CCI [redacted]

STATISTICAL ANALYSIS PLAN

AN ADAPTIVE PHASE I/II STUDY OF THE SAFETY OF CD4+ T LYMPHOCYTES AND CD34+ HEMATOPOIETIC STEM/PROGENITOR CELLS TRANSDUCED WITH LVsh5/C46, A DUAL ANTI-HIV GENE TRANSFER CONSTRUCT, WITH AND WITHOUT CONDITIONING WITH BUSULFAN IN HIV-1 INFECTED ADULTS PREVIOUSLY EXPOSED TO ART

Protocol No.: CAL-USA-11

Product Code: Cal-1

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SAP APPROVAL

By my signature, I confirm that this statistical analysis plan (SAP) has been approved for use on the CAL-USA-11 study:

Name	Title / Company	Signature	Date
PPD			

*Email approval sent by PPD at 17:46 PST

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List of Abbreviations

Term	Abbreviation
AE	Adverse Event
ANC	Absolute Neutrophil Count
ART	Antiretroviral Therapy
AUC	Area Under the Curve
Cal-1	LVsh5/C46, a Dual Anti-HIV Gene Transfer Construct
CI	Confidence Interval
CSR	Clinical Study Report
DBP	Diastolic Blood Pressure
DSMB	Data Safety Monitoring Board
eCRF	Electronic Case Report Form
EDC	Electronic Data Capture
GALT	Gut-associated Lymphoid Tissue
GCP	Good Clinical Practice
G-CSF	Granulocyte Colony-stimulating Factor
H	High
HIV-1	Human Immunodeficiency Virus Type 1
HSPC	CD34+ Hematopoietic stem/progenitor cells
$HSPC^{tn}$	Cal-1 transduced CD34+ Hematopoietic stem/progenitor cells
IP	Investigational Product
L	Low
MedDRA	Medical Dictionary for Regulatory Activities
MRC	Medical Review Committee
PD	Pharmacodynamic
PK	Pharmacokinetic
PT	Preferred Term
RCL	Replication Competent Lentivirus
RNA	Ribonucleic Acid
SAP	Statistical Analysis Plan

Term	Abbreviation
SAE	Serious Adverse Event
SAS	Statistical Analysis System
SBP	Systolic Blood Pressure
SD	Standard Deviation
SOC	System Organ Class
SOP	Standard Operating Procedures
SUSAR	Serious and Unexpected Suspected Adverse Reaction
T^{tn}	Cal-1 transduced CD4+ T lymphocytes
WBC	White Blood Cell

1.Introduction

The following SAP provides the outline for the statistical analysis of the data from the CAL-USA-11 study (protocol version 8 dated 08 July 2015).

The planned analyses identified in this SAP may be included in clinical study reports (CSRs), regulatory submissions, or future manuscripts. Also, post hoc analyses not necessarily identified in this SAP may be performed to further examine study data. Any post hoc, or unplanned, exploratory analyses performed will be clearly identified as such in the final CSR.

2.Project Overview

2.1.Description of Overall Study Design and Plan

Study Design Overview:

This is an adaptive design study to evaluate the safety and feasibility of LVsh5/C46, a Dual Anti-HIV Gene Transfer Construct (Cal-1) transduced CD34+ Hematopoietic stem/progenitor cells (HSPC) and CD4+ T lymphocytes in Human Immunodeficiency Virus Type 1 (HIV-1) infected subjects who have previously been exposed to Antiretroviral Therapy (ART) but are not currently taking any antiretroviral agent. Three cohorts of at least 3 subjects each will be enrolled:

- Cohort 1: No busulfan conditioning
- Cohort 2: Conditioning with a single dose of 4mg/kg busulfan.
- Cohort 3: Conditioning with two doses of busulfan, with first dose of 4, 3 or 2 mg/kg and second dose based on real-time pharmacokinetic-guided dosing to reach targeted drug exposure area under the curve (AUC).

Progressive enrollment of Cohort 1, then Cohort 2 and then Cohort 3 is dependent on the recommendation of an independent Data Safety Monitoring Board (DSMB).

Subjects will have cell collection procedures (aphereses) performed; first to collect unstimulated CD4+ T lymphocytes, followed by CD34+ HSPC after mobilization. The mononuclear cell populations obtained from each of these collection procedures will be separated into purified CD4+ T lymphocytes and CD34+ HSPC, respectively and processed (transduced, cultured and cryopreserved) separately.

The primary endpoint for all subjects will be at 48 weeks' post-infusion, after which they may continue follow-up for up to one additional year in the Follow-up Extension phase. All subjects will transfer to a separate long-term follow-up protocol that will monitor each subject for potential long term adverse effects of Cal-1. Secondary data points are collected up to study completion or until early withdrawal or secondary analysis endpoint criteria is met (discontinuation), whichever occurs first. Subjects should commence ART at any time during the study if CD4+ T lymphocyte counts are confirmed to decline < 350 cells/µl (mm3) and/or to less than 1/3 of the CD4+ T cell % at the defined assessment in section 3, if plasma HIV-1 RNA exceeds 250,000 copies/µl or > 20-fold increase from the defined assessment, or in event of pregnancy.

2.2.Objectives

2.2.1.Primary objective

The primary objectives are to evaluate in HIV-1 infected adults who have previously been on ART:

- The safety and feasibility of the introduction of Cal-1, gene-transduced, hematopoietic cell populations.
- The safety of intravenous busulfan to improve $HSPC^{tn}$ engraftment

Safety outcomes up to study completion will be measured per the safety evaluations. Feasibility measures will include the analysis of the Cal-1 transduced CD4+ T lymphocytes (T^{tn}) and Cal-1 transduced CD34+ Hematopoietic stem/progenitor cells ($HSPC^{tn}$) cells release criteria and characterization. Feasibility measures will include the number of T^{tn}

and HSPC^{tn} manufacturing procedures successfully completed (i.e. complying with all release criteria), CD4+ and CD34+ purity, expression of various surface markers, and the number of target cells harvested.

2.2.2.Secondary objective(s)

The secondary objectives of the study are to assess the difference between the 3 treatment cohorts in:

- The extent of HSPC^{tn} contribution to hematopoiesis and T^{tn} survival by evaluation of Cal-1 marking and expression in peripheral blood at time points up to study completion or discontinuation.
- The extent of HSPC^{tn} contribution to hematopoiesis and T^{tn} survival by evaluation of Cal-1 marking and expression in gut-associated lymphoid tissue (GALT).
- The potential benefit of busulfan conditioning as determined by;
    - The extent of engraftment and differentiation of HSPC^{tn} over time by evaluation of Cal-1 marking and expression in peripheral blood subpopulations (monocytes, granulocytes, CD4+ and CD8+ lymphocytes)
    - The extent of HSPC^{tn} engraftment by evaluation of Cal-1 marking and expression in bone marrow at Week 12, 24, 48 and early discontinuation.
- The potential efficacy of Cal-1 in controlling HIV-1 infection for each subject, as measured by;
    - Plasma HIV-1 RNA relative to the Assessment for Comparison (as defined in section 3).
    - Plasma HIV-1 RNA over time
    - CD4+ T lymphocyte count, percentage and CD4+/CD8+ T lymphocyte ratio relative to the Assessment for Comparison (as defined in section 3).
    - CD4+ T lymphocyte count, percentage and CD4+/CD8+ T lymphocyte ratio over time
    - Time to commencement of ART

  The endpoint for these secondary analyses is study completion, withdrawal, or until discontinuation due to secondary analysis endpoint criteria.
- Impact on lymphocyte development for each subject, as measured by changes in thymopoiesis and maturation markers (Absolute and % Total Recent Thymic Emigrant) in peripheral blood relative to the Assessment for Comparison (as defined in section 3), and impact on chronic inflammation for each subject as measured by changes in inflammation markers (%CD4+ HLA-DR+ and Total CD4+ HLA-DR+2) in peripheral blood relative to the Assessment for Comparison (as defined in section 3) over time.
- A tropism shift from R5 to dual/mixed or X4 at any time point post-infusion.

2.3.Sample Size

This is a phase I/II study, and the sample size has not been based on formal power considerations. No recruitment limit is set for any of the participating sites, with the only restriction being consecutive enrollment that does not exceed 4 subjects per cohort.

Subjects in Cohorts 2 and 3 will not receive busulfan or be infused until the DSMB recommendations are received.

In terms of safety data, pooling all subjects across the three arms, the study will have at least an 80% probability of observing one or more serious adverse events if the true rate of these events in a subject is 12.6% or higher for 12 subjects, or ≥ 16.4% if 9 or more subjects. Pooling the busulfan subjects will give at least an 80% probability of observing one or more serious adverse events that occur with a true rate of 18.2% per subject for n=8, and 23.5% for n=6. Within any arm of four subjects, the study will have 80% probability of detecting events with a true rate of 33.1% per subject.

Hence the study will be well powered to detect only serious adverse events that occur with a reasonably high probability. The study will not be well powered to detect events that are rarer than about 10-20% per subject.

2.4.Randomization and Assignment of Subjects to Treatment Groups

This trial will not be randomized. Subjects will be consecutively enrolled into cohorts and each cohort will not exceed 4 subjects.

3.Statistical Considerations

Data will be handled and processed per the sponsor's representative (CCI) Standard Operating Procedures (SOPs), which are written based on the principles of good clinical practice (GCP).

All data collected on the electronic case report forms (eCRFs) will be presented in the data sorted by cohort, subject number and visit, where applicable. All summaries will present the data by cohort, and overall (total subjects), as applicable.

Unless otherwise stated, the following statistical approaches will be taken:

- Continuous variables: Descriptive statistics will include the number of non-missing values (N), mean, standard deviation (SD), median, minimum, maximum.
- Categorical variables: Descriptive statistics will include frequency counts and percentages per category. Percentages will be rounded to one decimal place, with the denominator being the number of subjects in the relevant population with non-missing data, unless otherwise specified.
- Imputation: No missing data will be imputed.
- Confidence intervals (CIs): CIs will be two-sided and will use 95% confidence levels, unless otherwise specified.
- Repeat assessments (Safety): On occasion, when the initial assessment does not yield a valid value), repeat assessments may be included in summary presentations (Tables, Figures). Original values will be used in summary presentations, unless otherwise noted. All assessments captured in the Electronic Data Capture (EDC) system will be presented in the data listings.
- Correlation coefficients: Pearson's product-moment correlation and Spearman's correlation coefficient will both be produced for all correlation analyses.
- One Sample t-tests: Student t-test and the signed rank test (non-parametric) will be used to calculate whether the change from assessment for comparison (as defined in section 3) in $\log_{10}$ plasma HIV-1 RNA and CD4 count is 0 at peak CD4/HIV-RNA and Week 48. P-values and 95% CIs for the mean and median change will be presented.
- Assessments for Comparison:
    - Cal1-Marking/Expression: All data will be compared to the pre-infusion value for Cohort 1 or the pre-busulfan value for Cohorts 2 & 3.
    - CD4+ Count and HIV-1-RNA will be compared with Screening (average of Screening 1 and Screening 2 values), the final pre-Apheresis visit, and pre-infusion (Cohort 1) / pre-busulfan (Cohorts 2 and 3).
    - ANC and Platelets will be compared with Screening (average of Screening 1 and Screening 2 values), the final pre-Apheresis visit, and the pre-infusion (Cohort 1) / pre-busulfan (Cohorts 2 and 3) visit.
    - CD3/CD4, CD3/CD8. CD4:CD8 ratio will be compared with Screening (average of Screening 1 and Screening 2 values), the final pre-Apheresis visit, and the pre-infusion (Cohort 1) / pre-busulfan (Cohorts 2 & 3) visit.
    - $\log_{10}$HIV-1 Ribonucleic acid (RNA) (Abbott and Roche assays) will be compared with Screening (average of Screening 1 and Screening 2 values), the final pre-Apheresis visit, and the pre-infusion (Cohort 1) / pre-busulfan (Cohorts 2 & 3) visit.

- Peak marking/expression data: Peak marking/expression data is the maximum data value post the start of the transduced cell infusion.
- Week 48 Assessment data: The week 48 assessment data will be the data collected at the Week 48 visit, or the last visit with recorded data prior to the Week 48 visit if no data is available at the Week 48 visit.
- Cell dose will be defined as follows:
    - CD4+: Total Number of CD4+ Cells (unit is $x10^8$) divided by weight, then converted to $x10^6$, and finally rounded to two decimal places.
    - CD34+: Total Number of CD34+ Cells (unit is $x10^8$) divided by weight, then converted to $x10^6$, and finally rounded to two decimal places.

For all marking/expression data, if a “Not Detectable Value” is recorded, the value will be set to 0 for analysis purposes.

In the summary tables and figures, treatment groups will be summarized separately for each dose cohort and overall (all subjects).

4.Analysis Populations

Two analysis populations are defined for this study, the Enrolled and the Safety populations. Data for Screen Failures will not be included in any summary tables, figures, or data listings.

Furthermore, any additional analysis populations not identified in the SAP will be identified in the final CSR as post hoc analyses. This may include the addition of additional study populations or subgroups of interest.

The analysis population each subject is included in will be listed.

4.1.Population Descriptions

4.1.1.Enrolled Population

All Subjects enrolled who have signed informed consent will be included in the enrolled population. Screening failures will not form part of this population. Subjects who are eligible but discontinue the trial before starting the intervention phase will only form part of the enrolled population.

4.1.2.Safety Population

The safety population will include all enrolled subjects have signed informed consent who started the interventional phase of the trial (i.e. CD4+ Apheresis).

5.Subject Disposition

5.1.Subject disposition

All subjects who provide informed consent (signed) and enrolled will be accounted for in this study. Subject disposition will be listed.

By-subject data listings for subject disposition will be generated, including informed consent date, study status (Ongoing, Discontinuation, Withdrawal or Completion), date of discontinuation, withdrawal or completion, reason for discontinuation or withdrawal, last schedule visit attended and inclusion into the enrolled and safety populations.

The time to starting ART from CD4+ Apheresis start will be summarized using Kaplan-Meier plots. If no antiretroviral treatment is commenced, the time period will be censored on the date of study completion/discontinuation.

The calculation will be done as follows: Date Antiretroviral Therapy Commenced / Date of Discontinuation, Withdrawal or Completion - CD4+ Apheresis Date + 1.

6.Protocol Deviations

Unless there is a safety concern, there should be no deviations or violations of the study protocol.

Overall protocol deviations (major and minor deviations combined) will be summarized by study visit by frequency counts and % of the total number of subjects per cohort.

In addition, major/minor protocol deviations reported, dependent on the number of events, will be summarized by cohort and overall by frequency counts and the % of the total deviations and/or listed for each subject in the by-subject data listings. The total number of events related to the protocol and GCP will also be represented.

Prior to database lock, all protocol deviations will be reviewed and assigned a status of Minor or Major.

Major/minor protocol deviations may include the following, depending on the timing and nature of the deviation:

- Non-compliance to study medication (i.e. subjects did not receive Investigational Product (IP) as per Clinical Study Protocol)
- Lost to follow-up (i.e. not all safety, pharmacokinetic (PK) data, efficacy data points recorded as per Clinical Study Protocol)
- Use of prohibited concomitant medication
- Violation of inclusion/exclusion criteria
- Deviation from study specific instructions
- If, for any reason, the cell infusions cannot proceed as planned, busulfan conditioning should be postponed and rescheduled for as soon as possible. A delay of up to 2 weeks will not be considered a protocol deviation

7.Demographic and Baseline Information

Demographic will be summarized using the Enrolled Population.

7.1.Demographics

Demographic data, including age, gender, race, ethnicity will be summarized by cohort and overall.

A by-subject data listing for demographic characteristics will be generated.

7.2.HIV-1 history

HIV-1 History (including lowest CD4+ T lymphocyte count (cells/µl) and highest plasma HIV-1 viral load (copies/mL) will be summarized by cohort and overall.

The date of HIV-1 diagnosis, lowest CD4+ T Lymphocyte count (cells/µl), the date of the, lowest CD4+ T lymphocyte count (cells/µl), highest plasma HIV-1 viral load (copies/mL) and the date of the highest plasma HIV-1 viral load (copies/mL) will be listed.

7.3.Medical history

Medical history will be presented in the by-subject data listings. Condition, affected body system, onset date, resolution date (ongoing) and planned treatment for the condition will be recorded.

7.4.Eligibility

Inclusion/exclusion eligibility criteria information, including evaluation date, criteria not met, will be listed for each subject. Eligibility will be listed for all screened subjects.

7.5.Chest X-Ray

Chest x-ray results will be listed by visit and cohort by subject. Abnormal chest x-ray results will be flagged.

7.6.ART Therapy History

History of ART therapy will be listed by subject and cohort. All drugs and associated doses will be listed per regimen. For each ART, details of each regimen (drug, start date, ongoing yes/no, stop date, reason for treatment discontinuation and lowest plasma HIV-1 viral load achieved during the regimen) will be listed.

8.Study Drug Administration

Study cellular product administration results will be presented using the Safety Population.

8.1.Transduced CD4+ and CD34+ Cell Product Dose

Transduced CD4+ and CD34+ Cell Product Dose (Transduction efficiency, viability and cell numbers for T^{tn} and $HSPC^{tn}$) data will be included in the by-subject data listings.

8.2.CD4+ and CD34+ Apheresis

CD4+ and CD34+ (including Granulocyte Colony-stimulating Factor (G-CSF) Day 4 & plerixafor and G-CSF Day 5) apheresis data (successful completion, filtration volume (L), number of CD4+ Lymphocytes Collected ($x10^8$ cells) and number of CD34+ Lymphocytes Collected ($x10^8$ cells) will be included in the by-subject data listings.

8.3.CD4+ and CD34+ Manufacturing

Feasibility measures such as the number of T^{tn} and $HSPC^{tn}$ manufacturing procedures successfully completed (i.e. complying with all release criteria); number of gene modified cells, as affected by CD4+ and CD34+ purity, transduction efficiency, and viability; and the number of target cells harvested will be summarized overall.

Several associations between feasibility and safety measures and efficacy endpoints will be investigated, including:

- Associations between the number of gene-modified cells infused, peripheral blood marking and expression, plasma HIV-1 RNA, CD4+ T cell counts
- Integration profile of the T^{tn} and $HSPC^{tn}$ products and observations in integration analysis of peripheral blood post infusion
- $HSPC^{tn}$ methylcellulose colony forming units and peripheral blood subpopulation marking

The CD4+ T Lymphocyte manufacturing and the CD34+ HSPC manufacturing process along with any deviation from the manufacturing process will be documented for each subject.

8.4.Mobilization Therapy

The administration of G-CSF with and without plerixafor therapy (administered yes/no, dose administered (µg/kg), total daily dose (µg), full dose administered (yes/no), date of administration will be included in the by-subject data listings.

8.5.Busulfan Administration and PK Data

For Cohorts 2 and 3, busulfan administration data will be listed. The listing will also be inclusive of busulfan AUC (µMol/min), concentration at steady state (ng/mL) and clearance (mL/min/Kg).

In addition, busulfan AUC and clearance data will be represented graphically. Busulfan concentrations will be plotted over time (hours post dose 1 & 2) and histograms will be created for the AUC and clearance by subject by visit.

9.Pharmacokinetics

Included as part of busulfan administration.

10.Pharmacodynamics (PD)

Not applicable to this study.

11.Efficacy

All efficacy summary analyses will be based on the safety population. Descriptive summaries will be presented with parameters to be correlated listed by subject by cohort by visit and overall adjacent to one another.

In addition to the descriptive statistics, Pearson's product-moment correlation and Spearman's correlation coefficient will both be produced for all correlation analyses and will be presented by cohort and overall.

Scatterplots of all correlated parameters by visit by cohort (different color markings will be used for each cohort) will also be presented on a subject level.

