Thalassemia Pre-Infusion Data

CIBMTR®
A RESEARCH COLLABORATION BETWEEN THE MEDICAL COLLEGE OF WISCONSIN AND NMDP

Registry Use Only

Sequence Number:

Date Received:

CIBMTR Center Number: ___ ___ ___ ___ ___

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

Event date: ___ ___ ___ ___ — ___ ___ — ___ ___

YYYY MM DD

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

Subsequent Transplant or Cellular Therapy

1. Is this a second or subsequent transplant or cellular therapy for the same disease?

☐ Yes – ***Go to question 27***

☐ No – ***Go to question 2***

Thalassemia Diagnosis

2. What is the recipient's beta-globin genotype?

☐ B / B **-** *(normal genotype, no beta mutation)* **–** ***Go to question 4***

☐ B^{+}/B^{+} **–** ***Go to question 4***

☐ B^{+}/B^{0} **–** ***Go to question 4***

☐ B^{E}/B^{+} **–** ***Go to question 4***

☐ B^{E}/B^{0} **–** ***Go to question 4***

☐ B^{0}/B^{0} **–** ***Go to question 4***

☐ Other genotype **–** ***Go to question 3***

☐ Unknown **–** ***Go to question 5***

3. Specify other beta-globin genotype:_____

4. Is documentation being attached? *(CIBMTR recommends source documentation)*

☐ Yes

☐ No

5. What is the recipient's alpha-globin genotype?

☐ aa / aa **–** *(normal genotype, no alpha mutation)* **–** ***Go to question 7***

☐ aa / a- **–** ***Go to question 7***

☐ - - / aa **–** ***Go to question 7***

☐ a- / a- **–** ***Go to question 7***

☐ - - / a- **–** ***Go to question 7***

☐ - - / $a^{CS}a$ **–** ***Go to question 7***

☐ - - / - - **–** ***Go to question 7***

☐ Other genotype **–** ***Go to question 6***

☐ Unknown **–** ***Go to question 8***

6. Specify other alpha-globin genotype:_____

7. Is documentation being attached? *(CIBMTR recommends source documentation)*
   - ☐ Yes
   - ☐ No

8. Is alpha-gene triplication present?
   - ☐ Yes
   - ☐ No
   - ☐ Unknown

9. Was hemoglobin electrophoresis performed at diagnosis? *(do not include results if an RBC transfusion occurred within 4 weeks of the electrophoresis study)*
   - ☐ Yes – ***Go to question 10***
   - ☐ No – ***Go to question 24***
   - ☐ Not applicable *(transfused within 4 weeks)* – ***Go to question 24***
   - ☐ Unknown – ***Go to question 24***

10. Date of diagnostic electrophoresis: ___ ___ ___ ___ — ___ ___ - ___ ___ ☐ Date estimated
    YYYY MM DD

Specify the hemoglobin allele types based on the sample tested in question 9.

