**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\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_