11.1.Cell Dose vs. Marking/Expression

The total number of infused CD4+ T^{tn} and CD34+ $HSPC^{tn}$ (Cell Dose) cells on Day 0 will be compared to the peak marking/expression data post transduced cell infusion and to the marking/expression at Week 48.

The marking/expression data that will be included are:

- Peripheral Blood:
  - Cal-1 Marking (WPRE qPCR) (%)
  - Cal-1 Marking (WPRE qPCR) (copies/cell)
  - Cal-1 C46 Expression (C46 RTqPCR) (relative expression)
  - Cal-1 sh5 Expression (sh5 RTqPCR) (relative expression)
- Peripheral Blood Subsets: monocytes, granulocytes, CD8+ lymphocytes, CD4+ lymphocytes):
  - Cal-1 Marking (WPRE qPCR) (copies/cell)
  - Cal-1 C46 Expression (C46 RTqPCR) (relative expression)
  - Cal-1 sh5 Expression (sh5 RTqPCR) (relative expression)
- Bone Marrow:
  - Cal-1 Marking (WPRE qPCR) (copies/cell)
  - Cal-1 C46 Expression (C46 RTqPCR) (relative expression)
  - Cal-1 sh5 Expression (sh5 RTqPCR) (relative expression)
- GALT (Location 1 & Location 2):
  - Cal-1 Marking (WPRE qPCR) (copies/cell)
  - Cal-1 C46 Expression (C46 RTqPCR) (relative expression)
  - Cal-1 sh5 Expression (sh5 RTqPCR) (relative expression)

Cal1-Marking/Expression will be listed by cohort by visit for each subject. This will include peripheral blood, bone marrow and GALT (both locations). The Cal-1 marking/Cal-1 C46 expression/Cal-1 sh5 expression will be presented by result, quantification, quantification relative expression and quantification % (peripheral blood and subsets (including population purity %)) only.

In addition to the figures (correlations) discussed above the following figures will also be produced:

- Line plot of Peripheral Blood Marking by week relative to the pre-infusion (Cohort 1) or pre-busulfan (Cohorts 2 & 3) value.
- Line plot of CD4 Marking by week relative to the pre-infusion (Cohort 1) or pre-busulfan (Cohorts 2 & 3) value.
- Line plot of CD8 Marking by week relative to the pre-infusion (Cohort 1) or pre-busulfan (Cohorts 2 & 3) value.

11.2.Busulfan AUC (Cohorts 2 and 3) vs. Marking/Expression

Busulfan AUCs on Day -2 (Cohort 2) and Days -4 and -2 (Cohort 3) will be compared to the peak marking/expression data and to the marking/expression data at Week 48.

The marking/expression data is as noted for section 11.1 above (Cell dose vs. Marking/Expression).

11.3.CD4+ and HIV-1RNA vs. Marking/Expression

CD4+ count and HIV-1RNA (Roche and Abbott assays) assessments to be compared as referenced in section 3 will be evaluated against the peak marking/expression data and to the marking/expression data at Week 48.

HIV-1 RNA nadir values (minimum Log10 HIV-1-RNA Roche and Abbott Assays value post the start of the transduced cell infusion) will be compared to the peak marking/expression data and to the marking/expression data at Week 48.

Further assessment of changes in CD4+_count and HIV-1 RNA (Roche and Abbott) over tine in comparison to marking/expression data, including sub-population may be performed post hoc of the final analysis.

The marking/expression data is as noted for section 11.1 above (Cell dose vs. Marking/Expression).

Changes in $\log_{10}$ plasma HIV-1 RNA and CD4+ count will be formally compared in all subjects using one-sample t-tests at the peak value and Week 48 (Student's t-test and the Signed rank test).

Exploratory analyses of $\log_{10}$ plasma HIV-1 RNA, lymphocytes (CD4+ T lymphocyte count, CD4+ %, CD4:CD8 ratio), measures of Cal-1 marking/expression and lymphocyte development (thymopoiesis, maturation and inflammation) may use repeated measures ANOVA techniques to explore changes in these endpoints over time, and any differences between treatment groups. These analyses are not well powered, and it is accepted they will be exploratory in nature and interpreted cautiously. This exploratory analysis will not be performed as part of this SAP.

11.4.Absolute Neutrophil Counts (ANC) and Platelets vs. Marking/Expression

ANC and Platelet nadir values (minimum ANC and Platelet values post the start of the transduced cell infusion) will be compared to the peak marking/expression data and to the marking/expression data at Week 48.

The marking/expression data is as noted for section 11.1 above (Cell dose vs. Marking/Expression).

For ANC and Platelets values, actual values and actual changes from the timepoints in section 3 will be presented at the timepoints noted above.

11.5.Lymphocyte Development and Chronic Inflammation vs. Marking/Expression

Lymphocyte development (thymopoiesis and maturation markers - Absolute and % Total Recent Thymic Emigrant in peripheral blood) and impact of chronic inflammation as measured by changes in inflammation markers CCI [redacted] in peripheral blood will be compared to the peak marking/expression data and to the marking/expression data at Week 48.

For inflammation markers, the average marker values of all timepoints (post the start of the transduced cell infusion) will be compared to the peak marking/expression data and to the marking/expression data at Week 48.

The marking/expression data is as noted for section 11.1 above (Cell dose vs. Marking/Expression).

12.Safety

Statistical methods for the safety analyses will be primarily descriptive in nature. Each cohort will be summarized separately and combined overall. No formal statistical comparisons will be made.

Safety endpoints include Adverse Events (AEs), clinical laboratory assessments (hematology, chemistry, lymphocyte phenotype, HIV-1 RNA), abnormal vital signs (pulse rate, systolic and diastolic blood pressure, temperature and oxygen saturation), abnormal physical examination, Cal-1 marking/expression data, (peripheral blood, bone marrow and GALT) and integration analysis (including re-test), replication competent lentivirus (RCL), HIV-1 Tropism, the use of concomitant medications and C46 Immunogenicity (Humoral and Cellular Response).

Safety endpoints will be analyzed using the safety population and will be based on actual treatment received.

12.1.Adverse Events

Adverse events (AEs) will be coded using the Medical Dictionary for Regulatory Activities (MedDRA®, Current), and data will be summarized by System Organ Class (SOC) and Preferred Term (PT). The number and percent of subjects reporting each AE will be summarized for each cohort and overall. A subject with two or more AEs within the same level of summarization (i.e., SOC or PT) will be counted only once in that level. Subjects will also be summarized by event severity and relationship to Cal-1 and study procedures (G-CSF, plerixafor or busulfan). Percentages will be based on the number of subjects in the safety population within each cohort and overall. The number of AEs reported will also be presented.

A by-subject AE data listing including verbatim term, MedDRA SOC and PT, severity, outcome, relationship to Cal-1, relationship to study procedures (e.g. G-CSF, plerixafor, busulfan, etc.), action taken and outcome will be provided. Separate listings will be generated for serious adverse events (SAEs), grade 3/4 events and grade 3/4 Cal-1 related and study procedures (G-CSF, plerixafor or busulfan) related events.

In addition, a listing of deaths will also be created. Date of death and autopsy findings will be included.

12.2.Concomitant medication

Concomitant medications are medications taken at least once after the start of CD4+ Apheresis. Medication name, dose, unit, frequency, route, start date, stop date, indication and status (continuing) will be listed.

Medications stopped prior to Apheresis will not be considered concomitant medication but will be listed as well.

12.3.Laboratory

All hematology, biochemistry, lymphocyte phenotype and HIV-1 RNA parameters will be listed by cohort for each subject.

Laboratory values will be compared to normal range of the single local laboratory and values that fall outside of the normal ranges will be flagged as: H (High) and L (Low) in the data listings and the clinical significance of the abnormal results noted.

In addition, separate listings will be created for all out of range hematology and biochemistry values.

Figures of the following will also be created:

- Line plot of post busulfan white blood cell (WBC) counts by cohort by subject over time (visit);
- Line plot of post busulfan ANC by cohort by subject over time (visit);
- Line plot of post busulfan Platelets by subject over time (visit);
- Line plot of %CD3/CD4 by cohort by subject over time (visit);
- Line plot of absolute CD3/CD4 by cohort by subject over time (visit);
- Line plot of absolute CD3/CD8 by cohort by subject over time (visit);
- Line plot of CD4:CD8 ratio by cohort by subject over time (visit);
- Line plot of HIV-1 Ribonucleic (RNA) (Abbott and Roche assays) by cohort by subject over time (visit);
- Line plot of log_{10}HIV-1-RNA (Abbott and Roche assays) by cohort by subject over time (visit). In addition, all subjects will be presented on a single page for all cohorts to further assess the time to viral load suppression of Cal-1. Time of peripheral blood gene marking and ART recommencement will be added to the figure for each subject.

12.4.Vital Signs

Actual values for vital signs (Systolic Blood Pressure (SBP) (mmHg), Diastolic Blood Pressure (DBP) (mmHg), Pulse Rate (beats/min), Temperature (°C) and Oxygen Saturation (%)) will be listed by cohort by visit/timepoint for each subject.

In addition, abnormal vital sign values for all parameters will be listed by cohort by visit/timepoint for each subject. Vital sign values will be compared to normal range and values that fall outside of the normal ranges will be presented.

12.5.Physical Examination

Physical examination values will be listed by cohort by visit/timepoint for each subject.

Additionally, abnormal physical examination values and clinical significance associated with the abnormal values will also be presented by cohort by visit/timepoint for each subject.

12.6.Pregnancy Test

Pregnancy results will be listed by visit and cohort by subject. For positive pregnancy tests, further laboratory results and co-infection values will be presented.

12.7.Other Safety

RCL (including re-test) and HIV-1 Tropism Assay will be listed by cohort by visit for each subject. Integration analysis (including re-test values), will be listed by cohort by visit/timepoint for each subject.

13.Immunogenicity Endpoints

Immunogenicity endpoints will be analyzed using the safety population and will be based on actual treatment received.

C46 Immunogenicity (Humoral Response and Cellular Response) will be listed by cohort by visit for each subject.

14.Handling of Missing Data

Missing values will generally not be imputed for all data included in the analyses.

15.Changes to the Planned Analysis

Changes to the study design that occurred during the life cycle of the trial will be further described in an Appendix (Appendix 1) of the SAP detailing all protocol and design changes

16.Interim and Final Analysis

16.1.Medical Review Committee (MRC)

A medical review committee will be established to ensure that the safety of subjects participating in the study is not compromised. The MRC will include, but will not be limited to, at least two expert physicians in the area of HIV medicine and/or hematology. The MRC will include members from the Sponsor and external consultants. The MRC will monitor the safety of the study by reviewing AE/SAE, clinical, and laboratory data in a regular and timely manner. Any grade three or four toxicities will be comprehensively reviewed and the MRC will be required to make formal recommendations. Specific recommendations concerning the dose of busulfan will be made by the MRC following assessment of post-conditioning hematopoietic recovery. Delayed hematopoietic recovery is defined as an ANC < $0.5x10^3$/μL (mm^3) and/or platelet count of < $25x10^3$/μL (mm^3) after 42 days' post-infusion, and observation of this adverse event in any subject will prompt a reduction in the busulfan dose. The MRC will communicate any occurrence of delayed hematopoietic recovery to the DSMB in real-time.

The MRC will assess the hematopoietic recovery of every subject, and following completion of week 12 make formal recommendation if the back-up apheresis product is no longer required. With the agreement of the Principal Investigator, these samples may then be destroyed, or donated to research, in accordance with the subject's wishes at time of study consent.

The MRC will also be responsible for the review and/or input into the case narratives for Serious and Unexpected Suspected Adverse Reactions (SUSARs), including adjudication of possible causality to Cal-1 or study procedures.

Details of the composition, roles, responsibilities, and relationship with the DSMB are documented in the MRC charter.

16.2.Data Safety Monitoring Board (DSMB) Analyses

An independent DSMB will be commissioned for this study to evaluate the risk/benefit of busulfan and to review all safety and selected efficacy (such as the degree of Cal-1 marking in peripheral blood, GALT and bone marrow) data from cohorts 1 and 2 prior to proceeding with the next planned cohort. Progression to each cohort is dependent on the positive recommendation of the DSMB. Details of the composition, roles, responsibilities and timelines will be documented in the DSMB charter.

There are no formal statistical stopping rules, however the DSMB will have the responsibility for independently evaluating safety on the bases of there being no evidence of safety concern(s) to warrant study cessation. The occurrence of bone marrow failure (defined as a protracted neutropenia (ANC < $0.5x10^3$/μL (mm^3)) and/or thrombocytopenia (platelets < $25x10^3$/μL (mm^3)) after Week 12 post-infusion) will trigger a temporary hold on the administration of busulfan and subject enrolment pending a safety assessment by the DSMB. The occurrence of grade 4 laboratory abnormalities, unanticipated incidence, severity or frequency of AE/SAEs, or failure of T^{tn}/$HSPC^{tn}$ manufacturing processes may also be events that could present sufficient safety concerns to warrant study cessation. Following completion of the safety data review, the DSMB may also recommend that the study be modified to improve monitoring of safety, additional subjects be enrolled to assess safety, or the study be terminated for safety reasons.

In addition to scheduled review of Cohort data, the DSMB chairperson will receive a copy of all MRC recommendations and all SAE reports that have possible suspect causality to Cal-1 and/or the study procedures in real time (i.e. at the same time as the MRC). The Chairperson is responsible for determining if a special meeting of the DSMB is required to review any event in further detail and make a recommendation between cohorts. Recommendations will be submitted to the Sponsor and authorities as required, according to local guidelines and regulations.

There are two planned DSMB safety analyses and one preliminary analysis for safety and secondary endpoints at Week 24. The independent DSMB will conduct a complete review of data to Week 12 for at least 3 subjects in the preceding Cohort before subjects in Cohorts 2 and 3 receive any busulfan and are infused. At each analysis, all subjects' safety data will be summarized including all available data up to the time the analysis is conducted. Measures of Cal-1 marking/expression will also be summarized. There will be no formal statistical stopping rules, rather the study will continue on the basis of there being no evidence of sufficient safety concerns to warrant study cessation. The occurrence of bone marrow failure (defined as a protracted neutropenia (ANC < $0.5x10^3/\mu L$ (mm^3)) and/or thrombocytopenia (platelets < $25x10^3/\mu L$ (mm^3)) after Week 12 post-infusion), the occurrence of grade 4 laboratory abnormalities, unanticipated incidence, severity or frequency of AE/SAEs, or failure of T^{tn}/$HSPC^{tn}$ manufacturing processes may be events that could present sufficient safety concerns to warrant study cessation. Efficacy indicators, such as the degree of Cal-1 marking in peripheral blood, GALT and bone marrow will also be assessed.

16.3.Interim Analyses

A formal preliminary analysis was planned to be conducted once all subjects completed 24 weeks follow-up. No formal preliminary analysis was conducted by CCI at Calimmune's request. Safety and efficacy data review was conducted by the MRC for all subjects who completed 24 weeks follow up and beyond.

16.4.Final Analysis (End of Study).

Formal final analyses will be performed at the end of the study generating Statistical Analysis System (SAS) tables, figures and listings for use in drafting the CSR as described in the SAP. These will be considered the final analyses.

The final end of study analysis (i.e. results post lock of the data base) will be based on the final version of the SAP.

17.Software

The following software will be used to perform the statistical analyses: SAS® Version 9.4 or higher (SAS Institute, Cary, North Carolina, USA).

18.Tables

No.	Title	Analysis Population
1	Protocol Deviations	All Enrolled
2.1	Protocol Deviations: Major Deviations Summary	All Enrolled
2.2	Protocol Deviations: Minor Deviations Summary	All Enrolled
3	Demographic and Baseline Information	All Enrolled
4	HIV-1 History	All Enrolled
5	Manufacturing CD4+ – Cohorts 1 and 2 and 3	Safety
6	Manufacturing CD34+ – Cohorts 1 and 2 and 3	Safety
7	Manufacturing Deviations	Safety
8	Cell Dose (x10e8) at Day 0 vs. Marking/Expression Data	Safety
9	Busulfan AUC (uMol/min) vs. Marking/Expression Data	Safety
10.1	CD4+ Count (mm^3)	Safety
10.2	CD4+ Count mm^3) vs. Marking/Expression Data	Safety
10.3.1	HIV-1-RNA Roche Assay (copies/mL)	Safety
10.3.2	HIV-1-RNA Abbott Assay (copies/mL)	Safety
10.4.1	HIV-1-RNA Roche Assay (copies/mL) vs. Peak Marking/Expression Data	Safety
10.4.2	HIV-1-RNA Abbot Assay (copies/mL) vs. Peak Marking/Expression Data	Safety
11.1	ANC (10^3/uL) vs. Peak Marking/Expression Data	Safety
11.2	Platelets (10^3/uL) vs. Peak Marking/Expression Data	Safety
12	Lymphocyte Development and Chronic Inflammation vs. Marking/Expression	Safety
13.1	Adverse Events (Summary SOC, PT)	Safety
13.2	Adverse Events by CTC grade (Severity)	Safety
13.3	Treatment Emergent Adverse Events by Relationship to Study Drug (Causality)	Safety
13.4	Adverse Events by Relationship to Study Procedure (Causality)	Safety

19.Listings

No.	Title	Analysis Population
1	Subject Disposition	All Enrolled
2	Subject Withdrawal	All Enrolled
3	Subject Discontinuation	All Enrolled
4.1	Demographics	All Enrolled
4.2	Pre-Busulfan Body Weight	All Enrolled
5	HIV-1 History	All Enrolled
6	Medical History	All Enrolled
7	Eligibility Criteria	All Enrolled
8.1	Pregnancy Test	All Enrolled
8.2	Positive Pregnancy Test	All Enrolled
9	Chest X-Rays	All Enrolled
10	ART Therapy History (regimen overview)	All Enrolled
11	Transduced CD4+ Cell Product Dose (Transduction efficiency, viability, cell numbers for T^{tn})	All Enrolled
12	Transduced CD34+ Cell Product Dose (Transduction efficiency, viability, cell numbers for $HSPC^{tn}$)	All Enrolled
13.1	Plerixafor Therapy	All Enrolled
13.2	G-CSF Therapy	All Enrolled
14.1	Apheresis – CD4+	All Enrolled
14.2	Apheresis – CD34+	All Enrolled
15.1	Busulfan Dosing	
15.2	Busulfan Administration and Pharmacokinetics	All Enrolled
16	Cal-1 Marking/Expression - Peripheral Blood	All Enrolled
17	Cal-1 Marking/Expression - Bone Marrow	All Enrolled
18	Cal-1 Marking/Expression – GALT	All Enrolled
19.1	Cal-1 Marking/Expression – Subsets: Monocytes	All Enrolled
19.2	Cal-1 Marking/Expression – Subsets: Granulocytes	All Enrolled
19.3	Cal-1 Marking/Expression – Subsets: CD4+ Lymphocytes	All Enrolled
19.4	Cal-1 Marking/Expression – Subsets: CD8+ Lymphocytes	All Enrolled
20.1	Serious Adverse Events, including event narratives	All Enrolled
20.2	Deaths	All Enrolled
21.1	Adverse Events (All Grades)	All Enrolled
21.2	Adverse Events (Grades 3 and /or 4)	All Enrolled
21.3	Related Adverse Events (Grades 3 and /or 4)	All Enrolled
22.1	Concomitant Medication	All Enrolled
22.2	Concomitant Procedures and Therapies	All Enrolled
23.1	Laboratory – Hematology	All Enrolled
23.2	Laboratory – Hematology (Abnormal only)	All Enrolled
24.1	Laboratory – Biochemistry	All Enrolled
24.2	Laboratory – Biochemistry (Abnormal only)	All Enrolled
25.1	Laboratory – Lymphocyte phenotype	All Enrolled
25.2	Laboratory – Thymopoieisis	All Enrolled
25.3	Laboratory – Inflammation	All Enrolled
25.4	Laboratory – Maturation	All Enrolled
26	Laboratory – HIV-1 RNA	All Enrolled
27.1	Vital Signs	All Enrolled