11. Hb A
    - ☐ Yes – ***Go to question 12***
    - ☐ No – ***Go to question 13***

12. Hb A: ___ ___ %

13. Hb A2
    - ☐ Yes – ***Go to question 14***
    - ☐ No – ***Go to question 15***

14. Hb A2: ___ ___ %

15. Hb C
    - ☐ Yes – ***Go to question 16***
    - ☐ No – ***Go to question 17***

16. Hb C: ___ ___ %

17. Hb F

☐ Yes – ***Go to question 18***

☐ No – ***Go to question 19***

18. Hb F: ___ ___ %

19. Hb E

☐ Yes – ***Go to question 20***

☐ No – ***Go to question 21***

20. Hb E: ___ ___ %

21. Other thalassemia related hemoglobin allele type

☐ Yes- ***Go to question 22***

☐ No – ***Go to question 24***

22. Specify: _____

23. Level: ___ ___ %

24. Which genetic mutations were identified at diagnosis? *(check all that apply)*

☐ HBG2 (XmnI) – rs7482144 C>T – ***Go to question 26***

☐ BCL11A – rs1427407 G>T – ***Go to question 26***

☐ BCL11A – rs10189857 A>G – ***Go to question 26***

☐ HMIP – rs66650371 wt>3bp deletion – ***Go to question 26***

☐ KLF1 – c.892 G>C – ***Go to question 26***

☐ KLF1 – c.115 A>C – ***Go to question 26***

☐ HBA1 – HBA deletion (– $a^{3.7}$ / aa) – ***Go to question 26***

☐ HBA1 – HBA triplication ($aaa^{anti–3.7}$ / aa) – ***Go to question 26***

☐ Not done – ***Go to question 27***

☐ Unknown – ***Go to question 27***

☐ Other – ***Go to question 25***

25. Specify other: _____

26. Is documentation being attached? *(CIBMTR recommends source documentation)*

☐ Yes

☐ No

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

Donor Related Information

27. What is the donor's beta-globin genotype?

- ☐ B / B – *(normal genotype, no beta mutation)* – **Go to question 29**
- ☐ B/B^0, B/B^+ – **Go to question 29**
- ☐ B^+/B^+ – **Go to question 29**
- ☐ B^+/B^0 – **Go to question 29**
- ☐ B^E/B^+ – **Go to question 29**
- ☐ B^E/B^0 – **Go to question 29**
- ☐ B^0/B^0 – **Go to question 29**
- ☐ Other genotype – **Go to question 28**
- ☐ Unknown – **Go to question 30**

28. Specify other beta-globin genotype:_____

29. Is documentation being attached? *(CIBMTR recommends source documentation)*

- ☐ Yes
- ☐ No

30. What is the donor's alpha-globin genotype?

- ☐ aa / aa – *(normal genotype, no alpha mutation)* – ***Go to question 32***
- ☐ aa / a- – ***Go to question 32***
- ☐ - - / aa – ***Go to question 32***
- ☐ a- / a- – ***Go to question 32***
- ☐ - - / a- – ***Go to question 32***
- ☐ - - / $a^{CS}a$ – ***Go to question 32***
- ☐ - - / - - – ***Go to question 32***
- ☐ Other genotype – ***Go to question 31***
- ☐ Unknown – ***Go to question 33***

31. Specify other alpha-globin genotype:_____

32. Is documentation being attached? *(CIBMTR recommends source documentation)*

- ☐ Yes
- ☐ No

33. Hemoglobin *(for donor) (most recent prior to collection of infusion product)*

☐ Known – ***Go to question 34***

☐ Unknown – ***Go to question 36***

34. Hemoglobin: ___ ___ ___ ___ • ___ ___ ☐ g/dL
☐ g/L
☐ mmol/L

35. Were (red blood cells) RBCs transfused ≤ 30 days before date of test?

☐ Yes

☐ No

36. Mean corpuscular volume (MCV) *(for donor) (most recent prior to collection of infusion product)*

☐ Known – ***Go to question 37***

☐ Unknown – ***Go to question 38***

37. MCV: ___ ___ ___ ___ • ___ ___ ☐ X 10^{-15} L (fL)
☐ X 10^{6} / µL

38. Was hemoglobin electrophoresis performed for the donor? *(do not include results if an RBC transfusion occurred within 4 weeks of the electrophoresis study)*

☐ Yes – ***Go to question 39***

☐ No – ***Go to question 53***

☐ Not applicable *(transfused within 4 weeks)* – ***Go to question 53***

☐ Unknown – ***Go to question 53***

39. Date of most recent electrophoresis: ___ ___ ___ ___ — ___ ___ - ___ ___ ☐ Date estimated
YYYY MM DD

Specify the hemoglobin allele types based on the sample tested in question 38.

40. Hb A

☐ Yes – ***Go to question 41***

☐ No – ***Go to question 42***

41. Hb A: ___ ___ %

42. Hb A2

☐ Yes – ***Go to question 43***

☐ No – ***Go to question 44***

43. Hb A2: ___ ___ %

44. Hb C

☐ Yes – ***Go to question 45***

☐ No – ***Go to question 46***

45. Hb C: ___ ___ %

46. Hb F

☐ Yes – ***Go to question 47***

☐ No – ***Go to question 48***

47. Hb F: ___ ___ %

48. Hb E

☐ Yes – ***Go to question 49***

☐ No – ***Go to question 50***

49. Hb E: ___ ___ %

50. Other thalassemia related hemoglobin allele type

☐ Yes- ***go to question 51***

☐ No – ***Go to question 53***

51. Specify: _____

52. Level: ___ ___ %

Transfusion Therapy

For questions 53-112 report findings from ANY TIME PRIOR to the preparative regimen / infusion unless otherwise specified. If more than one test was performed report the most recent unless otherwise stated.

