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<th><strong>Gene Therapy Product</strong></th>
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**Registry Use Only**

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**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: ___ ___ ___ • ___ ___ /µL (mm³)

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: ___ • ___ ___ /µL (mm³)

10. Date of first collection for this mobilization: ___ ___ ___ ___ — ___ ___ — ___ ___

11. Was more than one collection required?
   - Yes – *Go to question 12*
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
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
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
Other Cell Type 2

46. Specify other method: __________________________________

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
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