No.	Title	Analysis Population
27.2	Abnormal Vital Signs	All Enrolled
28.1	Physical Examination	All Enrolled
28.2	Abnormal Physical Examination	All Enrolled
29.1	Cal-1 Integration Analysis	All Enrolled
29.2	Cal-1 Integration Analysis (Re-test)	All Enrolled
30	Replication Competent Lentivirus (RCL)	All Enrolled
31	HIV-1 Tropism Assay	All Enrolled
32.1	C46 Immunogenicity – Humoral Response	All Enrolled
32.2	C46 Immunogenicity – Cellular Response	All Enrolled

20.Figures

No.	**Title**	**Analysis Population**
1.1.1	T^{tn} (CD4+) Cell Dose at Day 0 vs. Peak Marking/Expression Data Protocol: CAL-USA-11	Safety
1.1.2	T^{tn} (CD4+) Cell Dose at Day 0 vs. Week 48 Marking/Expression Data	Safety
1.2.1	$HSPC^{tn}$ (CD34+) Cell Dose at Day 0 vs. Peak Marking/Expression Data	Safety
1.2.2	$HSPC^{tn}$ (CD34+) Cell Dose at Day 0 vs. Week 48 Marking/Expression Data	Safety
2.1.1	Busulfan AUC (uMol/min) at Day -4 vs. Peak Marking/Expression	Safety
2.1.2	Busulfan AUC (uMol/min) at Day -4 vs. Week 48 Marking/Expression Data	Safety
2.2.1	Busulfan AUC (uMol/min) at Day -2 vs. Peak Marking/Expression	Safety
2.2.2	Busulfan AUC (uMol/min) at Day -2 vs. Week 48 Marking/Expression Data	Safety
3.1.1	CD4+ Count (mm^3) at Screening vs. Peak Marking/Expression Data	Safety
3.1.2	CD4+ Count (mm3) at pre-Apheresis vs. Peak Marking/Expression Data	Safety
3.1.3	CD4+ Count (mm^3) at pre-infusion (Cohort 1) /pre-busulfan (Cohorts 2 and 3) vs. Peak Marking/Expression Data	Safety
3.1.4	Peak CD4+ Count (mm^3) change from Screening vs. Peak Marking/Expression Data	Safety
3.1.5	Peak CD4+ Count (mm^3) change from pre-Apheresis vs. Peak Marking/Expression Data	Safety
3.1.6	Peak CD4+ Count (mm^3) change from pre-infusion (Cohort 1) /pre-busulfan (Cohorts 2 and 3) vs. Peak Marking/Expression Data	Safety
3.1.7	Week 48 CD4+ Count (mm^3) change from Screening vs. Peak Marking/Expression Data	Safety
3.1.8	Week 48 CD4+ Count (mm^3) change from pre-Apheresis vs. Peak Marking/Expression Data	Safety
3.1.9	Week 48 CD4+ Count (mm^3) change from pre-infusion (Cohort 1) /pre-busulfan (Cohorts 2 and 3) vs. Peak Marking/Expression Data	Safety
3.2.1 – 3.2.9	As per 3.1.1 – 3.1.9 for Week 48 Marking/Expression Data	Safety
3.3.1 – 3.3.9	As per 3.1.1 – 3.1.9 for HIV-1-RNA (copies/mL) vs. Peak Marking/Expression Data	Safety
3.4.1 – 3.4.9	As per 3.1.1 – 3.1.9 for HIV-1-RNA (copies/mL) vs. Week 48 Marking/Expression Data	Safety
4.1.1 – 4.1.9	As per 3.1.1 – 3.1.9 for nadir ANC (10^3/uL) vs. Peak Marking/Expression Data	Safety
4.2.1 – 4.2.9	As per 3.1.1 – 3.1.9 for nadir ANC (10^3/uL) vs. Week 48 Marking/Expression Data	Safety
4.3.1 – 4.3.9	As per 3.1.1 – 3.1.9 for Platelets (10^3/uL) vs. Peak Marking/Expression Data	Safety

No.	**Title**	**Analysis Population**
4.4.1 – 4.4.9	As per 3.1.1 – 3.1.9 for Platelets (10^3/uL) vs. Week 48 Marking/Expression Data	Safety
5.1.1 – 5.1.9	As per 3.1.1 – 3.1.9 for Lymphocyte Development and Chronic Inflammation vs. Peak Marking/Expression Data	Safety
5.2.1 – 5.2.9	As per 3.1.1 – 3.1.9 for Lymphocyte Development and Chronic Inflammation vs. Week 48 Marking/Expression Data	Safety
6	Busulfan Pharmacokinetics, AUC and Clearance Rate	Safety
7.1	Post busulfan – WBC (10^3/uL)	Safety
7.2	Post busulfan – ANC (10^3/uL)	Safety
7.3	Post busulfan – Platelets (10^3/uL)	Safety
8.1 – 8.2	%CD3/CD4 by Cohort	Safety
8.3 – 8.4	%CD3/CD8 by Cohort	Safety
8.5 – 8.6	Absolute CD3/CD4 by Cohort	Safety
8.7 – 8.8	Absolute CD3/CD8 by Cohort	Safety
8.9 – 8.10	CD4:CD8 Ratio by Cohort	Safety
9.1 – 9.8	HIV-1 RNA (copies/mL) by Subject	Safety
9.9 – 9.10	HIV-1 RNA (copies/mL) Roche $\log_{10}$ by Cohort	Safety
10.1 – 10.2	Peripheral Blood Marking by Cohort	Safety
10.3 – 10.4	CD4 Marking by Cohort	Safety
10.5 – 10.6	CD8 Marking by Cohort	Safety
10.7 – 10.8	Granulocytes by Cohort	Safety
10.9 – 10.10	Monocytes Marking by Cohort	Safety
10.11 – 10.12	Bone Marrow Marking by Cohort	Safety
10.13 – 10.14	GALT Marking (Location 1) by Cohort	Safety
10.15 – 10.16	GALT Marking (Location 2) by Cohort	Safety
11	Kaplan Meier Plot of Time to ART start	Safety

21.References

1. Clinical Study Protocol Version 8, dated 08 JUL 2015.

22.Appendix I: Summary of Protocol Changes

CAL-USA-11 PROTOCOL VERSION 3.0 (dated 12 December 2013) – SUMMARY OF CHANGES

Protocol Section	Nature of Change	Rationale
Contact information	Update	
Glossary of terms	Update/addition	New terms added. Changed SUA to SUSAR.
Synopsis	Addition	Added clinical trials.gov registration number Added participating countries (USA).
	Update	All units for laboratory values were updated to ISO (conventional) format for consistency.
Schedule of Events	Modification & addition	Separated tables to pre-infusion and post-infusion Pre-infusion: 1. Added option pre-screening to help facilitate selection of suitable potential subjects and reduce the screen-fail rate 2. Separated consent procedure to ensure consent obtained prior to pre-screening 3. Increased window period for Screening 1 visit to assist sites in the consenting and scheduling of concurrent subjects for screening 4. Moved co-infection screening to Screening 1 due to better accommodate assay turn-around times and the Screening 2 window period 5. Added busulfan PK monitoring in response to FDA and DSMB request 6. Increased the window between CD34 apheresis and Baseline visit to better accommodate the turn-around time for product release testing Post-infusion: 1. Added 'Conditioning F/U' visits at week 0.5, 1.5, 2.5, 3, 3.5, 4.5, 5, and 5.5 time points to increase intensity of post-conditioning and transplant subject safety monitoring in response to FDA request and in line with DSMB recommendations Footnotes: 1. Updated in line with above changes and change to lymphocyte phenotyping studies (see section 7.5). 2. Added unscheduled HIV-1 tropism assessment if subject discontinues the study post-infusion to recommence ART (see section 6.2.4) 3. Added stored EDTA-plasma sample for HIV viral load batch analysis (see section 7.5).

Protocol Section	Nature of Change	Rationale
1.1 Background	Addition	In vivo studies added to section 1.1.1. Additional references cited section 1.1.2 literature update. Additional references cited section 1.1.2 (busulfan) regarding the use of low and intermediate doses of busulfan for non-myeloablative and reduced-intensity conditioning effects.
3. Objectives and Outcome measures/endpoints	Modification	Update in line with changes to lymphocyte phenotyping studies (per section 7.3).
4.Overview of study design	Modification	Updated timeline schematic in line with changes to the pre-infusion schedule of events. Updated DSMB recommendations to include Cohort 3 proceeding with an increased dose of 6 to 8mg/kg busulfan (See section 6.2.2)
5.2 Exclusion criteria	Modification	Ex-A updated to be in ISO units Ex-N modified to specify venous assessment prior to Screening1, to allow flexibility in scheduling.
5.4 Rescreening	Modification	Re-screening procedures were adjusted in line with the changes to Screening 1 and Screening 2 procedures.
6.1.3 Dosage and administration	Modification	The order of infusion was changed to administer the HSPC^{tn} first. This was a safety consideration, as an adverse reaction to the infusion products is potentially more likely with the T^{tn}. HSPC^{tn} are the priority infusion for subjects who have received busulfan conditioning. The order of infusion for Cohort 1 subjects was T^{tn}, followed by HSPC^{tn}.

Protocol Section	Nature of Change	Rationale
6.2.2 Busulfan	Modification	The dosing range for busulfan was modified to include 6mg/kg within the 'moderate dose' cohort (III). Dose adjustment rules, based on observation of delayed hematopoietic recovery were added. These changes are an additional safety precaution to ensure the dose of busulfan does not result in a more severe myeloablation than intended. The description of the busulfan dosing was corresponding updated throughout the protocol. As a safety precaution to ensure there would be sufficient CD34+ cells to 'rescue' a subject in the event of busulfan-induced myeloablation, a clause was added that busulfan will not be administered if the minimum anticipated HSPC^{tn} dose ($0.5x10^8$ total CD34+ cells) is not achieved. If cell infusion is postponed, the administration of busulfan will also be postponed. Busulfan will be given 2 days prior to the revised planned cell infusion date (baseline visit) with the contingent that all release criteria were met.
6.2.3 Post-busulfan infection prophylaxis	Addition	New section added to include specification on the post-conditioning infection prophylaxis regimen. Details of agents, doses and duration are provided.
6.2.4 Antiretroviral Therapy	Addition	HIV-1 tropism will also be performed if a subject is required to recommence ART post-infusion.
7.1.1 Consent	Addition	New section created to align with the re-design of the schedule of events. Details are provided on the permitted timing of study consent.
7.1.2 Pre-screening	Addition	New section created to align with the re-design of the schedule of events. The pre-screening visit is an optional visit included to assist sites in the selection of potential suitable subjects to participate. Consent requirements, optional visit procedures/laboratory tests and timing are described.

Protocol Section	Nature of Change	Rationale
7.1.3 Screening 1	Modification	A screen fail rate of > 50% was experienced with Cohort 1. Screen failures present a risk and inconvenience to study subjects and also increase study timelines. The timing of Screening has been adjusted to allow a maximum 3 month interval between consent and Screening 1 (increase from 2 months) to allow sites more time to obtain and evaluate pre-existing medical history as part of eligibility and suitability determination. Procedures were revised to include the co-infection screening and chest x-ray (moved from Screening 2). This was to simplify the screening 2 procedures, to minimise the risk of late notice screen fails and therefore reduce impact on both the individual subjects and the manufacturing facility preparation. A window period was added for the venous assessment, to allow this to be performed any time between consent and Screening 1. This will improve subject convenience, and also enable assessment of this eligibility criterion before the more invasive assessments are performed. Medical history instructions were expanded to specify collection of vaccination, drug allergy and recurrent infection history, in line with standard bone marrow transplant procedures
7.1.4 Screening 2	Modification	Window period modified to be within 1-2 weeks of CD4 apheresis. Window between Screening 1 and Screening 2 modified to 2-12 weeks to allow concurrent screening (within limitations specified in Section 12.1). Visit laboratory tests simplified to confirmation of HIV-1 RNA and CD4+ eligibility criteria. Chest x-ray and co-infection screening moved to Screening 1 because of assay turn-around times.
7.1.5 CD4+ T lymphocyte apheresis	Modification & addition	Added subject's weight to be measured and provided to the manufacturing lab to enable calculation of the anticipated dose for the finished T^{tn} product. CCI [redacted] Removed description of tests to be performed on the collected apheresis product, as these are manufacturing-specific procedures.

Protocol Section	Nature of Change	Rationale
7.1.7 CD34 + apheresis	Modification & addition	Added subject's weight to be measured and provided to the manufacturing lab to enable calculation of the anticipated dose for the finished HSPC^{tn} product CCI [redacted] Removed description of tests to be performed on the collected apheresis product, as these are manufacturing-specific procedures.
7.1.8 Administration of busulfan	Modification & addition	Added stipulation that this visit can only occur after confirmation that the T^{tn} and HSPC^{tn} products have been released and are available on site Clarified the timing of each busulfan dose to be in the morning Added instruction in the event of a delay in the planned cell infusion (Baseline) visit Added specification for collection of blood samples and batch testing for busulfan PK monitoring.
7.1.9 Baseline (day of infusion)	Modification	Removed the provision to postpone the infusion if busulfan conditioning has occurred CCI [redacted]
7.2.1 Conditioning follow up	Addition	New section to describe the content and timing of additional visits to increase intensity of post-conditioning and cell-infusion safety monitoring. Both laboratory and clinical monitoring is specified Extended or more frequent safety follow up is permitted if clinically indicated for any study subject.
7.2.2 Weeks 1, 2, 6, 8, 16 & 20	Addition	Provision to perform a local STAT CBC/differential has been added to Weeks 1, 2 and 6, in line with conditioning follow up visits in section 7.2.1 Safety limits are provided, along with appropriate steps to ensure subjects are further evaluated and – as necessary – treated by an experienced, accredited transplant center.
7.2.3 Week 4	Addition	Provision to perform a local STAT CBC/differential has been added, in line with conditioning follow up visits in section 7.2.1

Protocol Section	Nature of Change	Rationale
7.3 Safety evaluations	Modification & addition	C46 immunogenicity studies were moved from exploratory analysis (Section 7.6) to safety, in line with FDA request to perform these studies as part of the Cal-1 safety data collection.
7.5 Efficacy evaluations	Modification & addition	Lymphocyte phenotyping studies have been added and grouped under the heading of 'Lymphocyte development studies'. In addition to the thymopoiesis analysis, these now include 'maturation' and 'inflammation' to expand potential data on the impact of Cal-1 on lymphocyte dynamics. An additional baseline time point at the CD4+ apheresis visit was added to obtain baseline prior to apheresis and G-CSF mobilization. Potential cross-reactivity with Cal-1 in HIV-1 viral load assays has been identified as an area for further laboratory investigation. The HIV-1 RNA plasma assay details have been updated with the addition of a stored EDTA-plasma sample for the purposes of batch analysis with an alternative FDA approved plasma HIV-1 RNA assay, pending the outcome of these investigations. Corresponding updates to the Schedule of Events, visit-specific procedures and statistical analysis have been made for consistency throughout the protocol.
7.6 Exploratory evaluations	Modification & addition	CCI [redacted]
7.7 Specimen storage	Modification	Storage of blood and tissue samples was previously described within the exploratory evaluations section. This has been moved into a separate section for clarity. Priority of use of the stored specimens has been clarified.
10.1.4	Modification	The term Serious Unexpected Associated (SUA) Adverse Event was replaced with Serious & Unexpected Suspected Adverse Reaction (SUSAR) to be consistent with regulatory guidance and other study-related safety operational documentation (such as the DSMB charter and safety management plan) The term SUSAR replaces SUA throughout the protocol for consistency.

Protocol Section	Nature of Change	Rationale
11.2 Ethical and regulatory information	Addition	Details of the IND # and clinicaltrials.gov registration were added DSMB recommendation was added to the list of informational study updates to be provided to investigators.
11.5 Privacy and confidentiality	Addition	Reference to adherence to HIPPA regulations was added.
12.1 Subject screening and enrolment	Addition	Provision has been added to allow the concurrent screening of subjects in order to minimize the potential negative impact of screen failures on the participating subjects, sites and manufacturing schedules. The total number of subjects in screening at any time cannot exceed the number of available places within the cohort. Details on the timing and scheduling of subject visits is addressed. Consequent changes to the schedule of events and visit specific information are addressed throughout the protocol for consistency.
12.7.1 MRC	Addition	Specification was added for the MRC review of hematopoietic recovery post busulfan and for the MRC to document their recommendation concerning an adjustment of the busulfan dose within the range specified.
12.7.2 DSMB	Addition	Bone marrow failure has been defined by hematologic parameters and added as a stopping rule in addition to the events previously listed. Provision is also added for the DSMB chairperson to receive copies of MRC recommendations in real-time, and scope added to convene a special meeting of the DSMB and recommendations to be made between formal cohort reviews.
12.10.1 Sources of funding and disclosure of financial interest	Modification	Section updated to include information on source of Calimmune financial support, including disclosure of a CIRM grant.
Appendix I	Modification	In order ensure up to date information, the DAIDS grading tables have been deleted and replaced with a link to the on line published document. The current version is December 2004, with clarifications dated August 2009. Specific guidance for values below Grade 1 have been added, in line with the DAIDS instructions.

Protocol Section	Nature of Change	Rationale
Appendix III	Modification & addition	CCI [redacted]
Appendix IV	Modification	Section 3 - updated with details on prophylactic medications, dose and timing - flow rate reduced to deliver total dose over 4 hours Section 5 updated with medications list.
Appendix V	Modification & addition	Specific apheresis collection parameters provided for both CD4+ and CD34+ collections.

CAL-USA-11 PROTOCOL VERSION 4.0 (dated 22 March 2014) – SUMMARY OF CHANGES

Protocol Section	Nature of Change	Rationale
Schedule of events	Removed footnotes referring to 4-week acute and 12-week sub-acute data time points	Original footnotes were potentially misleading. It is intended that data inclusive to Week 12 (not only weeks 4 and 12) to be presented to the DSMB.
	Busulfan PK monitoring footnote revised	Details on PK sampling now described in Appendix IV. Additionally, it was clarified that for Cohort 3, the busulfan dosing will start on Day -3 (and therefore completing at the same time relative to infusion as Cohort 1). Corresponding updates to text made throughout the document to correct references to Day -2 and Day-3
	Additional column for busulfan (Day -3)	Additional column added to separate the 2 days of busulfan and highlight the difference between cohorts 2 and 3
	Footnote 18 (unscheduled HIV-1 tropism) deleted	Unscheduled HIV-1 tropism no longer to be performed. Footnote 19 (Plasma HIV-1 RNA) renumbered to 18
	Additional visits: Week 28, 36 & 44	Additional visits in the final 12 weeks of the follow up period have been added so the frequency of follow up remains 4-weekly. The rationale for this change is that ongoing monthly follow up intervals allows closer follow up of HIV-1 viral load and CD4+ T-cell counts.
3. Objectives & Outcome measure/Endpoints	Secondary efficacy data description modified	Format change to better describe evaluation of lymphocyte inflammation studies (separate bullet point from thymopoiesis and maturation). Various text updates then required throughout for consistency.
4.1 Overview	Minimum number of subjects per cohort reduced from 4 to 3. DSMB review consequently will proceed on data from 3 subjects, with an option for a fourth subject if requested.	There has been a higher than expected screen failure rate (70%) and correspondingly an overall delay in study timelines. The original subject numbers were not based on formal power considerations, and no major safety concerns have presented to date. Thus, the change in sample size is being made in order to restore study timelines, yet not significantly impacting the probability of obtaining meaningful safety data from the study. Various text updates then required throughout for consistency.

