



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 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

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**
- Other methodology – **Go to question 26**
- Unknown – **Go to question 27**

26. Specify other methodology: _____

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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**

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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**

46. Specify other method: _____

Other Cell Type 2

47. Specify other cell type: _____

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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**

71. Specify other route of infusion: _____

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

- Yes – **Go to question 73**
- No – **Go to First Name**

