



Gene Therapy Product

Registry Use Only

Sequence Number:

Date Received:

CIBMTR Center Number: _____

CIBMTR Research ID: _____

Event date: _____
 YYYY MM DD'

HCT type (check only one)

- Autologous
- Allogeneic, unrelated
- Allogeneic, related

Product type (check only one)

- Bone marrow
- PBSC
- Single cord blood unit
- Other product

Specify: _____

Product Identification

1. Name of product
 - Betibeglogene autotemcel (Zynteglo ®)
 - Elivaldogene autotemcel (Skysona ®)
 - Exagamglogene autotemcel
 - Other name

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

3. Gene therapy product ID: _____

4. Batch number: _____

5. Lot number: _____

Product Collection

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

7. Baseline number of peripheral blood CD34+ cells: _____ ▪ _____ / μ L (mm^3)

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

9. Day 1 pre-apheresis number of peripheral blood CD34+ cells: _____ ▪ _____ / μ L (mm^3)

10. Date of first collection for this mobilization: _____ – _____ – _____

YYYY MM DD

11. Was more than one collection required?
 - Yes – **Go to question 12**

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No – **Go to question 13**

12. Specify the number of subsequent days of collection: _____

Product Processing / Manipulation

13. Where was the gene therapy product manufactured / processed?

Cell processing laboratory at the same center as the product is being infused – **Go to question 17**

Cell processing laboratory off site – **Go to question 17**

Pharmaceutical / biotech company – **Go to question 14**

Other site – **Go to question 16**

14. Specify pharmaceutical / biotech company

Aruvant – **Go to question 17**

Avrobio – **Go to question 17**

Beam – **Go to question 17**

Bluebird Bio – **Go to question 17**

CRISPR – **Go to question 17**

Editas – **Go to question 17**

Graphite Bio – **Go to question 17**

Mustang Bio– **Go to question 17**

Orchard Therapeutics – **Go to question 17**

Rocket Pharmaceuticals – **Go to question 17**

Vertex– **Go to question 17**

Other pharmaceutical / biotech company – **Go to question 15**

15. Specify other pharmaceutical / biotech company: _____ – **Go to question 17**

16. Specify other site: _____

17. Specify the portion of the gene therapy product manipulated

Entire product - **Go to question 18**

Portion of product - **Go to question 18**

Unknown – **Go to question 18**

18. Was the manipulated product cryopreserved?

Yes

No

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19. Was the unmanipulated (“back-up”) portion of the product cryopreserved?

- Yes
- No

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

- Ex vivo transduction – **Go to question 21**
- Gene editing – **Go to question 25**
- Other genetic manipulation – **Go to question 29**

Ex Vivo Transduction

21. Type of vector

- Adeno-associated virus (AAV) – **Go to question 23**
- Lentivirus – **Go to question 23**
- Retrovirus – **Go to question 23**
- Transposon – **Go to question 23**
- Other type of vector – **Go to question 22**
- Unknown – **Go to question 23**

22. Specify other type of vector: _____

23. Specify the transgene

- ABCD1 – **Go to question 25**
- Beta globin (wild type, T87Q, AS3) – **Go to question 25**
- Gamma globin (G16D, other) – **Go to question 25**
- shRNA/siRNA to BCL11A – **Go to question 25**
- Other transgene – **Go to question 24**
- Unknown – **Go to question 25**

24. Specify other transgene: _____

Gene Editing

25. Methodology

- Base editor – **Go to question 27**
- Cas protein – **Go to question 27**
- Transcription activator-like effector nucleases (TALENs) – **Go to question 27**
- Zinc finger nucleases (ZFNs) – **Go to question 27**

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Other methodology – **Go to question 26**

Unknown – **Go to question 27**

26. Specify other methodology: _____

27. Specify the gene target

BCL11A – **Go to question 29**

Beta globin – **Go to question 29**

Gamma globin – **Go to question 29**

Other gene target – **Go to question 28**

Unknown – **Go to question 29**

28. Specify other gene target: _____

Other Genetic Manipulation

29. Specify other genetic manipulation: _____

Product Analysis (All Products)

Copy questions 30-68 to report multiple instances of Product Analysis

30. 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

31. Date of product analysis: _____

YYYY

MM

DD

32. 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.