Protocol Section	Nature of Change	Rationale
5.1 General Considerations	Timing for commencement of each subsequent cohort revised	Screening for the next cohort will not commence until after the data for 3 subjects to Week 12 of post-infusion follow up is available for the DSMB review.
6.2.2 Busulfan / 12.7.1 MRC / 12.7.2 DSMB	Text changes for consistency.	Busulfan dose adjustment and temporary hold on further dosing had been included in the protocol with version 3.0 changes. There were however small inconsistencies between the relevant sections that have now been addressed, and both dose adjustment or a hold on dosing are possible options for the MRC and DSMB in the event delayed hematopoietic recovery or/and bone marrow failure is observed with any subject.
6.2.4 Antiretroviral Therapy	Specific instructions added with respect to the confirmatory re-test in the event that s subjects HIV-1 viral load exceeds the specified safety limit.	Cross-reactivity with the Cal-1 vector has been demonstrated in the Roche COBAS® AmpliPrep/COBAS® TaqMan® HIV-1 RNA PCR assay. Because this cross-reactivity has the potential to artificially elevate viral load results, the confirmatory assay is to be performed with an alternative HIV-1 assay (Abbott RealTime m2000), which does not appear to cross-react with the Cal-1 vector. To ensure accurate interpretation of results, complete batch analysis of all time points is specified in the event a confirmatory re-test is required. Abbott RealTime m2000 batch data will be used for formal analyses.
7.1.8 Administration of Busulfan	Details of PK sampling removed	Details on sampling have been removed from this section and now described in Appendix IV.
7.3 Safety Evaluations	Reference to RCL biological assay removed	As both Cal-1 vector and the disease indication are a lentivirus, biological RCL assay is not technically feasible.
7.5 Efficacy Evaluations	Details of cross-reactivity updated, with specific alternative assay added Further details on Cal-1 marking &expression added	Cross reactivity with Cal-1 has been confirmed and the Abbott RealTime m2000 assay has been identified as a suitable alternate assay for batch analysis. Clarified the PCR targets for monitoring of Cal-1 presence and activity

Protocol Section	Nature of Change	Rationale
8. Subject Withdrawal/Discontinuation	Definition of discontinuation and withdrawal clarified	Discontinuation has been clarified as referring to the population of subjects that are discontinued from secondary analysis due to commencement of ART or other prohibited concomitant medication. These subjects continue in safety follow up. Withdrawal has been clarified as referring to the population of subjects that have ceased study participation. Various text updates then required throughout for consistency, particularly section 9.1.
9. Statistical Methods	Power calculations updated with new subject numbers	Changes to text within the Safety and Key Efficacy sections made to include power calculations for 3 subjects per cohort as well as 4
9.1 Statistical Analysis	Clarification on which assay data to be used in formal analyses	Formal statistical analysis will be performed using the batch data from the Abbott assay.
12.7.2 DSMB	Clarification added regarding data for DSMB review and scope of recommendations	Removed reference to acute and sub-acute time points (per Schedule of Events), and revised section text accordingly. Added description of the potential scope for DSMB recommendation to include improved safety monitoring, study modification, additional subject recruitment and termination.

Protocol Section	Nature of Change	Rationale
Appendix IV	PK sampling information added	PK sampling moved from section 7.1.8 to include together with other instructions on the administration and monitoring of busulfan. The PK sampling schedule was improved with additional collection time points. Additionally, the site for PK blood draw details were expanded to increase consistency across sites in collection and handling of samples. For logistical reasons, a window period of 3 days prior to Day -2 has been added for subject's weight for the purposes of busulfan dose calculation.
	Other significant changes made to improve safety and enhance clarity for implementation	Changes to the busulfan preparation, administration and monitoring include: - Adjusted ideal body weight dose calculation, and specification on value to use for dose calculation. - Further specifications provided to provide clarity for pharmacists and clinical staff (e.g. use of an IV tube filter, infusion timing, IV placement etc.). - For operator safety, the IV line is to be primed with saline (not busulfan), and an over-fill has been added to the infusion volume to adjust for the IV line volume. - Needle size and infusion time reduced in line with standard practice. - Lorazepam replaced with levetiracetam (Keppra™) as the prophylactic agent for busulfan-induced seizure. Levetiracetam is less sedating and provides safe and stable seizure prophylaxis. - Extended anti-emetic medication to include PRN oral dosing in addition to IV pre-medication. - Timing of vital signs adjusted to make more consistent though the total infusion and PK period. - Specified additional prohibited concomitant medications that may interfere with busulfan clearance.

CAL-USA-11 PROTOCOL VERSION 5.0 (dated 12 June 2014) – SUMMARY OF CHANGES

Protocol Section	Nature of Change	Rationale
Section 6.1.1 Section 6.1.3 Appendix III	Update anticipated dose of T^{tn} (cell numbers and volume)	CCI [redacted]
Section 6.1.1.2 Section 6.2.2 Section 7.1.8 Section 7.1.9 Appendix III	CD34 minimum dose revised	Previously expressed as a total number of cells, the minimum dose of HSPC required to proceed with busulfan conditioning has been revised to be expressed as a dose per kg of subject weight. An aliquot of $1x10^6$ CD34+ cells/kg will be cryopreserved prior to *in vitro* manipulations with the manufacture of the $HSPC^{tn}$ dose. These reserved cells will be administered of the $HSPC^{tn}$ dose is less than $2x10^6$/kg.
Section 7.1.4	Updated description of timing between Screening 1 and 2	Inconsistency between protocol text and SOE addressed by updating section 7.1.4 wording
Section 7.1.8 Appendix III Appendix IV	O2 saturation included in busulfan infusion observations	Vital sign monitoring to include O2 saturation during the actual infusions, but not required during the PK collection period. Subjects to continue to be visually monitored during this period, with vital signs to be measured as clinically indicated.
Section 7.2.4 Section 8 Schedule of Events Appendix V	Addition of a pre-busulfan assessment visit	A scheduled study visit allows for more comprehensive safety assessment prior to commencement of busulfan

Protocol Section	Nature of Change	Rationale
Section 7.5	Update description of batch analysis using the Abbott HIV viral load assay	Inconsistency between protocol text and SOE footnotes addressed by updating section 7.5 wording
Section 8 Schedule of Events	Addition of withdrawal/discontinuation visit	A withdrawal/discontinuation visit allows timely capture of important safety and efficacy data at the time of subject withdrawal/discontinuation visit.
Appendix IV	Updated list of contraindicated medications Removed specification for IV size for PK sampling	Azithromycin added to the list of contraindicated medications as it may significantly slow busulfan clearance. Specification for a 22 gauge IV for the PK sampling has been removed, allowing the site discretionary choice of the IV size

CAL-USA-11 PROTOCOL VERSION 6.0 (dated 14 October 2014) – SUMMARY OF CHANGES

Protocol	Nature of Change	Rationale
SOE 5.4/5.5 6.1.2 7.1.5 8	Permit repeat of small volume apheresis in event that the initial T^{tn} manufacturing is not successful or some other event prevents release or infusion of the product.	Multiple, small volume aphereses are performed in clinical practice following sufficient time for PBMCs to return to pre-apheresis levels (~ 4weeks). In event of a manufacturing incident, this provision would allow a subject the option to consent to the repeat apheresis and continue in the study.
SOE 5.3 5.4/5.5 6.1.2 6.2.1 7.1.7 8	Permit repeat of G-CSF mobilization and large volume apheresis in event that the initial $HSPC^{tn}$ manufacturing is not successful or some other event prevents release or infusion of the product	Repeat apheresis procedures are performed in clinical practice following sufficient time for hematopoietic stem cells to return to pre-apheresis levels (~ 4weeks). In event of a manufacturing incident, this provision would allow a subject the option to consent to the repeat procedures and continue in the study.
5.2	Redefinition of the HIV RNA (viral load) lower and upper limits of eligibility to account for normal variability of the assay	The viral load assay can have a standard variability of 0.2 $\log_{10}$. Amended inclusion criteria G to specify "Plasma HIV-1 viral RNA ≥ 5,000 copies/ml +/0.2 $\log_{10}$ and ≤ 100,000 copies/ml +/- 0.2 $\log_{10}$ at Screening 1 and Screening 2 (≥ 3,200 copies/ml and ≤ 160,000 copies/ml)". Improves the screening process without impact on subject characteristics and safety.
SoE 7.1.4	Inclusion of chemistry panel in Screen 2 visit	Feedback from apheresis units has been that electrolytes required within at least 2 weeks of planned apheresis to assist with apheresis settings. Inclusion in the study visit (and consequently central lab arrangement) simplifies procedures for the site and subject. In the event that Screening is performed > 2 weeks prior to the planned apheresis, there is now provision for the site to perform local lab CBC/differential with platelets and serum electrolytes.

Protocol	Nature of Change	Rationale
SoE 5.2 7.1.4 7.1.5	'Uncoupling' of Screen 2 visit from commencement of apheresis procedures	In practice, there are a number of factors that can impact scheduling for apheresis. The protocol requirement to link Screening to the apheresis visit was often creating stress on the study participants as well on the clinical, apheresis and laboratory facilities involved, because eligibility was not confirmed until all laboratory tests results were reported. By removing the 2-week time stipulation, there is greater flexibility and time for study sites to receive results of labs confirming subjects' eligibility and scheduling of apheresis visit with their apheresis units, thus improving logistics for the sites, without impact on subject safety. The need to have recent CBC and electrolyte data for apheresis settings has been addressed by the inclusion of an additional supplementary ('Pre-apheresis') visit if Screening 2 is performed >2 weeks prior to the planned apheresis.
SoE 6.2.2 7.1.8	Increased timing of pre-busulfan visit to up to 7 days	Improves logistics for the sites and subjects, without impact on subject safety.
Appendix IV	Change nausea prophylaxis dose from 16mg IV Ondansetron (Zofran™) to 8mg IV prior to busulfan then, post-busulfan 4mg to 8 mg orally every 8 hours as needed for nausea, not to exceed a total dose of 24 mg in 24 hours.	Maximum ondansetron dose in 24 hour period is 16 mg (stated in the product information). Upon further review of current practice for stem cell transplant procedures, the new dosing schedule has been advised not to exceed 24mg in 24 hours. This dosing schedule has been advised and approved by the Sponsor Medical Review Committee, and allows follow up oral dosing as required, thus potentially improving management of potential nausea secondary to the busulfan.
SoE 7.2.2 – 7.2.6	Additional peripheral blood marking and expression studies at Weeks 1, 2 and 6	Additional time points allow for better monitoring in the early post-transplant period
Appendix I - V	Removed provision to update appendix without a formal protocol amendment	This provision has been removed to improve protocol version control. This change was requested by one study site's IBC.
Appendix IV	Modified instructions for storage of diluted busulfan for intravenous infusion	Busulfan diluted in saline for infusion has a limited stability period (approximately 12 hours with refrigeration). Instructions modified to recommend refrigerated storage of diluted busulfan to maximize stability. Stability information added for pharmacy and site reference.

Protocol	Nature of Change	Rationale
Appendix V	Added provision for use of central venous access for apheresis, if clinically indicated	Short term central venous catheter placement is included as an option for subjects in whom it is clinically indicated to improve apheresis collection conditions and/or outcome.

CAL-USA-11 PROTOCOL VERSION 7.0 (24 March 2015) – SUMMARY OF CHANGES

Protocol Section	Nature of Change	Rationale
Contact Information	Updates	Changes to sponsor and vendor staff and contact details
Glossary of Abbreviations	New information	Additional abbreviations added
Schedule of Events: Pre-infusion	Changed use of 'Baseline' to 'Day 0' Change to various visit names Changed how window period for Screening 2 expressed Additional new line items, per protocol changes Specimen storage time point added HIV monitoring tests added to pre-busulfan assessment visit	Avoid confusion with analytical baseline reference points, such as Screening. Changes made throughout protocol text for consistency Updated visit names around mobilization and conditioning for consistency and clarity Change for clarity only Line items for plerixafor, and pre-emptive HLA typing added. Specimen storage added to pre-busulfan assessment visit to enable collection of pre-conditioning 'baseline' storage samples Additional CD4+ T lymphocyte panel and HIV-1 RNA in order to gain a more timely pre-conditioning and pre-infusion reference value.
Schedule of Events: Post-infusion	Change to various visit names New visit	Updated visit names around post-busulfan monitoring for consistency and clarity and changed how the window period/visit schedule is expressed for clarity only Follow-up Extension added
Section 4	Schematic updated	Schematic of overall study timeline updated to include changes to mobilization and apheresis procedures, as well as addition of the follow up extension.
Section 4.2.1	Change to apheresis rationale	Summary description of yields to date, providing rationale for proposed mobilization and apheresis schedule changes.
Section 4.2.3 & 7.2.7	New section (Follow-Up Extension)	Addition of an 'extension phase' for up to 12 months after Week 48 to allow ongoing monitoring of HIV data after Week 48 in subjects who complete to Week 48 off ART. If a subject had resumed ART prior to week 48, but then again stopped ART prior to Week 48, they would remain eligible to participate in the extension. Various updates to language throughout for consistency and subsequent subsection re-numbered.

Protocol Section	Nature of Change	Rationale
Section 5.2	Wording change to In-E	Re-phrased the term 'viable ART' to 'virologically-effective ART' for clarity.
Section 5.3	Change to Ex-P Wording change to Ex-V	Expanded to include NSAIDS in the contraindicated medications around the period of mobilisation, apheresis. Expanded to include the 6 week period post-busulfan conditioning Change reference to 'baseline' to 'cell infusion (day 0)'
Section 5.5	Changes to provisions for repeat procedures	Expanded potential criteria for repeat of apheresis or mobilization/apheresis procedures to include the event that the minimum HSPC^{tn} dose (2.0x10^6/kg) is not achieved, or failure of storage or shipment conditions (per changes to section 6.1) Simplified and clarified instructions and timing in the event of a process repeat.
Section 6.1.1.2 & 6.1.3	Increase dose on un- manipulated back-up apheresis product. Change HSPC^{tn} anticipated dose to a required minimum dose.	The back-up apheresis product is increased from 1x10^6 CD34+ cells/kg to 1.2x10^6 CD34+ cells/kg to make allowances for potential losses during the freeze/thaw and administration process. Increasing the cell number is not expected to deplete the final HSPC^{tn} dose, as this product is cryopreserved prior to CD34+ selection/purification. Further to experience with 4mg/kg busulfan in Cohort 2, and the degree of hematopoietic impact observed, a minimum dose of 2.0x10^6 HSPC^{tn}/kg has been specified. This is in line with common bone marrow transplant standards, and also enables preservation of the back-up apheresis product for rescue purposes, if required. Various updates throughout for consistency with this change.
Section 6.2 & 12.7.1	Modification to the 'back- up' apheresis product preparation and intention for use.	With the change to specify a minimum 2.0x106/kg HSPC^{tn} dose, the back-up apheresis product is now intended to be retained for the treatment of delayed hematopoietic recovery, or bone marrow failure should that occur in any subject. Subjects will be asked at time of initial consent to indicate if they want their back-up apheresis product to be destroyed or donated to Calimmune for research purposes if it is not required (based on recovery status at 12 weeks post-infusion). Various updates throughout and Appendix III for consistency with this change.

Protocol Section	Nature of Change	Rationale
Section 6.3.2	New section for addition of plerixafor as a mobilizing agent.	Plerixafor can result in up to 2-fold increase in CD34+ cell yields during apheresis; such a boost in CD34+ cell yields will maximise the potential HSPC^{tn} dose as well as minimise the possibility of not achieving the minimum target dose. Use of a single dose of plerixafor on Day 4 of G-CSF after the first apheresis and as late as possible in the day to aim for dosing within 16 hours of the next apheresis. Potential short-term gastro-intestinal side-effects will be limited by use of a single dose and managed with anti-diarrhoeal and anti-emetic medications. Various updates throughout for consistency with this change and renumbering of subsequent sections.
Section 6.3.3	Inclusion of provision for HLA typing. Increased busulfan dose range from 6-8mg/kg to 4- 8mg/kg given as 2 doses of 2, 3 or 4mg/kg.	Provision is added at the pre-busulfan assessment visit for each site to collect samples for HLA typing according to their standard clinical practice (i.e. testing is to be performed locally and included as a pass-through cost in the study budget). This a safety provision for the unlikely event the autologous back-up product is not effective and allogeneic transplant is required. Increased busulfan dose range gives flexibility to either continue or revert back to the busulfan dose used in Cohort 2. The DSMB will recommend the starting dose. The MRC has the discretion to reduce the dose in response to observation within the cohort. Various changes throughout for consistency
Section 6.3.3	Modification to the definition 'delayed hematopoietic recovery' to extend the time frame from 21 to 42 days post-infusion. Change in platelet limit in definition of delayed hematopoietic recovery and bone marrow failure.	Grade 4 neutropenia and grade 3 or 4 thrombocytopenia has been observed in the first 3 subjects of Cohort 2. The nadir for all 3 has occurred at Day 18 post –infusion. Originally it was anticipated that the nadir would occur approximately 9 days post-infusion. As this nadir is occurring much later than anticipated, the timeline for definition of delayed hematopoietic recovery correspondingly needs adjustment. The platelet limit is increased from $20x10^3/\mu L$ to $25x10^3/\mu L$, in line with the DAIDS grading scale for grade 4 thrombocytopenia. Various changes throughout for consistency
Section 6.3.5	Removed reference to US Guidelines for the Use of Antiretroviral Agents in HIV-1 Infected Adults and Adolescents.	Guidelines have changed, and no longer specify a threshold CD4+ T lymphocyte count for commencement of ART.

Protocol Section	Nature of Change	Rationale
Section 7.1.6 & 7.1.7	Change to 2 x 15L apheresis collections on Days 4 and 5 of G-CSF will be performed instead of the current 1 x 20L apheresis on Day 5. Include provision to collect 100ml autologous plasma on the first day of CD34 apheresis. Additional blood tests during mobilization Added description of optimal apheresis conditions.	Three of the eight CD34+ manufacturing runs to date (Cohorts 1 & 2) have yielded the desired $2x10^6$ $HSPC^{tn}$/kg dose. Multiple smaller volume apheresis collections are often performed clinically and is expected to result in a higher yield of CD34+ cells than the current single collection, which, along with the changes to the mobilization protocol, will improve the feasibility of achieving the required minimum $HSPC^{tn}$ dose. The additional plasma is required for overnight storage of the CD34+ cells from the first apheresis. Peripheral blood CD34+ counts added on days 3 and 4 of mobilization and additional biochemistry monitoring, including creatinine clearance, for improved efficacy and toxicity monitoring of the new mobilization protocol. Description added to clarify goal with apheresis collections to minimise both red cell and platelet contamination, to assist with manufacturing processes. Various changes throughout and in the Schedule of Events for consistency
Section 7.2.1	Manual differential specified	To improve reliability of local laboratory results, specification for a manual differential added to the required STAT CBC/differential during the busulfan follow up period.
Section 7.2.5	Additional bone marrow and GALT biopsies	Additional biopsies will improve the available data on engraftment and distribution of the transplanted Cal-1-modified cell. Feedback from Investigator's is that the biopsy procedures have been well tolerated. Bone marrow aspirate and GALT biopsies added to Weeks 24 and 48, as well as early discontinuation at the discretion of the Investigator. Various changes throughout, including Schedule of Events and Appendix V for consistency.
Appendix II	Update to list of prohibited medication	Clarification around the use of systemic glucocorticisteriods and updates to listed biological immunomodulatory agents and immunostimulants.
Appendix IV	Updates to contraindicated medication and delivery specifications	Clarification added around use of common analgesia which is contraindicated with busulfan administration, or during the busulfan recovery period. Removed specific gauge requirement for the IV administration set, allowing investigator discretion.

