Gene Therapy Product

Registry Use Only

Sequence Number:

Date Received:

CIBMTR Center Number: ___ ___ ___ ___ ___

CIBMTR Research ID: ___ ___ ___ ___ ___ ___ ___ ___ ___ ___

Event date: ___ ___ ___ ___ — ___ ___ — ___ ___
YYYY MM DD

CIBMTR Center Number: ___ ___ ___ ___ ___ CIBMTR Research ID: ___ ___ ___ ___ ___ ___ ___ ___ ___ ___

Product Identification

1. Name of product
   - ☐ Betibeglogene autotemcel (Zynteglo ®) – ***Go to question 2***
   - ☐ Elivaldogene autotemcel (Skysona ®) – ***Go to question 2***
   - ☐ Exagamglogene autotemcel – ***Go to question 2***
   - ☐ Other name – ***Go to question 3***

2. Is the product out of specification? *(only for commercially available products)*
   - ☐ Yes
   - ☐ No

3. Specify the identifier(s) associated with this gene therapy product *(check all that apply)*
   - ☐ Gene therapy product ID – ***Go to question 4***
   - ☐ Batch number – ***Go to question 5***
   - ☐ Lot number – ***Go to question 6***

4. Gene therapy product ID: _____

5. Batch number: _____

6. Lot number: _____

Product Collection

7. Peripheral blood CD34+ cell count prior to first dose of cytokine for mobilization *(baseline)*
   - ☐ Done – ***Go to question 8***
   - ☐ Not done – ***Go to question 9***

8. Baseline number of peripheral blood CD34+ cells: ___ ___ ___ . ___ ___ /µL (mm^3)

9. Peripheral blood CD34+ cell count on Day 1 apheresis, just prior to start of the procedure
   - ☐ Done – ***Go to question 10***
   - ☐ Not done – ***Go to question 11***

10. Day 1 pre-apheresis number of peripheral blood CD34+ cells: ___ ___. ___ __/µL (mm^3)

11. Date of first collection for this mobilization:___ ___ ___ ___ — ___ ___ — ___ ___
    YYYY MM DD

12. What agents were used to mobilize the recipient for this HCT? *(check all that apply)*
    - ☐ G-CSF (TBO-filgrastim, filgrastim, Granix, Neupogen) – ***Go to question 14***
    - ☐ GM-CSF (sargramostim, Leukine) – ***Go to question 14***
    - ☐ Pegylated G-CSF (pegfilgrastim, Neulasta) – ***Go to question 14***
    - ☐ Motixafortide (Aphexda) – ***Go to question 14***
    - ☐ Plerixafor (Mozobil) – ***Go to question 14***
    - ☐ Combined with chemotherapy – ***Go to question 14***
    - ☐ Anti-CD20 (rituximab, Rituxan) – ***Go to question 14***
    - ☐ Other agent – ***Go to question 13***

13. Specify other agent: _____

14. Was more than one day of collection required?
    - ☐ Yes – ***Go to question 15***
    - ☐ No – ***Go to question 16***

15. Specify the number of subsequent days of collection: ___ ___

Product Processing / Manipulation

16. Where was the gene therapy product manufactured / processed?
    - ☐ Cell processing laboratory at the same center as the product is being infused – ***Go to question 20***
    - ☐ Cell processing laboratory off site – ***Go to question 20***
    - ☐ Pharmaceutical / biotech company – ***Go to question 17***
    - ☐ Other site – ***Go to question 19***

17. Specify pharmaceutical / biotech company
    - ☐ Aruvant – ***Go to question 20***
    - ☐ Avrobio – ***Go to question 20***
    - ☐ Beam – ***Go to question 20***
    - ☐ Bluebird Bio – ***Go to question 20***
    - ☐ CRISPR – ***Go to question 20***
    - ☐ Editas – ***Go to question 20***
    - ☐ Graphite Bio – ***Go to question 20***
    - ☐ Mustang Bio– ***Go to question 20***
    - ☐ Orchard Therapeutics – ***Go to question 20***
    - ☐ Rocket Pharmaceuticals – ***Go to question 20***
    - ☐ Vertex– ***Go to question 20***

