counted only once for that event and with subjects who have more than one adverse event within a System Organ Class counted only once in that System Organ Class. The numbers and percentages of subjects reporting an event as well as the number of events that were reported will be displayed.

Frequency tables will be ordered by decreasing percentage in the SOCs, then alphabetically. Within each SOC, PTs will be ordered by decreasing percentage then alphabetically.

7.1.1 Clinical Events Committee (CEC)

To apply some level of consistency and standardization, all potential primary safety endpoints will be adjudicated by a CEC. The CEC can combine AEs reported separately into a single AE, can change the event description (i.e., the term used to report the AE), and can change the status of the reported event to a non-AE.

Any adjudicated event, including changed event descriptions, will be MedDRA-coded.

7.2 Partner Reported Pregnancy

Data from the Pregnancy Report Form will be listed, if applicable.

7.3 Laboratory Tests

Hematological testing will include complete blood count (CBC) with white blood cell (WBC) differential (absolute and percentage), hemoglobin, hematocrit and platelet count.

Serum chemistry testing will include: basic metabolic panel (Glucose, Sodium, Potassium, Chloride, Bicarbonate, Blood Urea Nitrogen (BUN), Creatinine, Calcium), comprehensive hepatic panel (Albumin, Alkaline Phosphatase, Total Protein, Alanine Aminotransferase (ALT), Aspartate Aminotransferase (AST), Direct Bilirubin, Total Bilirubin), and Creatine Kinase.

Cardiac enzymes testing will include CK-MB and Troponin I.

For hematology, serum chemistry, and cardiac enzyme quantitative tests, summary statistics will be presented for the observed values at each visit. The units to be used for each parameter are listed in APPENDIX A. Summary statistics will also be presented for the change from baseline values to each post-baseline visit and at End-of-Study (EOS). Scheduled assessments will be summarized, and unscheduled events will only be listed. The EOS value per subject is the last post-baseline value during the study.

The shift from baseline to each timepoint and at EOS will also be shown for the hematology and chemistry panels and cardiac enzyme parameters with results classified as low (L), Normal (N), and high (H) according to the laboratory supplied normal ranges. A summary of shift to low and high will be presented for each parameter, showing the number of subjects whose values shifted as a percentage of the number of subjects at risk for shifting, defined as follows:

- The number at risk for shifting to high is those with normal or low values at baseline.
- The number at risk for shifting to low is those with normal or high values at baseline.

For all tests, results will be displayed in subject listings, with those values falling outside the laboratory reference range flagged. Laboratory reference ranges will be provided by the laboratory site(s) and will be included in an appendix of the clinical study report.

7.4 Vital Signs, Height, Weight and BMI

Vital signs will include systolic and diastolic blood pressure, heart rate, respiratory rate, and body temperature and pulse oximetry (SpO_2). Height will either be measured (standing height) or calculated from the subject's ulna length. Observed values and changes from baseline will be summarized at each post-baseline visit and at EOS. Scheduled assessments will be summarized, and unscheduled events will only be listed. The EOS value per subject is the last post-baseline value during the study.

Listings of vital signs, ulna length, height, weight, and BMI will be provided.

7.5 12-Lead Electrocardiograms

Observed values and changes from baseline in ventricular rate and interval parameters (QRS, PR, RR, QT, QTc) will be summarized at each post-baseline visit and at EOS. Scheduled

assessments will be summarized, and unscheduled events will only be listed. The EOS value per subject is the last post-baseline value during the study.

All 12-lead ECG results will be displayed in subject listings.

APPENDIX A: LABORATORY PARAMETERS AND CORRESPONDING UNITS TO BE REPORTED

Test Panel Name	Test Code	Test Code Name	Reported Unit
Hematology	RBC	RED CELL COUNT (ERYTHROCYTES)	x10^6/uL or x10^12/L
	WBC	WHITE CELL COUNT (LEOKOCYTES)	x10^3/uL or x10^9/L
	HG9	HEMOGLOBIN	G/DL
	HCT	HEMATOCRIT	%
	PLT	PLATELET COUNT	x10^3/uL or x10^9/L
	DNN	BASOPHILS	%
	ZB4	BASOPHILS ABSOLUTE	x10^3/uL or x10^9/L
	DLN	EOSINOPHILS	%
	ZB3	EOSINOPHILS ABSOLUTE	x10^3/uL or x10^9/L
	DJN	MONOCYTES	%
	ZB2	MONOCYTES ABSOLUTE	x10^3/uL or x10^9/L
	DDN	LYMPHOCYTES	%
	ZB1	LYMPHOCYTES ABSOLUTE	x10^3/uL or x10^9/L
	ZB0	NEUTROPHILS ABSOLUTE	x10^3/uL or x10^9/L
	DCM	TOTAL NEUTROPHILS	%
Serum Chemistry	XGU	GLUCOSE	MG/DL
	XNA	SODIUM	MMOL/L
	XKB	POTASSIUM	MMOL/L
	DCL	CHLORIDE	MMOL/L
	XO2	CARBON DIOXIDE (CO2)	MMOL/L
	XUN	UREA NITROGEN	MG/DL
	PD93	CREATININE ENZ, SER	MG/DL
	XAB	CALCIUM	MG/DL
	XAL	ALBUMIN	G/DL
	XLK	ALKALINE PHOSPHATASE	U/L
	XTP	PROTEIN, TOTAL SERUM	G/DL
	XGO	ASAT (SGOT)	U/L
	XGP	ALAT (SGPT)	U/L
	XDB	BILIRUBIN, DIRECT	MG/DL
	XBT	BILIRUBIN, TOTAL	MG/DL
	CK0	CPK, TOTAL	U/L
CK-MB	CK5	CK-MB	NG/ML
Troponin I	UTNI	ULTRA TROPONIN I	NG/ML