CAL-USA-11 PROTOCOL VERSION 8.0 (08 July 2015) – SUMMARY OF CHANGES

Protocol Section	Nature of Change	Rationale
Overview of Study Design	Changed description of Cohort 3 dosing to include second dose based on real-time pharmacokinetic-guided dosing to reach targeted drug exposure (AUC).	Recommendation of the DSMB at the June 24, 2015 data review meeting to utilize a targeted busulfan dosing strategy (target of 8000 µMol/L*min based on PK) vs a fixed dose scheme at the 6mg/kg level, as a reduced intensity regimen.
Schedule of Events: Pre Infusion	Pre busulfan assessment visit updated to reflect ≤11 days	Updated to correct for error in previous protocol version and adjust for change to Day -4 for first dose on busulfan in Cohort 3. Intent remains unchanged to perform pre conditioning assessment within 7 days prior to schedule busulfan infusion.
	Changed Cohort 3, 1st dose of busulfan to Day -4 from Day -3	Allows for needed turn-around-time for real-time PK analysis of 1st day (Day -4) busulfan dose in order to calculate targeted 2nd busulfan dose (Day -2).
	Added wording to clarify Day -4 busulfan administration is for Cohort 3 only and Day -2 busulfan dosing for Cohort 2 & 3	Change for clarity.
	Updated various superscript footnote numbers	Updated to correct for errors in previous protocol version.
	Footnote 9 – changed to clarify that at visits G-CSF 3 and G-CSF 4 & plerixafor and $CD34^+$ Apheresis, need CBC from Local Lab (in addition to Central Laboratory CBC)	Clarification only, as local CBC needed for safety before proceeding to next day's dose of G-CSF but Central Labs required for data capture purposes.

Protocol Section	Nature of Change	Rationale
Schedule of Events: Post Infusion	Update post busulfan monitoring schedule from every 2 days ±1 to every 3 days ±1 days, for 6 weeks. Weeks 12, 24, 48 visits- Changed visit window for study visit and associated BM Aspirate and GALT to ±1 week.	Adjusted window allowance for consistency across the 6-week intensive monitoring period to enhance site and subject adherence with the protocol. Logistical adjustment to better accommodate procedural specialists and study subject availability; enhancing protocol adherence while maintaining reasonable visit and procedural window periods.
6.3.3, 7.1.8, Appendix 4	Changed text to reflect that Cohort 3 receives two doses of Busulfex® over 3 days (Day -4 and Day -2) and how it will be accomplished with real- time, pharmacokinetic-guided AUC-targeted dosing.	Recommendation of the DSMB from the June 24, 2015 data review meeting to utilize a targeted busulfan dosing strategy (target of 8000 µMol/L*min based on PK of 1st day busulfan infusion) instead of a fixed dose scheme at the 6mg/kg. The AUC target of 8,000 µMol/L*min is recommended by the DSMB as a reduced intensity myeloconditioning regimen derived from experience with busulfan in oncology patients. Updated reference to busulfan visits and dosing in Schedule of Events and throughout document as applicable.
6.3.4	Changed Post-busulfan infection prophylaxis with trimethoprim-sulfamethoxazole (TMP-SMX) to be administered based on CD4+ T cell count	Due to potential myelosuppressive effects of trimethoprim-sulfamethoxazole (TMP-SMX), changed instructions for administration of TMP-SMX from default administration commencing with busulfan dosing, but rather be based on a subject's risk for Pneumocystis pneumonia (if CD4+ T cell count falls below 200/ml).
7.2.1	Update post busulfan monitoring schedule from every 2 days ±1 to every 3 days ±1 days, for 6 weeks.	Adjusted window allowance for consistency across the 6-week intensive monitoring period to enhance site and subject adherence with the protocol.

Protocol Section	Nature of Change	Rationale
7.2.5, Appendix V	Weeks 12, 24, 48 visits-Changed visit windows for study visit and associated BM Aspirate and GALT to ±1 week.	Logistical adjustment to better accommodate procedural specialists and study subject availability; enhancing protocol adherence while maintaining reasonable visit and procedural window periods. Updated reference to window periods in Schedule of Events and throughout document as applicable.
Administrative/Clerical Updates		
Contacts	Update	Integrated an earlier update to the study contacts (version 7.1) into this protocol amendment.
6.3.3	Changed text to clarify that dose is to be based on subject's weight at the pre-busulfan assessment visit.	Change for clarity only.
7.3	Changed requirement for creatinine clearance to be estimated by the Cockcroft-Gault equation to now be	The central lab already provides the value utilizing the MDRD equation. This formula does not require the subject's weight to be taken at every visit and is sufficient for this study indication.
Appendix 5	Added wording to allow the autologous plasma collection amounts to be adjusted.	Depending on the cell processing laboratory specifications, these amounts may need to be adjusted for each collection.
6.3.3 7.1.5, 7.1.8, 7.1.9	Update "Conditioning Assessment" to "Pre-busulfan Assessment" Corrected lab listings to match Schedule of Events	Changed to correct visit name. Updated to correct for errors in previous protocol version.

CAL-USA-11: Mock Tables: Table of Contents

Contents

GENERAL COMMENTS

- Where a count is 0, the percentage will not be shown (e.g. 0(0.0%) will be displayed as 0)
- Unless otherwise states, parameters will be listed in alphabetical order
- Percentages will be presented to one decimal place
- The minimum and maximum values will be presented to the same number of decimal places as recorded in the electronic Case Report Form (eCRF)
- Mean, median, and SD will be presented to one more decimal place than the raw data
- Percentages will be rounded to one decimal place, with the denominator being the number of subjects in the relevant population with non-missing data, unless otherwise specified
- Change from Baseline:
  Change from Baseline will be calculated as:

$$Change\ from\ baseline = new\ value - baseline\ value$$

- Unscheduled visits will be excluded from summary tables
- Names and order of Treatment Groups
  - Cohort 1 (No Busulfan)
  - Cohort 2 (One Dose Busulfan)
  - Cohort 3 (Two Dose Busulfan)
  - All

- Names of visits
  - Screening 1
  - Screening 2
  - CD4+ Apheresis
  - G-CSF 1
  - G-CSF 2
  - G-CSF 3
  - G-CSF 4
  - G-CSF 5 + CD34+ Apheresis (For cohorts 1 and 2, CD34 apheresis visit is on the same day as G-CSF 5 (G-CSF 5 + CD34 apheresis). For cohort 3, there are 2 CD34 aphereses: G-CSF 4 & plerixafor + CD34 apheresis & G-CSF 5 + CD34 apheresis)
  - Pre-Busulfan
  - Busulfan (Day -3)
  - Busulfan (Day -2)
  - Infusion (Day 0)
  - Week 1
  - Week 2
  - Week 4
  - Week 6

  - Week 8
  - Week 12
  - Week 16
  - Week 20
  - Week 24
  - Week 32
  - Week 40
  - Week 48
  - Busulfan Follow-up Regimen
  - Early Discontinuation
  - Follow-up Extension
- Column widths and text-wrapping may be altered in final output to best present the data
- Footnotes may be added/amended if required

Table 1: Protocol Deviation – Summary
Protocol: CAL-USA-11
Population: All Enrolled

n(%)	Cohort 1 (No Busulfan) (N=X)	Cohort 2 (One Dose Busulfan) (N=X)	Cohort 3 (Two Dose Busulfan) (N=X)	ALL (N=X)
Subjects with at least 1 deviation over all	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Number of deviations	X	X	X	X
Screening 1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Screening 2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
CD4+ Apheresis	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
G-CSF Administration	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
CD34+ Apheresis	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Busulfan Administration and PK	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Baseline	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 4	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 6	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 8	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 12	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 16	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 20	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 24	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 32	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 40	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Week 48	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)

Note: Percentage is based on all enrolled subjects.

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: This table will be generated from the non-compliance tracker, as provided by the Clin Ops Team.

Table 2.1 Protocol Deviation – Major Deviations Summary
Protocol: CAL-USA-11

Clinical data cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: This table will be prepared by Callimmune.

Table 2.2 Protocol Deviation – Minor Deviations Summary
Protocol: CAL-USA-11

Clinical data cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: This table will be prepared by Callimmune.

Table 3: Demographics and Baseline Information
Protocol: CAL-USA-11
Population: All Enrolled

n(%)	Cohort 1 (No Busulfan) (N=X)	Cohort 2 (One Dose Busulfan) (N=X)	Cohort 3 (Two Dose Busulfan) (N=X)	ALL (N=X)
Age (years)*				
n	X	X	X	X
Mean	X.X	X.X	X.X	X.X
Median	X.X	X.X	X.X	X.X
SD	X.X	X.X	X.X	X.X
Min	X	X	X	X
Max	X	X	X	X
Gender (n%)				
Female	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Male	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Race n(%)				
Asian	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Black	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
White	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
……	……	……	……	……
Ethnicity n(%)				
Hispanic/Latino	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
Non-Hispanic/Latino	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)

*Age calculated at date of informed consent: = Integer(Date at informed consent – date of birth +1)/365.25)
SD: Standard Deviation

Clinical data cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 4: HIV-1 History
Protocol: CAL-USA-11
Population: All Enrolled

	Cohort 1 (No Busulfan) (N=X)	Cohort 2 (One Dose Busulfan) (N=X)	Cohort 3 (Two Dose Busulfan) (N=X)	ALL (N=X)
Lowest CD4+ T Lymphocyte Count (cells/µl)				
n	X	X	X	X
Mean	XXX.X	XXX.X	XXX.X	XXX.X
Median	XXX.X	XXX.X	XXX.X	XXX.X
SD	XXX.X	XXX.X	XXX.X	XXX.X
Min	XXX	XXX	XXX	XXX
Max	XXX	XXX	XXX	XXX
Highest Plasma HIV-1 Viral Load (copies/mL)				
n	X	X	X	X
Mean	XXX,XXX.X	XXX,XXX.X	XXX,XXX.X	XXX,XXX.X
Median	XXX,XXX.X	XXX,XXX.X	XXX,XXX.X	XXX,XXX.X
SD	XXX,XXX.X	XXX,XXX.X	XXX,XXX.X	XXX,XXX.X
Min	XXX,XXX	XXX,XXX	XXX,XXX	XXX,XXX
Max	XXX,XXX	XXX,XXX	XXX,XXX	XXX,XXX

SD: Standard Deviation

Clinical data cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 5: Manufacturing CD4+ – Cohorts 1 and 2 and 3
Protocol: CAL-USA-11
Population: Safety

Programming Note: Create this table for all cohorts.

Cohort: Cohort 1 (No Busulfan)

Subject Number	xx-xxxxx	xx-xxxxx	xx-xxxxx	xx-xxxxx
CD4+ Apheresis				
Apheresis Volume (L)	x.xx	x.xx	x.xx	x.xx
Total number of cells collected (x 10^8 cells)	xxx.x	xxx.x	xxx.x	xxx.x
Age of Subject (at Apheresis visit) (yrs)	xx	xx	xx	xx
% of CD4+ in the collected cells (% CD4+)	xx.x	xx.x	xx.x	xx.x
Total number of CD4+ cells collected (x 10^8 CD4+)	xx.x	xx.x	xx.x	xx.x
CD4+ Final Cell Product (T^{tn})				
Total viable CD4+ cells (At the end of the manufacturing process) (x 10^8 cells)	xx.x	xx.x	xx.x	xx.x
Purity (% CD4+)	xx.x	xx.x	xx.x	xx.x
Viability (%)	xx.x	xx.x	xx.x	xx.x
Transduction Efficiency (determined by FACS) (%)	xx.x	xx.x	xx.x	xx.x
Transduction Efficiency (determined by WPRE qPCR) (vector/cell)	x.xx	x.xx	x.xx	x.xx
Estimated T^{tn} Dose (based on Body Weight at Apheresis visit) (x 10^6 cells/kg)	xx.x	xx.x	xx.x	xx.x
Infusion (Baseline)				
Body Weight (at Baseline visit) (kg)	xxx.x	xxx.x	xxx.x	xxx.x
Actual T^{tn} Dose (based on Body Weight on the Day of Infusion) (x 10^6 cells/kg)	xx.x	xx.x	xx.x	xx.x

Clinical data cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 6: Manufacturing CD34 – Cohorts 1 and 2 and 3
Protocol: CAL-USA-11
Population: Safety

Programming Note: Create this table for all cohorts.

Cohort: Cohort 1 (No Busulfan)

Subject Number	xx-xxxxx	xx-xxxxx	xx-xxxxx	xx-xxxxx
CD34+ Apheresis				
Apheresis Volume (L)	x.xx	x.xx	x.xx	x.xx
Total number of cells collected (x 10^8 cells)	xxx.x	xxx.x	xxx.x	xxx.x
Age of Subject (at Apheresis visit) (yrs)	xx	xx	xx	xx
% of CD34+ in the collected cells (% CD34+)	xx.x	xx.x	xx.x	xx.x
Total number of CD34+ cells collected (x 10^8 CD4+)	xx.x	xx.x	xx.x	xx.x
CD34+ Final Cell Product (HSPC^{tn})				
Total viable CD34+ cells (At the end of the manufacturing process) (x 10^8 cells)	xx.x	xx.x	xx.x	xx.x
Purity (% CD34+)	xx.x	xx.x	xx.x	xx.x
Viability (%)	xx.x	xx.x	xx.x	xx.x
Transduction Efficiency (determined by FACS) (%)	xx.x	xx.x	xx.x	xx.x
Transduction Efficiency (determined by WPRE qPCR) (vector/cell - day 7	x.xx	x.xx	x.xx	x.xx
Estimated HSPC^{tn} Dose (based on Body Weight at Apheresis visit) (x 10^6 cells/kg)	xx.x	xx.x	xx.x	xx.x
Infusion (Baseline)				
Body Weight (at Baseline visit) (kg)	xxx.x	xxx.x	xxx.x	xxx.x
Actual HSPC^{tn} Dose (based on Body Weight on the Day of Infusion) (x 10^6 cells/kg)	xx.x	xx.x	xx.x	xx.x

Clinical data cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 7: Manufacturing Deviations
Protocol: CAL-USA-11
Population: Safety

Clinical data cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: This table will be prepared by Callimmune.

Table 8.1 Cell Dose (x10e^8) at Day 0 vs. Marking/Expression Data
Protocol: CAL-USA-11
Population: Safety

Parameters: Peripheral Blood
Marking/Expression: Cal-1 Marking (WPRE qPCR) (%)

	Cohort 1 (No Busulfan) (N=X)			
	Cell Dose at Day 0 (x10e^8)		Cal-1 Marking (WPRE qPCR) (%)	
Subject Number	T^{tn} (CD4+) Dose	$HSPC^{tn}$ (CD34+) Dose	Peak	Week 48
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX
n	X	X	X	X
Mean	XXX.X	XXX.X	XXX.X	XXX.X
Median	XXX.X	XXX.X	XXX.X	XXX.X
SD	XXX.X	XXX.X	XXX.X	XXX.X
Min	XXX	XXX	XXX	XXX
Max	XXX	XXX	XXX	XXX
Cell Dose (x10e^8) vs. Peak Cal-1 Marking (WPRE qPCR) (%)				
Pearson product-moment correlation	x.xxx	x.xxx		
Spearman correlation coefficient	x.xxx	x.xxx		
Cell Dose (x10e^8) vs. Week 48 Cal-1 Marking (WPRE qPCR) (%)				
Pearson product-moment correlation	x.xxx	x.xxx		
Spearman correlation coefficient	x.xxx	x.xxx		

SD: Standard Deviation; "Not Detectable" Cal-Marking WPRE qPCR) (%) is set to 0.

Subject XX-XXXXX: Peak Cal-1 Marking (WPRE qPCR) (%) collected at Week X. Latest time point used as Week 48: Week X.

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat this table for all cohorts and overall.
This table will also be repeated (below)for all parameters (Peripheral Blood, Peripheral Blood Subsets: monocytes, granulocytes, CD8+ lymphocytes, CD4+ lymphocytes, Bone Marrow and GALT (both locations) and all markings/expression (Cal-1 Marking (WPRE qPCR) (copies/cell), Cal-1 C46 Expression (C46 RTqPCR) (relative expression), Cal-1 sh5 Expression (sh5 RTqPCR) (relative expression)).

Table 9.1: Busulfan AUC (uMol/min) vs. Marking/Expression Data
Protocol: CAL-USA-11
Population: Safety

Parameters: Peripheral Blood
Marking/Expression: Cal-1 Marking (WPRE qPCR) (%)

		Cohort 2 (One Dose Busulfan) (N=X)			
	Busulfan AUC (uMol/min) at Day -4	Busulfan AUC (uMol/min) at Day -2	Total Busulfan AUC (uMol/min) (Day -4 + Day -2)	Cal-1 Marking (WPRE qPCR) (%)	
Subject Number				Peak	Week 48
xx-xxxxx	N/A	XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx		XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx		XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx		XX.XX	XX.XX	XX.XX	XX.XX
n		X	X	X	X
Mean		XXX.X	XXX.X	XXX.X	XXX.X
Median		XXX.X	XXX.X	XXX.X	XXX.X
SD		XXX.X	XXX.X	XXX.X	XXX.X
Min		XXX	XXX	XXX	XXX
Max		XXX	XXX	XXX	XXX
Busulfan AUC (uMol/min)at Day -2 vs. Peak Cal-1 Marking (WPRE qPCR) (%)					
Pearson product-moment correlation		x.xxx	x.xxx		
Spearman correlation coefficient		x.xxx	x.xxx		
Busulfan AUC (uMol/min)at Day -2 vs. Week 48 Cal-1 Marking (WPRE qPCR) (%)					
Pearson product-moment correlation		x.xxx	x.xxx		
Spearman correlation coefficient		x.xxx	x.xxx		

SD: Standard Deviation; "Not Detectable" Cal-Marking WPRE qPCR) (%) is set to 0.

Subject XX-XXXXX: Peak Cal-1 Marking (WPRE qPCR) (%) collected at Week X. Latest time point used as Week 48: Week X.

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filep

Programming Note:
This table will be repeated (below) for all parameters (Peripheral Blood, Peripheral Blood Subsets: monocytes, granulocytes, CD8+ lymphocytes, CD4+ lymphocytes, Bone Marrow and GALT (both locations) and all markings/expression (Cal-1 Marking (WPRE qPCR) (copies/cell), Cal-1 C46 Expression (C46 RTqPCR) (relative expression), Cal-1 sh5 Expression (sh5 RTqPCR) (relative expression)).

Table 10.1: CD4+ Count (mm^3)
Protocol: CAL-USA-11
Population: Safety

Subject Number	Cohort 1 (No Busulfan) (N=X)										
	Baseline CD4+ Count (mm^3)			Maximum CD4+ Count (mm^3) post the start of the transduced cell infusion				Week 48 CD4+ Count (mm^3)			
	Screening	Pre-Apheresis	Pre-Infusion	Actual Value	Change from Screening	Change from Pre-Apheresis	Change from Pre-Infusion	Actual Value	Change from Screening	Change from Pre-Apheresis	Change from Pre-Infusion
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
n	X	X	X	X	X	X	X	X	X	X	X
Mean	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X
Median	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X
SD	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X
Min	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX
Max	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX
H_0: Change = 0											
95% CI for the Mean					(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)		(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)
P-value (Student t)-					X.XXXX	X.XXXX	X.XXXX		X.XXXX	X.XXXX	X.XXXX
95% CI for the Median					(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)		(XX.XX, XX.XX)	(XX.XX, XX.XX)	(XX.XX, XX.XX)
P-value (Signed Rank)-					X.XXXX	X.XXXX	X.XXXX		X.XXXX	X.XXXX	X.XXXX

SD: Standard Deviation
CD4+ Count (mm3) at Screening = Average of Screening 1 and Screening 2 values
CI: Confidence Interval

Subject XX-XXXXX: Maximum value post the start of the transduced cell infusion collected at Week X. Latest time point used as Week 48: Week X.