53. Were any red blood cell (RBC) transfusions administered?

☐ Yes– ***Go to question 54***

☐ No – ***Go to question 60***

54. Number of RBC transfusion events within the last 12 months: __ __

55. Date of last transfusion administered: *(answer ONLY if a transfusion was administered within the last 12 months)* ___ ___ ___ ___ — ___ ___ — ___ ___ □ Date estimated
YYYY MM DD

56. Were the RBC units used for transfusion of an extended phenotype match (D, C, c, E, e, K)? *(includes partial extended phenotype matches)*

- ☐ Yes
- ☐ No
- ☐ Unknown

57. Were RBC alloantibodies present?

- ☐ Yes – ***Go to question 58***
- ☐ No – ***Go to question 59***
- ☐ Unknown – ***Go to question 59***

58. Specify the number of alloantibodies

- ☐ 1
- ☐ ≥ 2

59. Does recipient have donor specific antibodies present to the donor chosen for transplant? *(mean fluorescence intensity (MFI) >1000 for HLA-A, HLA-B, and DRB1; OR MFI >2000 for HLA-C, DQB1 and DPB1 or positive virtual cross match)*

- ☐ Yes – ***Go to question 60***
- ☐ No – ***Go to question 61***
- ☐ Not done – ***Go to question 61***
- ☐ Unknown – ***Go to question 61***

60. Were measures taken to lower the MFI for the presence of donor antibodies prior to infusion?

- ☐ Yes
- ☐ No

Hepatic Assessments

Laboratory studies within 60 days prior to the start of preparative regimen, use result closest to the start date

61. Direct bilirubin

- ☐ Known – ***Go to question 62***
- ☐ Unknown – ***Go to question 64***

62. Direct bilirubin: ___ ___ ___ • ___ □ mg/dL

□ µmol/L

63. Upper limit of normal for your institution : ___ ___ ___ • ___

Cardiac Assessments

Laboratory studies within 60 days prior to the start of preparative regimen, use result closest to the start date

64. Was an echocardiogram performed?

- ☐ Yes – ***Go to question 65***
- ☐ No ***– Go to question 69***
- ☐ Unknown ***– Go to question 69***

65. Was left ventricular ejection fraction (LVEF) or left ventricular shortening fraction reported?

- ☐ Yes – ***Go to question 66***
- ☐ No – ***Go to question 68***

66. LVEF: ___ ___ %

67. Left ventricular shortening fraction: ___ ___ %

68. Is documentation being attached? *(CIBMTR recommends attaching the echocardiogram report)*

- ☐ Yes
- ☐ No

69. Cardiac iron T2 imaging *(found on MRI results)*

- ☐ Known – ***Go to question 70***
- ☐ Unknown – ***Go to question 71***

70. Cardiac iron T2: ___ ___ msec

71. Was brain natriuretic peptide (BNP) assessed?

- ☐ Yes – ***Go to question 72***
- ☐ No – ***Go to question 73***
- ☐ Unknown – ***Go to question 73***

72. BNP: __ __ __ __ __ •__ pg/mL

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Renal Assessments

Laboratory studies within 60 days prior to the start of preparative regimen, use result closest to the start date

73. Was proteinuria detected? *(excluding microalbuminuria)*
- ☐ Yes
- ☐ No
- ☐ Not done

74. Glomerular filtration rate (GFR) *(only required if the recipient is 19 years of age or older)*
- ☐ Known – ***Go to question 75***
- ☐ Unknown – ***Go to question 76***

75. GFR: ___ ___ ___mL/min/1.73m^2 *(if the actual value cannot be reported use the Cockcroft- Gault equation to report the calculated value)OC*

