**VISIT [X] CRF (Time since Infusion):**

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| [ ]  Day 0 | [ ]  6 months | [ ]  1 year | [ ]  1.5 years | [ ]  2 years |
| [ ]  3 years | [ ]  4 years  | [ ]  5 years | [ ]  6 years | [ ]  7 years |
| [ ]  8 years | [ ]  9 years  | [ ]  10 years | [ ]  11 years | [ ]  12 years |
| [ ]  13 years | [ ]  14 years  | [ ]  15 years |  |  |

Date sample drawn: \_\_\_ \_\_\_ / \_\_\_ \_\_\_ \_\_\_ / \_\_\_ \_\_\_ \_\_\_ \_\_\_ (dd/mmm/yyyy)

Current CBC (use other CRF):

Current Hemoglobin analysis (use other CRF):

Tissue monitoring site:

[ ]  Peripheral blood

[ ]  Bone marrow

 [ ] Cells sorted?

 [ ]  Yes [ ]  No

 [ ]  Immunophenotype

**For Integrating Vectors: Vector Copy Number per diploid genome (VCN):**

[ ]  Blood VCN\_\_\_\_\_\_\_\_ **CD3 depleted?:** [ ]  Yes [ ]  No

[ ]  VCN in Specific Lineages: CD3+\_\_\_\_\_\_\_ CD19+\_\_\_\_\_\_ CD15+\_\_\_\_\_\_\_GPA+\_\_\_\_\_\_\_\_

 CD14+\_\_\_\_\_\_

[ ]  Percentage of Blood Cells Positive for Integrating Vector: PBMC \_\_\_\_\_\_\_\_\_

BFU-E\_\_\_\_\_\_\_\_\_\_

[ ]  Bone Marrow VCN: BMMC\_\_\_\_\_\_ CD34+\_\_\_\_\_\_\_\_ CFC\_\_\_\_\_\_

[ ]  Percentage of Bone Marrow Cells Positive for Integrating Vector: BMMC\_\_\_\_\_\_

CD34+\_\_\_\_\_\_\_\_ CFC\_\_\_\_\_\_

* Blood VCN should be performed at all of the visits listed.
* VCN in specific lineages should be performed at one year post-transplant.
* Measuring the percentage of blood cells positive for vector should be performed at one and two years post-transplant
* A bone marrow aspirate to measure VCN and the proportion of transduced cells is supplemental information that can be performed at one and two years post-transplant

**For Site-Specific Nucleases: Gene editing frequency (% ) on-target**

[ ]  Blood: **CD3 depleted?:** [ ]  Yes [ ]  No

Percent Conversion \_\_\_\_\_\_\_\_ or On-target Indels\_\_\_\_\_ and Off-target Indels\_\_\_\_\_\_\_

[ ]  Percent conversion in Specific Blood Lineages: CD3+\_\_\_\_\_\_\_ CD19+\_\_\_\_\_\_

CD15+\_\_\_\_\_\_\_GPA+\_\_\_\_\_\_\_\_ CD14+\_\_\_\_\_\_

[ ]  On-target Indels in Specific Blood Lineages: CD3+\_\_\_\_\_\_\_ CD19+\_\_\_\_\_\_

D15+\_\_\_\_\_\_\_GPA+\_\_\_\_\_\_\_\_ CD14+\_\_\_\_\_\_

[ ]  Off-target Indels in Specific Blood Lineages: CD3+\_\_\_\_\_\_\_ CD19+\_\_\_\_\_\_

CD15+\_\_\_\_\_\_\_GPA+\_\_\_\_\_\_\_\_ CD14+\_\_\_\_\_\_

Bone Marrow:

[ ]  BMMC Percent Conversion \_\_\_\_\_\_\_\_ or On-target Indels\_\_\_\_\_ and Off-target Indels\_\_\_\_\_\_\_

[ ]  CD34+ Percent Conversion \_\_\_\_\_\_\_\_ or On-target Indels\_\_\_\_\_ and Off-target Indels\_\_\_\_\_\_\_

[ ]  CFC Percent Conversion \_\_\_\_\_\_\_\_ or On-target Indels\_\_\_\_\_ and Off-target Indels\_\_\_\_\_\_\_\_

* Gene editing frequency in blood samples should be performed at all of the visits listed.
* Analysis of specific lineages should be performed at one-year post-transplant.
* A bone marrow aspirate to measure VCN and the proportion of transduced cells is supplemental information that can be performed at one- and two-years post-transplant

Method

[ ]  RFLP

[ ]  Sanger sequencing

[ ]  Western Blot

[ ]  NGS

[ ]  TIDE3

[ ]  Reporter gene assay

 [ ]  Specify: \_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

[ ]  Other

Is the on-target indel frequency associated with an adverse outcome? (e.g. thalassemia phenotype)

[ ]  Yes [ ]  No

If yes, describe\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_