



# Gene Therapy Product

## Registry Use Only

Sequence Number:

Date Received:

CIBMTR Center Number: \_\_\_\_\_

CIBMTR Research ID: \_\_\_\_\_

Event date: \_\_\_\_\_  
                  YYYY                  MM                  DD

### 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: \_\_\_\_\_ / $\mu$ L ( $\text{mm}^3$ )
  
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: \_\_\_\_\_ / $\mu$ L ( $\text{mm}^3$ )
  
11. Date of first collection for this mobilization: \_\_\_\_\_
 

YYYY
MM
DD

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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**

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- 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**

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- 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

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- 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**

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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: \_\_\_\_\_

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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**



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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: \_\_\_\_\_ — \_\_\_\_\_ — \_\_\_\_\_  
YYY Y 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: \_\_\_\_\_ — \_\_\_\_\_ — \_\_\_\_\_  
YYY Y MM DD

80. Specify the route of unmanipulated product infusion

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- Intravenous – **Go to End Form**
- Other route of infusion – **Go to question 81**

81. Specify other route of infusion: \_\_\_\_\_