APPENDIX B: SCHEDULE OF ASSESSMENTS

Procedure / Event [1]	**Screening**	**Day 1 (Infusion #1)**	**Post-Infusion #1 Safety Monitoring**	**Week 4**	**Month 3 (Infusion #2)**	**Post-Infusion #2 Safety Monitoring**	**Month 4**	**Month 6 / Early Termination**[2]
Trial Day (Visit Window)	***≤ 30 days prior to Day 1***		***14 days (±3) after Infusion #1***	***30 days (± 7)***	***90 days (±14)***	***14 days (±3) after Infusion #2***	***120 days (± 7)***	***180 (±14)***
Informed Consent / Assent	X							
Eligibility Assessment	X	X						
Prior & Concomitant Meds	X	X	X		X	X		X
Medical History	X							
Medical Status Questionnaire		X			X			X
Adverse Events	X	X	X		X	X		X
CAP-1002 IV Infusion[3]		X			X			
Post-Infusion Safety Call [4]			X			X		
Vital Signs		X			X			X
Height / Ulna Length[5]		X			X			X
Weight		X			X			X
Physical Examination		X			X			X
12-Lead ECG		X			X			X
Serum Chemistry[6] [7]	X	X		X	X		X	X
Hematology[6] [8]		X		X	X		X	X
Cardiac Enzymes[6] [9]		X			X			X
HLA Typing[6]		X						

Procedure / Event [1]	**Screening**	**Day 1 (Infusion #1)**	**Post-Infusion #1 Safety Monitoring**	**Week 4**	**Month 3 (Infusion #2)**	**Post-Infusion #2 Safety Monitoring**	**Month 4**	**Month 6 / Early Termination**[2]
Trial Day (Visit Window)	***≤ 30 days prior to Day 1***		***14 days (±3) after Infusion #1***	***30 days (± 7)***	***90 days (±14)***	***14 days (±3) after Infusion #2***	***120 days (± 7)***	***180 (±14)***
Donor-Specific Antibodies[6]		X		X	X		X	X
PUL 1.2		X			X			X
6-Minute Walk Test[10]		X			X			X
Pulmonary Function Testing[11]		X			X			X
Cardiac MRI[12]	X							X

[1] Sites will complete assessments in the following sequence: 1) safety and other trial assessments, excluding blood collection (e.g., vital signs, height, weight, 12- lead electrocardiogram [ECG], etc.), 2) pulmonary function testing (PFT), 4) PUL 1.2, 5) 6MWT, 6) blood collection, 7) cardiac MRI (if applicable), and 8) CAP-1002 Infusion (if applicable). All efforts must be made for the same clinical evaluator to complete assessments for the same subject at the same time of day throughout the trial (preferably in the morning).

[2] All attempts must be made to perform the trial assessments indicated for the Month 6 visit (i.e., final comprehensive visit) for subjects that decide to early terminate early from the trial after starting at least one CAP-1002 infusion.

[3] All CAP-1002 infusions will be conducted in an outpatient setting at the investigative site. Subjects will be observed for at least 2 hours post infusion, including pulse oximetry monitoring for at least 30 minutes post infusion. Sites will observe local institutional policies related to parenteral infusions and post infusion monitoring.

[4] A safety phone call will be performed 14 days (±3 days) after each CAP-1002 infusion, and if clinically indicated, an unscheduled in-person visit will be performed at the investigative site with targeted assessments based on presentation of signs and symptoms.

[5] If standing height cannot be measured, height will be calculated using a measurement of ulna length per Section 13.1.2.

[6] Blood samples will be collected using trial-specific laboratory kits, and then shipped to and tested at a central laboratory. Required sample collection for a visit may occur on multiple days; if the visit includes a CAP-1002 infusion, sample collections should occur prior to CAP-1002 administration.

[7] Basic metabolic panel (Glucose, Sodium, Potassium, Chloride, Bicarbonate, BUN, Creatinine, Calcium), comprehensive hepatic panel (Albumin, Alkaline Phosphatase, Total Protein, ALT, AST, Direct Bilirubin, Total Bilirubin)

[8] CBC with WBC differential, hemoglobin, hematocrit and platelet count

[9] CK-MB and troponin I

[10] Ambulatory subjects

[11] PFT testing sequence: SVC, forced maneuver (FEV_1/FVC/PEF), MIP, MEP, PCF, and IFR.

[12]Subjects will undergo cardiac MRI if they are physically capable as determined by an Investigator. Subjects that cannot complete a cardiac MRI as part of the trial will complete an echocardiogram at screening using the site's local equipment, acquisition protocol, and evaluation procedures. The reported LVEF from a local interpretation, whether it's measured via cardiac MRI or echocardiogram, will be used to assess eligibility (exclusion criterion 1).