☐ Other pharmaceutical / biotech company – ***Go to question 18***

18. Specify other pharmaceutical / biotech company: _____ – ***Go to question 20***

19. Specify other site: _____

20. Specify the portion of the gene therapy product manipulated

☐ Entire product - ***Go to question 21***

☐ Portion of product - ***Go to question 21***

☐ Unknown – ***Go to question 21***

21. Was the manipulated product cryopreserved?

☐ Yes

☐ No

22. Was the unmanipulated ("back-up") portion of the product cryopreserved?

☐ Yes

☐ No

23. Specify the type(s) of genetic manipulation *(check all that apply)*

☐ Ex vivo transduction – ***Go to question 24***

☐ Gene editing – ***Go to question 28***

☐ Other genetic manipulation – ***Go to question 32***

Ex Vivo Transduction

24. Type of vector

☐ Adeno-associated virus (AAV) – ***Go to question 26***

☐ Lentivirus – ***Go to question 26***

☐ Retrovirus– ***Go to question 26***

☐ Transposon– ***Go to question 26***

☐ Other type of vector – ***Go to question 25***

☐ Unknown – ***Go to question 26***

25. Specify other type of vector:_____

26. Specify the transgene

☐ ABCD1 – ***Go to question 28***

☐ Beta globin (wild type, T87Q, AS3) – ***Go to question 28***

- ☐ Gamma globin (G16D, other) – ***Go to question 28***
- ☐ shRNA/siRNA to BCL11A – ***Go to question 28***
- ☐ Other transgene – ***Go to question 27***
- ☐ Unknown – ***Go to question 28***

27. Specify other transgene:_____

Gene Editing

28. Methodology
    - ☐ Base editor – **Go to question 30**
    - ☐ Cas protein – **Go to question 30**
    - ☐ Transcription activator-like effector nucleases (TALENs) – ***Go to question 30***
    - ☐ Zinc finger nucleases (ZFNs) – ***Go to question 30***
    - ☐ Other methodology – ***Go to question 29***
    - ☐ Unknown – ***Go to question 30***

29. Specify other methodology: _____

30. Specify the gene target
    - ☐ BCL11A – ***Go to question 32***
    - ☐ Beta globin – ***Go to question 32***
    - ☐ Gamma globin – ***Go to question 32***
    - ☐ Other gene target – ***Go to question 31***
    - ☐ Unknown – ***Go to question 32***

31. Specify other gene target: ____

Other Genetic Manipulation

32. Specify other genetic manipulation: _____

Product Analysis (All Products)

Copy questions 33 - 71 to report multiple instances of Product Analysis

33. Specify the timepoint in the product preparation phase that the product was analyzed
    - ☐ Fresh manipulated product

☐ Prior to cryopreservation of manipulated product plus additives

☐ Post-thaw of cryopreserved manipulated product

34. Date of product analysis: ___ ___ ___ ___ — ___ ___ — ___ ___
YYYY MM DD

35. Total volume of product plus additives: ___ ___ ___ ___ ___ • ___mL

In this section, report the total number of cells (not cells per kilogram) and do not correct for viability.

36. CD34+ cells

☐ Done – ***Go to question 37***

☐ Not done – ***Go to question 42***

37. Total number of CD34+ cells: ___ ___ ___ ___ • ___ ___ x 10 ___ ___

38. Viability of CD34+ cells

☐ Done – ***Go to question 39***

☐ Not done – ***Go to question 42***

☐ Unknown – ***Go to question 42***

39. Viability of CD34+ cells: ___ ___ ___ %

40. Method of testing CD34+ cell viability

☐ Flow cytometry based – ***Go to question 42***

☐ Trypan blue – ***Go to question 42***

☐ Other method – ***Go to question 41***

41. Specify other method: _____

42. Other cell type

☐ Done – ***Go to question 43***

☐ Not done – ***Go to question 68***

The number of other cells reported in Question 43 will enable the appropriate number of instances (up to four) in questions 44-67.