33. CD34+ cells

Done – **Go to question 34**

Not done – **Go to question 39**

34. Total number of CD34+ cells: _____ • _____ x 10 _____

- 35. Viability of CD34+ cells
 - Done – **Go to question 36**
 - Not done – **Go to question 39**
 - Unknown – **Go to question 39**

36. Viability of CD34+ cells: _____ %

- 37. Method of testing CD34+ cell viability
 - Flow cytometry based – **Go to question 39**
 - Trypan blue – **Go to question 39**
 - Other method – **Go to question 38**

38. Specify other method: _____

- 39. Other cell type
 - Done – **Go to question 40**
 - Not done – **Go to question 65**

The number of other cells reported in Question 40 will enable the appropriate number of instances (up to four) in questions 41-64.

40. Specify the total number of other cell types tested: _____

Other Cell Type 1

41. Specify other cell type: _____

42. Total number of cells: _____ • _____ x 10 _____

- 43. Viability of cells
 - Done – **Go to question 44**
 - Not done – **Go to question 47**
 - Unknown – **Go to question 47**

44. Viability of cells: _____ %

- 45. Method of testing cell viability
 - Flow cytometry based - **Go to question 47**
 - Trypan blue - **Go to question 47**
 - Other method – **Go to question 46**

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46. Specify other method: _____

Other Cell Type 2

47. Specify other cell type: _____

48. Total number of cells: _____ • _____ x 10 _____

49. Viability of cells

- Done – **Go to question 50**
- Not done – **Go to question 53**
- Unknown – **Go to question 53**

50. Viability of cells: _____ %

51. Method of testing cell viability

- Flow cytometry based - **Go to question 53**
- Trypan blue - **Go to question 53**
- Other method – **Go to question 52**

52. Specify other method: _____

Other Cell Type 3

53. Specify other cell type: _____

54. Total number of cells: _____ • _____ x 10 _____

55. Viability of cells

- Done – **Go to question 56**
- Not done – **Go to question 65**
- Unknown – **Go to question 65**

56. Viability of cells: _____ %

57. Method of testing cell viability

- Flow cytometry based - **Go to question 65**
- Trypan blue - **Go to question 65**
- Other method – **Go to question 58**

58. Specify other method: _____

Other Cell Type 4

59. Specify other cell type: _____

60. Total number of cells: _____ • _____ x 10 _____

61. Viability of cells

- Done – **Go to question 62**
- Not done – **Go to question 65**
- Unknown – **Go to question 65**

62. Viability of cells: _____ %

63. Method of testing cell viability

- Flow cytometry based - **Go to question 65**
- Trypan blue - **Go to question 65**
- Other method – **Go to question 64**

64. Specify other method: _____

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

- Known – **Go to question 66**
- Unknown – **Go to question 67**

66. VCN: _____ • _____

67. Percentage of gene edited cells in the infused product

- Known – **Go to question 68**
- Unknown – **Go to question 69**

68. Percentage of gene edited cells _____ %

Product Infusion

69. Date of manipulated product infusion: _____ – _____ – _____
 YYYY MM DD

70. Specify the route of manipulated product infusion

- Intravenous – **Go to question 72**
- Other route of infusion – **Go to question 71**

CIBMTR Center Number: _____ CIBMTR Research ID: _____

71. Specify other route of infusion: _____

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

Yes – **Go to question 73**

No – **Go to First Name**

73. Date of unmanipulated product infusion: _____
 YYYY MM DD

74. Specify the route of unmanipulated product infusion

Intravenous – **Go to First Name**

Other route of infusion – **Go to question 75**

75. Specify other route of infusion: _____

First Name: _____

Last Name: _____

E-mail address: _____

Date: _____
 YYYY MM DD