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 10.2: CD4+ Count (mm^3) vs. Marking/Expression Data
Protocol: CAL-USA-11
Population: Safety

Parameters: Peripheral Blood
Marking/Expression: Cal-1 Marking (WPRE qPCR) (%)

Subject Number	Cohort 1 (No Busulfan) (N=X)												
	CD4+ Count (mm^3)											Cal-1 Marking (WPRE qPCR) (%)	
	Baseline			Maximum value post the start of the transduced cell infusion				Week 48					
	Screening	Pre-Apheresis	Pre-Infusion	Actual Value	Change from Screening	Change from Pre-Apheresis	Change from Pre-Infusion	Actual Value	Change from Screening	Change from Pre-Apheresis	Change from Pre-Infusion	Peak	Week 48
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
xx-xxxxx	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX	XX.XX
n	X	X	X	X	X	X	X	X	X	X	X	X	X
Mean	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X
Median	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X
SD	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X	XXX.X
Min	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX
Max	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX	XXX
CD4+ Count (mm^3) vs. Peak Cal-1 Marking (WPRE qPCR) (%)													
Pearson product-moment correlation	x.xxx	x.xxx	x.xxx		x.xxx	x.xxx	x.xxx		x.xxx	x.xxx	x.xxx		
Spearman correlation coefficient	x.xxx	x.xxx	x.xxx		x.xxx	x.xxx	x.xxx		x.xxx	x.xxx	x.xxx		
CD4+ Count (mm^3) vs. Week 28 Cal-1 Marking (WPRE qPCR) (%)													
Pearson product-moment correlation	x.xxx	x.xxx	x.xxx		x.xxx	x.xxx	x.xxx		x.xxx	x.xxx	x.xxx		
Spearman correlation coefficient	x.xxx	x.xxx	x.xxx		x.xxx	x.xxx	x.xxx		x.xxx	x.xxx	x.xxx		

SD: Standard Deviation; "Not Detectable" Cal-Marking WPRE qPCR) (%) is set to 0.
CD4+ Count (mm^3) at Screening = Average of Screening 1 and Screening 2 values

Subject XX-XXXXX: Maximum CD4+ Count (mm^3) value post the start of the transduced cell infusion collected at Week X. Latest time point used as Week 48: Week X.
Peak Cal-1 Marking (WPRE qPCR) (%) collected at Week X. Latest time point used as Week 48: Week X.

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat this table for all applicable cohorts and overall.
This table will also be repeated (below) for all parameters (Peripheral Blood, Peripheral Blood Subsets: monocytes, granulocytes, CD8+ lymphocytes, CD4+ lymphocytes, Bone Marrow and GALT (both locations) and all markings/expression (Cal-1 Marking (WPRE qPCR) (copies/cell), Cal-1 C46 Expression (C46 RTqPCR) (relative expression), Cal-1 sh5 Expression (sh5 RTqPCR) (relative expression)).

Table 10.3.1. Log_{10} HIV-1-RNA Roche Assay (copies/mL)

Repeat Table 10.1, but include minimum Log_{10} HIV-1-RNA Roche Assay value post the start of the transduced cell infusion, not maximum Log_{10} HIV-1-RNA Roche Assay value.

Table 10.3.2 HIV-1-RNA Abbot Assay (copies/mL)

Repeat Table 10.1, but include minimum Log_{10} HIV-1-RNA Roche Assay value post the start of the transduced cell infusion, not maximum Log_{10} HIV-1-RNA Abbot Assay value.

Table 10.4.1 HIV-1-RNA Roche Assay (copies/mL) vs. Marking/Expression Data

Repeat Table 10.2, but include minimum Log_{10} HIV-1-RNA Roche Assay value post the start of the transduced cell infusion, not maximum Log_{10} HIV-1-RNA Roche Assay value.

Table 10.4.2 HIV-1-RNA Abbot Assay (copies/mL) vs. Marking/Expression Data

Repeat Table 10.2, but include minimum Log_{10} HIV-1-RNA Roche Assay value post the start of the transduced cell infusion, not maximum Log_{10} HIV-1-RNA Abbot Assay value.

Table 11.1 ANC (10^3/uL) vs. Marking/Expression Data

Repeat Table 10.2, but include minimum ANC value post the start of the transduced cell infusion, not maximum ANC value.

Table 11.2 Platelets (10^3/uL) vs. Marking/Expression Data

Repeat Table 10.2, but include minimum Platelet value post the start of the transduced cell infusion, not maximum Platelet value.

Table 12 Lymphocyte Development and Chronic Inflammation vs. Marking/Expression

Repeat Table 10.2 for Lymphocyte Development and Chronic Inflammation parameters

Table 13.1: Adverse Events (Summary SOC, PT)
Protocol: CAL-USA-11
Population: Safety

System Organ Class Preferred Term	Cohort 1 (No Busulfan) (N=X)		Cohort 2 (One Dose Busulfan) (N=X)		Cohort 3 (Two Dose Busulfan) (N=X)		ALL (N=X)	
	Subjects	AEs	Subjects	AEs	Subjects	AEs	Subjects	AEs
All Body Systems	X (XX.X%)	X	X (XX.X%)	X	0	X	X (XX.X%)	X
SOC1	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X
PT1	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X
PT2	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X
….	….	….	….	….	….	….	….	….
PTx	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X
SOC2	X (XX.X%)	X	0	X	X (XX.X%)	X	X (XX.X%)	X
PT1	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X
PT2	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X
….	….	….	….	….	….	….	….	….
PTx	0	X	X (XX.X%)	X	X (XX.X%)	X	X (XX.X%)	X
…….								

AE: Adverse Events

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 13.2: Adverse Events by CTC grade (Severity)
Protocol: CAL-USA-11
Population: Safety

System Organ Class (SOC) Preferred Term (PT)	Cohort 1 (No Busulfan) (N=X)				Cohort 2 (One Dose Busulfan) (N=X)			
	Mild (Grade 1)	Moderate (Grade 2)	Severe (Grade 3)	Potentially Life-Threatening (Grade 4)	Mild (Grade 1)	Moderate (Grade 2)	Severe (Grade 3)	Potentially Life-Threatening (Grade 4)
All Body Systems	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)

Note: Subjects are counted once at maximum severity within each sub grouping
Note: Percentages are based on the number of subjects in each group

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 13.2: Adverse Events by CTC grade (Severity) (Continued)
Protocol: CAL-USA-11
Population: Safety

System Organ Class (SOC) Preferred Term (PT)	Cohort 3 (Two Dose Busulfan) (N=X)				ALL (N=X)			
	Mild (Grade 1)	Moderate (Grade 2)	Severe (Grade 3)	Potentially Life-Threatening (Grade 4)	Mild (Grade 1)	Moderate (Grade 2)	Severe (Grade 3)	Potentially Life-Threatening (Grade 4)
All Body Systems	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)

Note: Subjects are counted once at maximum severity within each sub grouping
Note: Percentages are based on the number of subjects in each group

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 13.3: Treatment Emergent Adverse Events by Relationship to Study Drug (Causality)
Protocol: CAL-USA-11
Population: Safety

System Organ Class (SOC) Preferred Term (PT)	Cohort 1 (No Busulfan) (N=X)					Cohort 2 (One Dose Busulfan) (N=X)				
	Not Related	Doubtful	Possible	Probable	Very Likely	Not Related	Doubtful	Possible	Probable	Very Likely
All Body Systems	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)

Note: Subjects are counted once at maximum relatedness within each sub grouping
Note: Percentages are based on the number of subjects in each group

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 13.3: Treatment Emergent Adverse Events by Relationship to Study Drug (Causality)
Protocol: CAL-USA-11
Population: Safety

System Organ Class (SOC) Preferred Term (PT)	Cohort 3 (Two Dose Busulfan) (N=X)					ALL (N=X)				
	Not Related	Doubtful	Possible	Probable	Very Likely	Not Related	Doubtful	Possible	Probable	Very Likely
All Body Systems	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)

Note: Subjects are counted once at maximum relatedness within each sub grouping
Note: Percentages are based on the number of subjects in each group

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Table 13.4: Treatment Emergent Adverse Events by Relationship to Study Procedure (Causality)
Protocol: CAL-USA-11
Population: Safety

Study Procedure: Busulfan

System Organ Class (SOC) Preferred Term (PT)	Cohort 1 (No Busulfan) (N=X)					Cohort 2 (One Dose Busulfan) (N=X)				
	Not Related	Doubtful	Possible	Probable	Very Likely	Not Related	Doubtful	Possible	Probable	Very Likely
All Body Systems	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)

Note: Subjects are counted once at maximum relatedness within each sub grouping
Note: Percentages are based on the number of subjects in each group

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat for all study procedures: GCSF, : Bone Marrow Aspiration, etc.

Table 13.4: Adverse Events by Relationship to Study Procedure (Causality)
Protocol: CAL-USA-11
Population: Safety

Study Procedure: Busulfan

System Organ Class (SOC) Preferred Term (PT)	Cohort 3 (Two Dose Busulfan) (N=X)					ALL (N=X)				
	Not Related	Doubtful	Possible	Probable	Very Likely	Not Related	Doubtful	Possible	Probable	Very Likely
All Body Systems	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
SOC2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT1	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PT2	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
….	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)
PTx	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)	X (XX.X%)

Note: Subjects are counted once at maximum relatedness within each sub grouping
Note: Percentages are based on the number of subjects in each group

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat for all study procedures: GCSF, : Bone Marrow Aspiration, etc.

CAL-USA-11: Mock Listings: Table of Contents

Contents

GENERAL COMMENTS

- Names and order of Treatment Groups
  - Cohort 1 (No Busulfan)
  - Cohort 2 (One Dose Busulfan)
  - Cohort 3 (Two Dose Busulfan)
  - All

- Names of visits
  - Screening 1
  - Screening 2
  - CD4+ Apheresis
  - G-CSF 1
  - G-CSF 2
  - G-CSF 3
  - G-CSF 4
  - G-CSF 5 + CD34+ Apheresis (For cohorts 1 and 2, CD34 apheresis visit is on the same day as G-CSF 5 (G-CSF 5 + CD34 apheresis). For cohort 3, there are 2 CD34 aphereses: G-CSF 4 & plerixafor + CD34 apheresis & G-CSF 5 + CD34 apheresis)
  - Pre-Busulfan
  - Busulfan (Day -3)
  - Busulfan (Day -2)
  - Infusion (Day 0)
  - Week 1
  - Week 2
  - Week 4
  - Week 6
  - Week 8
  - Week 12
  - Week 16
  - Week 20
  - Week 24
  - Week 32
  - Week 40
  - Week 48
  - Busulfan Follow-up Regimen
  - Early Discontinuation

    - Follow-up Extension
- Column widths and text-wrapping may be altered in final output to best present the data
- Footnotes may be added/amended if required

Listing 1.1: Subject Disposition
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Informed Consent Date (YYYY-MM-DD)	CD34+ Apheresis Date (YYYY-MM-DD)	Infusion Date (YYYY-MM-DD)	Enrolled Population	Safety Population
xx-xxxxxx	YYYY-MM-DD	YYYY-MM-DD	YYYY-MM-DD	Yes	No
xx-xxxxxx	YYYY-MM-DD	YYYY-MM-DD	YYYY-MM-DD	Yes	No
xx-xxxxxx	YYYY-MM-DD	YYYY-MM-DD	YYYY-MM-DD	Yes	Yes
xx-xxxxxx	YYYY-MM-DD	YYYY-MM-DD	YYYY-MM-DD	Yes	Yes

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 1.2: Subject Disposition (continued)
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	CD4+ Apheresis Date (YYYY-MM-DD)	Status (Ongoing, Discontinuation, Withdrawal or Completion)[1]	Date of Discontinuation, Withdrawal or Completion (YYYY-MM-DD)	Date Antiretroviral Therapy Commenced	Time from CD4+ Apheresis to Commencement of ART (days)[3]	Last Visit Prior to Discontinuation or Withdrawal	Last Scheduled Visit	Primary Reason for Discontinuation or Withdrawal2
xx-xxxxxx	YYYY-MM-DD	Discontinuation	YYYY-MM-DD	YYYY-MM-DD	xx	Week xx	Week xx	Commencement of antiretroviral therapy
xx-xxxxxx	YYYY-MM-DD	Discontinuation	YYYY-MM-DD		xx	Week xx	Week xx	Commencement of antiretroviral therapy
xx-xxxxxx	YYYY-MM-DD	Completed	YYYY-MM-DD		xx	-	Week xx	
xx-xxxxxx	YYYY-MM-DD	Completed	YYYY-MM-DD		xx	-	Week xx	

[1] Withdrawal: any subject who withdrew consent from the trial; Discontinuation: any subject who discontinued from primary endpoint analysis
[2] Last scheduled visit before discontinuation or withdrawal.
[3] Date Antiretroviral Therapy Commenced / Date of Discontinuation, Withdrawal or Completion - CD4+ Apheresis Date + 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Include all detail with regards to reason for discontinuation in "Primary Reason for Discontinuation or Withdrawal[2]":

- SAE number if applicable
- AE number if applicable
- Manufacturing failure details if applicable

Listing 2: Subject Withdrawal
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Date of Withdrawal (YYYY-MM-DD)	Phase of Withdrawal[1]	Primary Reason for Withdrawal	Secondary Reason for Withdrawal *
xx-xxxxxx	YYYY-MM-DD	Post-infusion phase	Commencement of antiretroviral therapy	Other: XXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX
xx-xxxxxx	YYYY-MM-DD	Post-infusion phase	Commencement of antiretroviral therapy	Other: XXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX
xx-xxxxxx	YYYY-MM-DD	Post-infusion phase	Commencement of antiretroviral therapy	Other: XXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXXX

* Withdrawal occurred in Post-Infusion phase.
[1] Withdrawal: any subject who withdrew consent from the trial

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 3: Subject Discontinuation
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Date of Discontinuation (YYYY-MM-DD)	Phase of Discontinuation	Last Visit Prior to Discontinuation	Primary Reason for Discontinuation
xx-xxxxxx	YYYY-MM-DD	Post-infusion phase	Week xx	Commencement of antiretroviral therapy
xx-xxxxxx	YYYY-MM-DD	Post-infusion phase	Week xx	Commencement of antiretroviral therapy
xx-xxxxxx	YYYY-MM-DD	Post-infusion phase	Week xx	Commencement of antiretroviral therapy

Note: Subject discontinuation refers to any subject who discontinued from the primary endpoint analysis

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 4.1: Demographics
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Center	Gender	Date of Birth (YYYY-MM-DD)	Age*	Race	Ethnicity	Child Bearing Potential
xx-xxxxxx	Quest Clinical Research: Dr Lalezari	Male	YYYY-MM-DD	xx	White	Not Hispanic or Not Latino	
xx-xxxxxx	UCLA CARE Center Clinic: Dr Mitsuyasu	Male	YYYY-MM-DD	xx	White	Not Hispanic or Not Latino	
xx-xxxxxx	Quest Clinical Research: Dr Lalezari	Female	YYYY-MM-DD	xx	White	Not Hispanic or Not Latino	Yes

* Age calculated at date of informed consent

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 4.2: Pre-Busulfan Body Weight
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 2

Subject Number	Date Performed (YYYY-MM-DD)	Time Performed (HH:MM)	Actual Weight (kg)	Adjusted ideal body weight (AIBW)	Was Actual Weight OR adjusted ideal body weight (AIBW) used for the Calculation of the Busulfan dose
xx-xxxxxx	YYYY-MM-DD	HH:MM	xx.x	xx.x	Actual Weight
xx-xxxxxx	YYYY-MM-DD	HH:MM	xx.x	xx.x	Actual Weight
xx-xxxxxx	YYYY-MM-DD	HH:MM	xx.x	xx.x	Actual Weight
xx-xxxxxx	YYYY-MM-DD	HH:MM	xx.x	xx.x	Actual Weight
xx-xxxxxx	YYYY-MM-DD	HH:MM	xx.x	xx.x	Actual Weight

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 5: HIV-1 History
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Date of HIV-1 infection Diagnosis (YYYY-MM-DD)	Lowest CD4+ T Lymphocyte Count (cells/µl)	Date of Lowest CD4+ Count (YYYY-MM-DD)	Highest Plasma HIV Viral Load (copies/mL)	Date of Highest HIV Viral Load (YYYY-MM-DD)
xx-xxxxxx	YYYY-MM-DD	xxx	YYYY-MM-DD	xxxxxxx	YYYY-MM-DD
xx-xxxxxx	YYYY-MM-DD	xxx	YYYY-MM-DD	xxxxxxx	YYYY-MM-DD
xx-xxxxxx	YYYY-MM-DD	xxx	YYYY-MM-DD	xxxxxxx	YYYY-MM-DD

NK: Not Known

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 6: Medical History
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	MH No	Condition	Body System Code	Onset Date (YYYY-MM-DD)	Resolution Date (YYYY-MM-DD)	Ongoing?	Any Treatment Planned?
xx-xxxxxx	1	xxxxxxxxxxxxxxxxxxxxxxxxxx	Dermatologic	YYYY-MM-DD		Yes	No
	3	xxxxxxxxxxxxxxxxxxxxxxxxxx	Neurological	YYYY-MM-DD	YYYY-MM-DD	No	No
	4	xxxxxxxxxxxxxxxxxxxxxxxxxx	Other: fainting	YYYY-MM-DD	YYYY-MM-DD	No	No
	6	xxxxxxxxxxxxxxxxxxxxxxxxxx	Allergies/Drug Sensitivity	NK		Yes	No
	Etc.						
xx-xxxxxx	X	xxxxxxxxxxxxxxxxxxxxxxxxxx	Ears	NK		Yes	No

NK: Not Known; MH No: Medical History Number

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 7: Eligibility
Protocol: CAL-USA-11
Population: All Screened

Cohort: Cohort 1

		Inclusion Criteria		Exclusion Criteria	
Subject Number	Evaluation Date (YYYY-MM-DD)	Criteria Not Met*	Reason Not Met	Criteria Met*	Reason Met
xx-xxxxxx	YYYY-MM-DD	xxxxx	HIV-1 VIRAL RNA LESS THAN 5000 COPIES/ML		
xx-xxxxxx	YYYY-MM-DD	xxxxx	screening cd4 <500	xxxxx	cd4 history <250
xx-xxxxxx	YYYY-MM-DD	xxxxx	Plasma HIV-1 viral RNA < 5,000 copies/ml reported on August 31, 2013		
xx-xxxxxx	YYYY-MM-DD	xxxxx	CD4 <500		
xx-xxxxxx	YYYY-MM-DD	xxxxx	HIV-1 RNA > 100,000 copies/ml at Screen 1 reported on December 29, 2013		
xx-xxxxxx	YYYY-MM-DD	xxxxx	viral load 190,000		
xx-xxxxxx	YYYY-MM-DD	xxxxx	CD4+ T lymphocyte count < 500 at Screen 2 reported on September 18, 2013"		

* Inclusion & Exclusion Criteria: refer to Protocol CAL-USA-11, Section 5

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 8.1: Pregnancy Test
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Date of Collection (YYYY-MM-DD)	Time of Collection (HH:MM)	Variable* (Serum ß-HCG or Urine Test)	Result	Unit	Clin Sig.**	Comment
xx-xxxxxx	Screening 1	YYYY-MM-DD	HH:MM	xxxxxxxxxxxxx	Negative	xxxxx		xxxxxxxxxxx
	Screening 2	YYYY-MM-DD	HH:MM	xxxxxxxxxxxxx	Negative	xxxxx		xxxxxxxxxxx
	G-CSF-1	YYYY-MM-DD	HH:MM	xxxxxxxxxxxxx	Negative	xxxxx		xxxxxxxxxxx
xx-xxxxxx								
xx-xxxxxx								