43. Specify the total number of other cell types tested: ___

Other Cell Type 1

44. Specify other cell type: _____

45. Total number of cells: ___ ___ ___ ___ • ___ ___ x 10 ___ ___

46. Viability of cells

- ☐ Done **– *Go to question 47***
- ☐ Not done **– *Go to question 50***
- ☐ Unknown – ***Go to question 50***

47. Viability of cells: ___ ___ ___ %

48. Method of testing cell viability

- ☐ Flow cytometry based ***- Go to question 50***
- ☐ Trypan blue - ***Go to question 50***
- ☐ Other method – ***Go to question 49***

49. Specify other method: _____

Other Cell Type 2

50. Specify other cell type: _____

51. Total number of cells: ___ ___ ___ ___ • ___ ___ x 10 ___ ___

52. Viability of cells

- ☐ Done **– *Go to question 53***
- ☐ Not done **– *Go to question 56***
- ☐ Unknown – ***Go to question 56***

53. Viability of cells: ___ ___ ___ %

54. Method of testing cell viability

- ☐ Flow cytometry based ***- Go to question 56***
- ☐ Trypan blue - ***Go to question 56***
- ☐ Other method – ***Go to question 55***

55. Specify other method: _____

Other Cell Type 3

56. Specify other cell type: _____

57. Total number of cells: ___ ___ ___ ___ • ___ ___ x 10 ___ ___

58. Viability of cells

- ☐ Done **– *Go to question 59***
- ☐ Not done **– *Go to question 68***
- ☐ Unknown – ***Go to question 68***

59. Viability of cells: ___ ___ ___ %

60. Method of testing cell viability

- ☐ Flow cytometry based **- *Go to question 68***
- ☐ Trypan blue - ***Go to question 68***
- ☐ Other method – ***Go to question 61***

61. Specify other method: _____

Other Cell Type 4

62. Specify other cell type: _____

63. Total number of cells: ___ ___ ___ ___ • ___ ___ x 10 ___ ___

64. Viability of cells

- ☐ Done **– *Go to question 65***
- ☐ Not done **– *Go to question 68***
- ☐ Unknown – ***Go to question 68***

65. Viability of cells: ___ ___ ___ %

66. Method of testing cell viability

- ☐ Flow cytometry based **- *Go to question 68***
- ☐ Trypan blue - ***Go to question 68***
- ☐ Other method – ***Go to question 67***

67. Specify other method: _____

68. Vector copy number (VCN; number of vector copies per diploid genome) in the infused product

- ☐ Known – ***Go to question 69***
- ☐ Unknown – ***Go to question 70***

69. VCN: ___ ____ • ___

70. Percentage of gene edited cells in the infused product

- ☐ Known – ***Go to question 71***
- ☐ Unknown – ***Go to question 72***

71. Percentage of gene edited cells ___ ___ ___ %

Copy questions 33-71 to report multiple instances of Product Analysis

Product Infusion

72. Date of manipulated product infusion: ___ ___ ___ ___ — ___ ___ — ___ ___
YYYY MM DD

73. Was the entire volume of product infused?

- ☐ Yes – ***Go to question 76***
- ☐ No – ***Go to question 74***

74. Specify what happened to the reserved portion

- ☐ Discarded – ***Go to question 76***
- ☐ Cryopreserved for future use – Go to ***question 76***
- ☐ Other fate – ***Go to question 75***

75. Specify other fate:_____

76. Specify the route of manipulated product infusion

- ☐ Intravenous – ***Go to question 78***
- ☐ Other route of infusion – ***Go to question 77***

77. Specify other route of infusion: _____

78. Was the unmanipulated (“back-up”) product infused?

- ☐ Yes – ***Go to question 79***
- ☐ No – ***Go to End of Form***

79. Date of unmanipulated product infusion: ___ ___ ___ ___ — ___ ___ — ___ ___
YYYY MM DD

80. Specify the route of unmanipulated product infusion

☐ Intravenous – ***Go to End Form***

☐ Other route of infusion – ***Go to question 81***

81. Specify other route of infusion: _____