* Serum ß-HCG testing from women of reproductive potential, except at commencement of G-CSF, busulfan and infusion (baseline), when urine test is performed
** Clinical Significance: CS=Clinically Significant, NCS=Not Clinically Significant

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 8.2: Positive Pregnancy Test
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Date of Collection (YYYY-MM-DD)	Time of Collection (HH:MM)	Variable* (Serum ß-HCG or Urine Test)	Result	Unit	Clin Sig.**	Comment
xx-xxxxxx	Screening 1	YYYY-MM-DD	HH:MM	xxxxxxxxxxxxx	Positive	xxxxx	NCS	xxxxxxxxxxx
	Screening 2	YYYY-MM-DD	HH:MM	xxxxxxxxxxxxx	Positive	xxxxx	NCS	xxxxxxxxxxx
	G-CSF-1	YYYY-MM-DD	HH:MM	xxxxxxxxxxxxx	Positive	xxxxx	NCS	xxxxxxxxxxx
xx-xxxxxx								
xx-xxxxxx								

* Serum ß-HCG testing from women of reproductive potential, except at commencement of G-CSF, busulfan and infusion (baseline), when urine test is performed
** Clinical Significance: CS=Clinically Significant, NCS=Not Clinically Significant

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 9: Chest X-Rays
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Date Performed (YYYY-MM-DD)	Result (If CS, specify)*
xx-xxxxxx	Screening 1	YYYY-MM-DD	Normal
	Screening 2	YYYY-MM-DD	Abnormal – NCS
xx-xxxxxx	Screening 1	YYYY-MM-DD	Norma;
	Screening 2	YYYY-MM-DD	Normal

*Clinical Significance: CS=Clinically Significant, NCS=Not Clinically Significant

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 10: ART Therapy History (regimen overview)
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Regimen Number	Drug	Start Date (YYYY-MM-DD)	Stop Date (YYYY-MM-DD)	Ongoing	Reason for Discontinuation of therapy	Lowest Plasma HIV Viral Load Achieved During Regimen (copies/mL)
xx-xxxxxx	1	3TC	YYYY-MM-DD	YYYY-MM-DD	No	Toxicity	<500
		D4T	YYYY-MM-DD	YYYY-MM-DD	No		
		INVIRASE	YYYY-MM-DD	YYYY-MM-DD	No		
	2	CRIXIVAN	YYYY-MM-DD	YYYY-MM-DD	No	Toxicity	<500
		COMBIVIR	YYYY-MM-DD	YYYY-MM-DD	No		
	3	COMBIVIR	YYYY-MM-DD	YYYY-MM-DD	No	Treatment fatigue	<50
		SUSTIVA	YYYY-MM-DD	YYYY-MM-DD	No		
xx-xxxxxx	1	Complera	YYYY-MM-DD	YYYY-MM-DD	No	Treatment fatigue	<20
xx-xxxxxx	1	ATRIPLA	YYYY-MM-DD	YYYY-MM-DD	No	Toxicity	<20
	2	COMPLERA	YYYY-MM-DD	YYYY-MM-DD	No	Treatment fatigue	<20
xx-xxxxxx	1	truvada	YYYY-MM-DD	YYYY-MM-DD	No	Treatment fatigue	<20
		isentress	YYYY-MM-DD	YYYY-MM-DD	No		
xx-xxxxxx	1	Miraviroc	YYYY-MM-DD	YYYY-MM-DD	No	Toxicity	<50
	2	Atripla	YYYY-MM-DD	YYYY-MM-DD	No	Toxicity	<75
	3	complera	YYYY-MM-DD	YYYY-MM-DD	No	Treatment fatigue	<75
xx-xxxxxx	1	stribild	YYYY-MM-DD	YYYY-MM-DD	No	Treatment fatigue	<20
	2	stribild	YYYY-MM-DD	YYYY-MM-DD	No	Treatment fatigue	44
xx-xxxxxx	1	ISENTRESS	YYYY-MM-DD	YYYY-MM-DD	No	Treatment fatigue	<20
		TRUVADA	YYYY-MM-DD	YYYY-MM-DD	No		
xx-xxxxxx	1	truvada	YYYY-MM-DD	YYYY-MM-DD	No	Toxicity	<40
		miraviroc	YYYY-MM-DD	YYYY-MM-DD	No		
	2	darunavir	YYYY-MM-DD	YYYY-MM-DD	No	Toxicity	unk
		ritonavir	YYYY-MM-DD	YYYY-MM-DD	No		
	3	truvada	YYYY-MM-DD	YYYY-MM-DD	No	Treatment fatigue	<40
		raltegravir	YYYY-MM-DD	YYYY-MM-DD	No		

NK: Not Known

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 11: Transduced CD4+ Cell Product Dose (Transduction efficiency, viability, cell numbers for T^{tn})
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

		Total Number of Cells Infused[1]					
Subject Number	Date of TCP Infusion (YYYY-MM-DD)	Absolute ($x10^8$ cells)	Expressed as cells/kg body weight ($x10^6$ cells/kg)	Viability (%)[2]	Purity of cells infused	Transduction Efficiency of Cells Infused (%)	Cal-1 Vector Copy Number (VCN) per T^{tn} cell (VCN/cell)
xx-xxxxxx	YYYY-MM-DD	xx	xx.x	xx	xx.x	xx.x	xx.x
xx-xxxxxx	YYYY-MM-DD	xx	xx.x	xx	xx.x	xx.x	xx.x
xx-xxxxxx	YYYY-MM-DD	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x
xx-xxxxxx	YYYY-MM-DD	xx.x	xx.x	xx	xx.x	xx.x	xx.x

[1] Viable, transduced cells (viability based on pre-thaw viability)
[2] Pre-thaw viability of T^{tn} cells (%)
TCP: Transduced Cell Product
T^{tn}: Cal-1 transduced CD4+ T lymphocytes

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 12: Transduced CD34+ Cell Product Dose (Transduction efficiency, viability, cell numbers for HSPC^{tn})
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

		Total Number of Cells Infused[1]						
Subject Number	Date of TCP Infusion (YYYY-MM-DD)	Absolute (x10^8 cells)	Expressed as cells/kg body weight (x10^6 cells/kg)	Viability (%)[2]	Purity of cells infused	Transduction Efficiency of Cells Infused (%)	Cal-1 Vector Copy Number (VCN) per Ttn cell (VCN/cell)	Was the CD34+ back-up apheresis product infused
xx-xxxxxx	YYYY-MM-DD	xx	xx.x	xx	xx.x	xx.x	xx.x	-
xx-xxxxxx	YYYY-MM-DD	xx	xx.x	xx	xx.x	xx.x	xx.x	-
xx-xxxxxx	YYYY-MM-DD	xx.x	xx.x	xx.x	xx.x	xx.x	xx.x	-
xx-xxxxxx	YYYY-MM-DD	xx.x	xx.x	xx	xx.x	xx.x	xx.x	-
								-

[1] Viable, transduced cells (viability based on pre-thaw viability)
[2] Pre-thaw viability of HSPC^{tn} cells (%)
TCP: Transduced Cell Product
HSPC^{tn}: Cal-1 transduced CD34+ Hematopoietic stem/progenitor cells

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 13.1: G-CSF Therapy
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Was G-CSF Administered?	Dose Administered (µg/kg)	Total Daily Dose Administered (µg)	Full Dose Administered?	Date/ Time Administered (YYYY-MM-DD) / HH:MM	Time Second Dose Administered HH:MM	Was the White blood cell count on Day 4 of G-CSF < 75,000x10e9/mL?
xx-xxxxxx	G-CSF 1	Yes	10µg/kg QD	xxx	-	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 2	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 3	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 4	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	CD34+ Apheresis							xx
xx-xxxxxx	G-CSF 1	Yes	10µg/kg QD	xxx	-	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 2	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 3	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 4	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	CD34+ Apheresis							xx
xx-xxxxxx	G-CSF 1	Yes	10µg/kg QD	xxx	-	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 2	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 3	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 4	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	CD34+ Apheresis							xx
xx-xxxxxx	G-CSF 1	Yes	10µg/kg QD	xxxxx	-	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 2	Yes	10µg/kg QD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 3	Yes	5µg/kg BD	-	No	YYYY-MM-DD/ HH:MM	HH:MM	
	G-CSF 4	Yes	5µg/kg BD	-	Yes	YYYY-MM-DD/ HH:MM	HH:MM	
	CD34+ Apheresis							xx

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 13.2: Plerixafor Therapy
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 3

Subject Number	Visit	Total Daily Dose Administered (µg)	Date/ Time Administered (YYYY-MM-DD) / HH:MM
xx-xxxxxx	G-CSF 4 & plerixafor	xxx	YYYY-MM-DD/ HH:MM
xx-xxxxxx	G-CSF 4 & plerixafor	xxx	YYYY-MM-DD/ HH:MM
xx-xxxxxx	G-CSF 4 & plerixafor	xxx	YYYY-MM-DD/ HH:MM
xx-xxxxxx	G-CSF 4 & plerixafor	xxx	YYYY-MM-DD/ HH:MM
xx-xxxxxx	G-CSF 4 & plerixafor	xxx	YYYY-MM-DD/ HH:MM
xx-xxxxxx	G-CSF 4 & plerixafor	xxx	YYYY-MM-DD/ HH:MM
xx-xxxxxx	G-CSF 4 & plerixafor	xxx	YYYY-MM-DD/ HH:MM
xx-xxxxxx	G-CSF 4 & plerixafor	xxx	YYYY-MM-DD/ HH:MM
xx-xxxxxx	G-CSF 4 & plerixafor	xxx	YYYY-MM-DD/ HH:MM

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name   Created: DDMMMYYYY HH:MM

Listing 14.1: Apheresis - CD4+
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Apheresis Completed Successfully? (if no, specify)	Date Performed (YYYY-MM-DD)	Star/Stop Time	Venous Access Location	Filtration Volume (L)	Number of CD4+ Lymphocytes Collected ($x10^8$ cells)	CD4+ % T-lymphocytes (%)	Total number of target CD4+ T-lymphocytes collected (x10e8) (x10e8)
xx-xxxxxx	CD34+ Apheresis	Yes	YYYY-MM-DD	HH:MM/ HH:MM	Central venous	x.x	x.x	xx	xx
xx-xxxxxx	CD34+ Apheresis	Yes	YYYY-MM-DD	HH:MM/ HH:MM	Central venous	x.xxx	xx.x	xx	xx
xx-xxxxxx	CD34+ Apheresis	Yes	YYYY-MM-DD	HH:MM/ HH:MM	Central venous	x	xx	xx	xx
xx-xxxxxx	CD34+ Apheresis	Yes	YYYY-MM-DD	HH:MM/ HH:MM	Central venous	x	xx.x	xx	xx

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat this listing for:
14.2 Apheresis - CD34+

Listing 14.2: Apheresis - CD34+
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 15.1: Busulfan Dosing
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 2

Subject Number	Busulfan Dose	Date Administered (YYYY-MM-DD)	Total Volume of the Intended Busulfan Dose (ml)	Residual Volume of the IV Line (ml)	Infusion Pump Flow Rate (ml/hour)	Dose Interrupted
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	No
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	No
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	No
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	No
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	No
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	No
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	Yes
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	Yes
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	Yes
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	xx	xx	xx	Yes

Note: Cohort 1 not included as this cohort did not receive Busulfan

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 15.1: Busulfan Interruptions
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 2

Subject Number	Busulfan Dose	Date Administered (YYYY-MM-DD)	Infusion Interruption Time (HH:MM)	Infusion restart time (HH:MM)	Reason for Interruption
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx
xx-xxxxxx	1 x 4mg/kg	YYYY-MM-DD	HH:MM	HH:MM	xxxxxxxxxxxxxxxxxxxxxxxx

Note: Cohort 1 not included as this cohort did not receive Busulfan

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name   Created: DDMMMYYYY HH:MM

Listing 15.3: Busulfan Administration and Pharmacokinetics
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 2

Subject Number	Date Administered (YYYY-MM-DD)	Infusion Start Time (HH:MM)	Infusion End Time (HH:MM)	Total Dose Administered (mg)	Scheduled PK Testing Time	Actual Collection Time (HH:MM)	PK AUC (umol/min)	Clearance Rate (mL/min/Kg)	Concentration at Steady State (ng/mL)
xx-xxxxxx	YYYY-MM-DD	HH:MM	HH:MM	xxx.x	Pre-Dose	-	xxxxx	x.xx	xxxxx
					EOI	HH:MM			
					EOI + 15 minutes	HH:MM			
					EOI + 1 hour	HH:MM			
					EOI + 2 hour	HH:MM			
					EOI + 3 hour	HH:MM			
					EOI + 4 hour	HH:MM			
					EOI + 5 hour	HH:MM			
xx-xxxxxx	YYYY-MM-DD	HH:MM	HH:MM	xxx	Pre-Dose	-	xxxxx	x.xx	xxxxx
					EOI	HH:MM			
					EOI + 15 minutes	HH:MM			
					EOI + 1 hour	HH:MM			
					EOI + 2 hour	HH:MM			
					EOI + 3 hour	HH:MM			
					EOI + 4 hour	HH:MM			
					EOI + 5 hour	HH:MM			
xx-xxxxxx	YYYY-MM-DD	HH:MM	HH:MM	xxx.xx	Pre-Dose	-	xxxxx	x.xx	xxxxx
					EOI	HH:MM			
					EOI + 15 minutes	HH:MM			
					EOI + 1 hour	HH:MM			
					EOI + 2 hour	HH:MM			
					EOI + 3 hour	HH:MM			
					EOI + 4 hour	HH:MM			
					EOI + 5 hour	HH:MM			

Note: Cohort 1 not included as this cohort did not receive Busulfan
EOI: End of Infusion; AUC: Area Under the Curve

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 16: Cal-1 Marking/Expression - Peripheral Blood
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

			Cal-1 Marking (WPRE qPCR)			Cal-1 C46 Expression (C46 RT-qPCR)		Cal-1 sh5 Expression (sh5 RT-qPCR)	
Subject Number	Visit	Collection Date (YYYY-MM-DD)	Result	Quantification (Copies/cell)	Quantification (%)	Result	Quantification (Relative Expression)	Result	Quantification (Relative Expression)
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Not detected			Not detected		Not detected	
	Week 4	YYYY-MM-DD	Detected, NQ			Not detected		Not detected	
	Week 12	YYYY-MM-DD	Detected, NQ			Not detected		Not detected	
	Week 24	YYYY-MM-DD	Not detected			Not detected		Not detected	
	Week 32	YYYY-MM-DD	Detected, NQ			Not detected		Not detected	
	Week 40	YYYY-MM-DD	Detected, NQ			Not detected		Not detected	
	Week 48	YYYY-MM-DD	Not detected			Not detected		Detected, NQ	
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Not detected			Not detected		Not detected	
	Week 4	YYYY-MM-DD	Detected, NQ			Not detected		Not detected	
	Week 12	YYYY-MM-DD	Detected	x.xxxxx	x.xx	Not detected		Not detected	
	Week 24	YYYY-MM-DD	Not detected			Not detected		Detected, NQ	
	Week 32	YYYY-MM-DD	Not detected			Not detected		Not detected	
	Week 40	YYYY-MM-DD	Detected, NQ			Not detected		Not detected	
	Week 48	YYYY-MM-DD	Not detected			Not detected		Not detected	
	Week 40	YYYY-MM-DD	Detected, NQ			Not detected		Not detected	

NQ: Not Quantifiable
Mandatory Fields: Cal-1 Marking
Optional Fields: Cal-1 Expression

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 17: Cal-1 Marking/Expression – Bone Marrow
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

			Cal-1 Marking (WPRE QPCR)		Cal-1 C46 Expression (C46 RT-qPCR)		Cal-1 sh5 Expression (sh5 RT-qPCR)	
Subject Number	Visit	Date BMA Obtained (YYYY-MM-DD)	Result	Quantification (Copies/cell)	Result	Quantification (Relative Expression)	Result	Quantification (Relative Expression)
xx-xxxxxx	Week 12	YYYY-MM-DD	Not detected		Not detected		Not detected	
xx-xxxxxx	Week 12	YYYY-MM-DD	Not detected		Not detected		Detected, NQ	
xx-xxxxxx	Week 12	YYYY-MM-DD	Not detected		Not detected		Detected, NQ	
xx-xxxxxx	Week 12	YYYY-MM-DD	Detected	0.xxxxxxx	Not detected		Detected, NQ	

NQ: Not Quantifiable
BMA: Bone Marrow Aspirate
Mandatory Fields: Cal-1 Marking
Optional Fields: Cal-1 Expression

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 18: Cal-1 Marking/Expression – GALT
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

				Cal-1 Marking (WPRE QPCR)		Cal-1 C46 Expression (C46 RT-qPCR)		Cal-1 sh5 Expression (sh5 RT-qPCR)	
Subject Number	Visit	Date GALT Biopsy Obtained (YYYY-MM-DD)	Location	Result	Quantification (Copies/cell)	Result	Quantification (Relative Expression)	Result	Quantification (Relative Expression)
xx-xxxxxx	Week 12	YYYY-MM-DD	1: 10-15cm	Detected	x.xxxxx	Not detected		Detected, NQ	
			2: 25-35cm	Detected	x.xxxxx	Not detected		Detected, NQ	
xx-xxxxxx	Week 12	YYYY-MM-DD	1: 10-15cm	Detected	x.xxxxx	Not detected		Not detected	
			2: 25-35cm	Detected	x.xxxxx	Not detected		Not detected	
xx-xxxxxx	Week 12	YYYY-MM-DD	1: 10-15cm	Detected	x.xxxxx	Not detected		Not detected	
			2: 25-35cm	Detected	x.xxxxx	Not detected		Not detected	
xx-xxxxxx	Week 12	YYYY-MM-DD	1: 10-15cm	Not detected		Not detected		Detected, NQ	
			2: 25-35cm	Not detected		Not detected		Detected, NQ	

NQ: Not Quantifiable
Mandatory Fields: Cal-1 Marking; Optional Fields: Cal-1 Expression

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 19.1: Cal-1 Marking/Expression – Subsets Monocytes
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

				Cal-1 Marking (WPRE QPCR)		Cal-1 C46 Expression (C46 RT-qPCR)		Cal-1 sh5 Expression (sh5 RT-qPCR)	
Subject Number	Visit	Collection Date (YYYY-MM-DD)	Population Purity (CD14+/CD45+) (%)	Result	Quantification (Copies/cell)	Result	Quantification (Relative Expression)	Result	Quantification (Relative Expression)
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	xx.x	Detected, NQ		Not detected		Detected, NQ	
	Week 4	YYYY-MM-DD	xx.x	Not detected		Not detected		Detected	x.xxxxx
	Week 12	YYYY-MM-DD	xx.x	Detected, NQ		Not detected		Detected, NQ	
	Week 24	YYYY-MM-DD	xx.x	Not detected		Not detected		Not detected	
	Week 48	YYYY-MM-DD	xx.x	Not detected		Not detected		Detected, NQ	
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	xx.x	Detected, NQ		Not detected		Detected, NQ	
	Week 4	YYYY-MM-DD	xx.x	Detected, NQ		Not detected		Not detected	
	Week 12	YYYY-MM-DD	xx.x	Not detected		Not detected		Not detected	
	Week 24	YYYY-MM-DD	xx.x	Not detected		Not detected		Not detected	
	Week 48	YYYY-MM-DD	xx.x	Detected	x.xxxxx	Not detected		Not detected	
	Early discontinuation / withdrawal	YYYY-MM-DD	xx.x	Detected, NQ		Not detected		Detected	x.xxxxx

NQ: Not Quantifiable

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat this listing for:
19.2 Cal-1 Marking/Expression – Subsets: Granulocytes
19.3 Cal-1 Marking/Expression – Subsets: CD4+ Lymphocytes
19.4 Cal-1 Marking/Expression – Subsets: CD8+ Lymphocytes

Listing 19.2: Cal-1 Marking/Expression – Subsets: Granulocytes
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 19.3: Cal-1 Marking/Expression – Subsets: CD4+ Lymphocytes
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 19.4: Cal-1 Marking/Expression – Subsets: CD8+ Lymphocytes
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 20.1: Serious Adverse Events, including event narratives
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	AE No.	SOC/ Preferred Term/ Verbatim Term	Start Date (YYYY-MM-DD)	Stop Date (YYYY-MM-DD)	CTC Grade	Action Taken	Outcome	Causality (Related to Cal-1)	Causality (Related to Procedure / Required Medication)	Details
xx-xxxxxx	1	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZ	YYYY-MM-DD	YYYY-MM-DD	Mild	None	Resolved	Not related	Very likely: GCSF	xxxxxxxxxxxxxx
	2	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZ	YYYY-MM-DD	YYYY-MM-DD	Mild	None	Resolved	Very likely	Not related	xxxxxxxxxxxxxx
	3	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZ	YYYY-MM-DD	-	Mild	Concomitant medication	Ongoing	Possible	Not related	xxxxxxxxxxxxxx
	4	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZ	YYYY-MM-DD	-	Mild	None	Ongoing	Not related	Not related	xxxxxxxxxxxxxx

SOC: System Organ Class, AE No.: Adverse Event Number; CTC: Common Terminology Criteria; NK: Not Known

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 20.2: Deaths
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	AE No.	SOC/ Preferred Term/ Verbatim Term	Start Date (YYYY-MM-DD)	Stop Date (YYYY-MM-DD)	Death Date (YYYY-MM-DD)	Autopsy Performed	Autopsy Date (YYYY-MM-DD)	Type of Tissue Collected
xx-xxxxxx	1	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZZ	YYYY-MM-DD	YYYY-MM-DD	YYYY-MM-DD	Yes	YYYY-MM-DD	xxxxxxxx
	2	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZZ	YYYY-MM-DD	YYYY-MM-DD	YYYY-MM-DD	No	YYYY-MM-DD	xxxxxxxx

SOC: System Organ Class, AE No.: Adverse Event Number; CTC: Common Terminology Criteria; NK: Not Known

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 21.1: Adverse Events (All Grades)
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	AE No.	SOC/ Preferred Term/ Verbatim Term	Start Date (YYYY-MM-DD)	Stop Date (YYYY-MM-DD)	Serious	CTC Grade	Action Taken	Outcome	Causality (Related to Cal-1)	Causality (Related to Procedure / Required Medication)	Details
xx-xxxxxx	1	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZ	YYYY-MM-DD	YYYY-MM-DD	No	Mild	None	Resolved	Not related	Very likely: GCSF	xxxxxxxxxxxxxx
	2	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZ	YYYY-MM-DD	YYYY-MM-DD	NO	Mild	None	Resolved	Very likely	Not related	xxxxxxxxxxxxxx
	3	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZ	YYYY-MM-DD	-	Yes	Mild	Concomitant medication	Ongoing	Possible	Not related	xxxxxxxxxxxxxx
	4	ZZZZZZZZZZZZ/ YYYYYYYYYY/ ZZZZZZZZZZZ	YYYY-MM-DD	-	No	Mild	None	Ongoing	Not related	Not related	xxxxxxxxxxxxxx

SOC: System Organ Class, AE No.: Adverse Event Number; CTC: Common Terminology Criteria; NK: Not Known

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat this listing for:
21.2 Adverse Events (Grades 3 and /or 4)
21.3 Related Adverse Events (Grades 3 and /or 4)

Listing 21.2: Adverse Events (Grades 3 and /or 4)
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 21.3: Related Adverse Events (Grades 3 and /or 4)
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 22.1: Concomitant Medication
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Taken any con med?	Con med No.	Preferred Term/ Medication Name	Dose	Unit	Frequency	Route	Start Date (YYYY-MM-DD)	Stop Date (YYYY-MM-DD)	Indication	Continuing?	Stopped Prior to Apheresis?
xx-xxxxxx	Yes	16	ZZZZZZZZZZZZ/ YYYYYYYYYY	xxx	MG	PRN	Oral	YYYY-MM-DD	-	AE	Yes	No
		19	ZZZZZZZZZZZZ/ YYYYYYYYYY	x	tab	Twice a day	Oral	YYYY-MM-DD	YYYY-MM-DD	AE	No	No
		20	ZZZZZZZZZZZZ/ YYYYYYYYYY	x	tab	Twice a day	Oral	YYYY-MM-DD	YYYY-MM-DD	AE	No	No
		22	ZZZZZZZZZZZZ/ YYYYYYYYYY	xx	mg	PRN	Oral	YYYY-MM-DD	-	AE	Yes	Yes
		24	ZZZZZZZZZZZZ/ YYYYYYYYYY	x	TAB	Once daily	Oral	YYYY-MM-DD	-	AE	Yes	No
		4	ZZZZZZZZZZZZ/ YYYYYYYYYY	x	tab	Once daily	Oral	YYYY-MM-DD	-	Gen. Wellbeing	Yes	No
		5	ZZZZZZZZZZZZ/ YYYYYYYYYY	x	tab	Once daily	Oral	YYYY-MM-DD	-	Gen. Wellbeing	Yes	No

Con med No. = Concomitant Medication number
Con med = Concomitant Medication; NK = Not Known

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 22.2: Concomitant Procedures and Therapies
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Procedure No.	Procedure Date (YYYY-MM-DD)	Procedure	Indication	Adverse Event	Medical History
xx-xxxxxx	1	YYYY-MM-DD	xxxxxxxxxxxxxxxxx	AE	xxxxxxxxxxxxxxx	
	2	YYYY-MM-DD	xxxxxxxxxxxxxxxxx	Other – xxxxx		
	3	YYYY-MM-DD	xxxxxxxxxxxxxxxxx	AE	xxxxxxxxxxxxxxx	
	4	YYYY-MM-DD	xxxxxxxxxxxxxxxxx	AE	xxxxxxxxxxxxxxx	
	5	YYYY-MM-DD	xxxxxxxxxxxxxxxxx	AE	xxxxxxxxxxxxxxx	
	6	YYYY-MM-DD	xxxxxxxxxxxxxxxxx	AE	xxxxxxxxxxxxxxx	

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 23.1: Laboratory – Hematology
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Date of Collection (YYYY-MM-DD)	Time of Collection (HH:MM)	Variable	Result	Unit	LLN	ULN	Flag*	Clin. Sig.**	Comment
xx-xxxxxx	Screening 1	YYYY-MM-DD	HH:MM	Hemoglobin	xx.x	g/dL	12.5	17.0	-	-	
				Hematocrit	xx.x	%	36.0	50.0	-	-	
				Platelet Count	xx.x	10^3/uL	140	415	-	-	
				Red Blood Cells	xx.x	10^6/uL	4.10	5.60	-	-	
				White Blood Cells	xx.x	10^3/uL	4.00	10.50	-	-	
				Neutrophils Absolute	xx.x	10^3/uL	1.80	7.80	-	-	
				Neutrophils Percent	xx.x	%	40	74	-	-	
				Lymphocytes Absolute	xx.x	10^3/uL	0.70	4.50	-	-	
				Lymphocytes Percent	xx.x	%	14	46	-	-	
				Monocytes Absolute	xx.x	10^3/uL	0.10	1.00	-	-	
				Monocytes Percent	xx.x	%	4	13	-	-	
				Eosinophils Absolute	xx.x	10^3/uL	0.00	0.40	-	-	
				Eosinophils Percent	xx.x	%	0	7	-	-	
				Basophils Absolute	xx.x	10^3/uL	0.00	0.20	-	-	
				Basophils Percent	xx.x	%	0	3	-	-	

LLN=Lower Limit of Normal; ULN=Upper Limit of Normal
* Flag: H=High, L=Low
** Clinical Significance: CS=Clinically Significant, NCS: Not Clinically Significant

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat this listing for:
23.2 Laboratory – Hematology (Abnormal only)
24.1 Laboratory – Biochemistry
24.2 Laboratory – Biochemistry (Abnormal only)
25 Laboratory – Lymphocyte phenotype

Listing 23.2: Laboratory – Hematology (Abnormal only)
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 24.1: Laboratory – Biochemistry
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 24.2: Biochemistry (Abnormal only)
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 25.1: Lymphocyte phenotype
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 25.2: Thymopoieisis
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Date of Collection (YYYY-MM-DD)	Time of Collection (HH:MM)	Variable	Result	Unit	LLN	ULN	Flag*	Comment
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	HH:MM	CCI						

LLN=Lower Limit of Normal; ULN=Upper Limit of Normal
* Flag: H=High, L=Low

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat this listing for:
25.3 Laboratory – Inflammation
25.4 Laboratory – Maturation

Listing 25.3: Inflammation
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 25.4: Maturation
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 26: Laboratory – HIV-1 RNA
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

		Roche Assay			Abbott Assay			
Subject Number	Visit	Date of Collection (YYYY-MM-DD)	Result (Copies/mL)	Flag*	Date of Collection (YYYY-MM-DD)	Result (Copies/mL)	Flag*	Comments
xx-xxxxxx	Screening 1	YYYY-MM-DD	xxxxxxxx	Indeterminate	YYYY-MM-DD	xxxxxxxx	-	XXXXXXXXXXXXXXXXXX
	Screening 2	YYYY-MM-DD	xxxxxxxx	Indeterminate	YYYY-MM-DD	xxxxxxxx	-	XXXXXXXXXXXXXXXXXX
	CD4+ Apheresis	YYYY-MM-DD	xxxxxxxx	Indeterminate	-	-	-	
	G-CSF 1	YYYY-MM-DD	xxxxxxxx	Indeterminate	-	-	-	
	Infusion (Day 0)	YYYY-MM-DD	xxxxxxxx	Indeterminate	YYYY-MM-DD	xxxxxxxx	-	XXXXXXXXXXXXXXXXXX
	Week 1	YYYY-MM-DD	xxxxxxxx	Indeterminate	-	-	-	
	Week 2	YYYY-MM-DD	xxxxxxxx	Indeterminate	-	-	-	

* Flag: H=High, L=Low

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 27.1: Vital Signs
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Date Performed (YYYY-MM-DD)	Time Performed (HH:MM)	Time Point	Weight (kg)	Systolic Blood Pressure (mmHg)	Diastolic Blood Pressure (mmHg)	Pulse Rate (beats/min)	Temperature (°C)	Oxygen Saturation (%)
xx-xxxxxx	Screening 1	YYYY-MM-DD	HH:MM		xx.x	xxx	xx	xx	xxL	-
	CD4+ Apheresis	YYYY-MM-DD	HH:MM			xxx	xx	xxL	xx.x	xx
	G-CSF 1	YYYY-MM-DD	HH:MM			xxx	xx	xxL	xx.x	xx
	G-CSF 5 CD34+ Apheresis	YYYY-MM-DD	HH:MM			xxx	xx	xxL	xx.x	xx
	Infusion (Day 0)	YYYY-MM-DD	HH:MM	Pre		xxx	xx	xxL	xxL	xx
			HH:MM	T1		xxx	xx			
			HH:MM	T3		xxx	xx			
			HH:MM	T5		xxx	xx			
			HH:MM	T7		xxx	xx			
			HH:MM	H1		xxx	xx			
			HH:MM	H2		xxx	xx			
;			HH:MM	H3		xxx	xx			
			HH:MM	H4		xxx	xx			
			HH:MM	H5		xxx	xx			
		YYYY-MM-DD	HH:MM	H6		xxx	xxH	xx	xx.x	xx
		YYYY-MM-DD	HH:MM	H7		xxx	xxH	xx	xx.x	xx
		YYYY-MM-DD	HH:MM	H8		xxx	xx	xx	xxL	xx
		YYYY-MM-DD	HH:MM	H9		xxx	xx	xx	xxL	xx
		YYYY-MM-DD	HH:MM	H10		xxx	xx	xx	xxL	xx
	Week 1	YYYY-MM-DD	HH:MM			xxx	xxH	xx	xxL	xx
	Week 2	YYYY-MM-DD	HH:MM			xxx	xx	xx	xx	xx
	Week 4	YYYY-MM-DD	HH:MM			xxx	xx	xx	xx	xx
	Week 6	YYYY-MM-DD	HH:MM			xxx	xx	xx	xx	xx
	Week 8	YYYY-MM-DD	HH:MM			xxx	xx	xx	xx	xx
	Week 12	YYYY-MM-DD	HH:MM			xxx	xx	xx	xx	xx

Flag: H=High, L=Low

Programming Note: Repeat this listing for:
27.2 Abnormal Vital Signs

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 27.2: Abnormal Vital Signs
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 28.1: Physical Examination
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Date of Examination (YYYY-MM-DD)	System	Abnormal; specify	Clinically Significant?
xx-xxxxxx	Screening 1	YYYY-MM-DD	General Appearance		
			Dermatologic		
			Hematologic		
			HEENT n		
			Respiratory		
			Cardiovascular n		
			Musculoskeletal n		
			Gastrointestinal n	skin rash resolved	No
			Pelvic/Genitourinary		
			Anal/Rectal nk		
			Detailed Neurologic		
			General Appearance		
			Dermatologic		
			Other	xxxxxxxxxxxxxxxxxxxxxx	No
	G-CSF 1	YYYY-MM-DD	General Appearance		
	Infusion (Day 0)	YYYY-MM-DD	General Appearance		
	Week 1	YYYY-MM-DD	General Appearance		
	Week 2	YYYY-MM-DD	General Appearance		
	Week 4	YYYY-MM-DD	General Appearance		
	Week 6	YYYY-MM-DD	General Appearance		

HEENT: Head Eyes Ears Nose Throat

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Programming Note: Repeat this listing for:
28.2 Abnormal Physical Examination

Listing 28.2: Abnormal: Physical Examination
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 29.1: Cal-1 Integration Analysis
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Collection Date (YYYY-MM-DD)	Analysis Performed? *	Are there any CS abnormalities on PE or CBC/diff? (mono-clonality)
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Not Required	
	Week 12	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
	Week 24	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
	Week 48	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Not Required	
	Week 12	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
	Week 24	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
	Week 48	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Not Required	
	Week 12	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
	Week 24	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
	Week 48	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Not Required	
	Week 12	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
	Week 24	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
	Week 48	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	
	Early discontinuation / withdrawal	YYYY-MM-DD	Not done (Cal-1 Marking – PB <1%)	

* Integration analysis performed only when Cal-1 Marking is >= 1%;
Not done (Cal-1 Marking – PB<1%); No predominant clone detected; Predominant mono-clonality detected or Predominant oligo-clonality detected;
If predominant clone detected, batch analysis of previous samples ('Baseline' or 'Not done' due to <1% marking) will be performed.

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 29.2: Cal-1 Integration Analysis (Re-test)
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

		Predominant mono-clonality confirmed				Predominant oligo-clonality confirmed			
Subject Number	Visit	Retest Date (YYYY-MM-DD)	Sequencing performed	Prominent integration site location	Are there any CS abnormalities on PE or CBC/diff? (mono-clonality)	Retest Date (YYYY-MM-DD)	Sequencing performed	Prominent integration site location	Clustering detected/ Site
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	Yes – xxxxxxxxxxx
	Week 12	YYYY-MM-DD	No	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
	Week 24	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
	Week 48	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
	Week 12	YYYY-MM-DD	No	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
	Week 24	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
	Week 48	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
	Week 12	YYYY-MM-DD	No	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
	Week 24	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
	Week 48	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	No
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	
	Week 12	YYYY-MM-DD	No	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	
	Week 24	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	
	Week 48	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	
	Early discontinuation / withdrawal	YYYY-MM-DD	Yes	xxxxxxxxxxxxx		YYYY-MM-DD		xxxxxxxxxxxxx	

* Integration analysis performed only when Cal-1 Marking is >= 1%;

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 30: Replication Competent Lentivirus (RCL)
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Collection Date (YYYY-MM-DD)	VSV-G PCR Signal *	Number of copies of VSV-G DNA **	Retest Sample Collection Date (YYYY-MM-DD)	Positive VSV-G PCR Signal Confirmed?	Number of copies of VSV-G DNA ***	AE# for Positive VSV-G PCR Signal
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Negative	-	-	-	-	
	Week 12	YYYY-MM-DD	Negative	-	-	-	-	
	Week 24	YYYY-MM-DD	Negative	-	-	-	-	
	Week 48	YYYY-MM-DD	Negative	-	-	-	-	
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Negative	-	-	-	-	
	Week 12	YYYY-MM-DD	Negative	-	-	-	-	
	Week 24	YYYY-MM-DD	Negative	-	-	-	-	
	Week 48	YYYY-MM-DD	Negative	-	-	-	-	
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Negative	-	-	-	-	
	Week 12	YYYY-MM-DD	Negative	-	-	-	-	
	Week 24	YYYY-MM-DD	Negative	-	-	-	-	
	Week 48	YYYY-MM-DD	Negative	-	-	-	-	
xx-xxxxxx	Infusion (Day 0)	YYYY-MM-DD	Negative	-	-	-	-	
	Week 12	YYYY-MM-DD	Negative	-	-	-	-	
	Week 24	YYYY-MM-DD	Negative	-	-	-	-	
	Week 48	YYYY-MM-DD	Negative	-	-	-	-	

* VSV-G PCR Signal: indicated by a blank when results are pending.
** Number of VSV-G DNA copies per 0.2ug peripheral blood DNA
*** Number of VSV-G DNA copies per 0.2ug peripheral blood DNA on re-test samples

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 31: HIV-1 Tropism Assay
Protocol: CAL-USA-11
Population: All Enrolled

Cohort: Cohort 1

Subject Number	Visit	Collection Date/Time (YYYY-MM-DD)	Result *	Clinical Significant	Comment
xx-xxxxxx	Screening 1	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
	Week 12	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
	Week 48	YYYY-MM-DD/ HH:MM	unreportable	No	xxxxxxxxxx
xx-xxxxxx	Screening 1	YYYY-MM-DD/ HH:MM	R5	No	xxxxxxxxxx
	Week 12	YYYY-MM-DD/ HH:MM	R5	No	xxxxxxxxxx
xx-xxxxxx	Screening 1	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
	Week 12	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
	Week 48	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
xx-xxxxxx	Screening 1	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
	Week 24	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
xx-xxxxxx	Screening 1	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
	Week 12	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
xx-xxxxxx	Screening 1	YYYY-MM-DD/ HH:MM	R5	No	xxxxxxxxxx
	Week 12	YYYY-MM-DD/ HH:MM	R5	No	xxxxxxxxxx
xx-xxxxxx	Screening 1	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx
xx-xxxxxx	Screening 1	YYYY-MM-DD/ HH:MM	r5	No	xxxxxxxxxx

* Results: CCR5 (R5), CXCR4 (X4) or dual mixed (R5/X4)

Clinical cut-off date: DDMMMYYYY
Program: filepath_name, Output: filepath_name Created: DDMMMYYYY HH:MM

Listing 32.1: C46 Immunogenicity – Humoral Response
Protocol: CAL-USA-11
Population: All Enrolled

Listing 32.2: C46 Immunogenicity – Cellular Response
Protocol: CAL-USA-11
Population: All Enrolled