Avascular Necrosis

76. Has avascular necrosis occurred?
- ☐ Yes – ***Go to question 77***
- ☐ No **– *Go to question 79***
- ☐ Unknown **– *Go to question 79***

77. Specify joint(s) affected *(check all that apply)*
- ☐ Hip **– *Go to question 79***
- ☐ Knee **– *Go to question 79***
- ☐ Shoulder **– *Go to question 79***
- ☐ Other – ***Go to question 78***

78. Specify other: _____

Other Symptoms

79. Have chronic leg ulcers developed?
- ☐ Yes
- ☐ No
- ☐ Unknown

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Additional Iron Overload Assessments

Laboratory studies within 60 days prior to the start of preparative regimen, use result closest to the start date

80. Serum ferritin

☐ Known – ***Go to question 81***

☐ Unknown – ***Go to question 84***

81. Serum ferritin: ___ ___ ___ ___ ___ ng/mL (µg/L)

82. Date sample drawn: ___ ___ ___ ___ — ___ ___ - ___ ___ ☐ Date estimated
YYYY MM DD

83. Upper limit of normal for your institution: __ __ __ __ __

84. Soluble transferrin receptor (sTfR)

☐ Known – ***Go to question 85***

☐ Unknown – ***Go to question 86***

85. sTfR : ___ ___ • ___ mg/L

86. Erythropoietin (EPO) level

☐ Known – ***Go to question 87***

☐ Unknown – ***Go to question 88***

87. EPO level: ___ ___ ___ IU/L

88. Serum hepcidin level

☐ Known – ***Go to question 89***

☐ Unknown – ***Go to question 90***

89. Serum hepcidin level: ___ ___ ___ ng/mL (µg/L)

Additional Hematologic Labs

Laboratory studies within 60 days prior to the start of preparative regimen, use result closest to the start date

90. Reticulocyte count

☐ Known – ***Go to question 91***

☐ Unknown – ***Go to question 92***

91. Reticulocyte count: ___ ___ ___ ___ • ___ ___ x 10 ___ ___ cells/µL

92. Haptoglobin

☐ Known – ***Go to question 93***

☐ Unknown – ***Go to question 95***

93. Haptoglobin: ___ ___ ___ ___ ___ • ___ ___ ☐ mg/dL
☐ g/L

94. Lower limit of normal for your institution: ___ ___ ___ ___ ___ • ___ ___

Existing Organ Impairments

Copy and complete 95-105 to report multiple impairments

95. Specify co-existing diseases or organ impairments any time prior to start of preparative regimen.

☐ Amenorrhea - ***Go to question 96***
☐ Cardiomyopathy - ***Go to question 96***
☐ Cholelithiasis- ***Go to question 96***
☐ Growth hormone deficiency / short stature - ***Go to question 96***
☐ Hypersplenism- ***Go to question 96***
☐ Hypothyroidism requiring replacement therapy - ***Go to question 96***
☐ Osteopathies (porosis, penia) – ***Go to question 96 and 97***
☐ Pulmonary hypertension- ***Go to question 96***
☐ Retinal changes - ***Go to question 96***
☐ Thrombosis - ***Go to question 96***
☐ None - ***Go to question 106***

96. Date of diagnosis: ___ ___ ___ ___ — ___ ___ — ___ ___ ☐ Date estimated
YYYY MM DD

97. Method used to assess osteopathies (report the most recent Z or T-score available; Z-scores are used in patients younger than or equal to 20 and T-scores in patients older than 20) (check all that apply)

☐ Dual-energy X-ray absorptiometry (DEXA) scan – ***Go to question 98***
☐ Quantitative CT – ***Go to question 102***
☐ Unknown - ***Go to question 106***

98. DEXA scan vertebral

☐ Known – ***Go to question 99***

☐ Unknown – ***Go to question 100***

99. Z or T – score: __ __ . __ __ ☐ Negative value

100. DEXA scan hip

☐ Known – ***Go to question 101***

☐ Unknown – ***Go to question 102***

101. Z or T – score: __ __ . __ __ ☐ Negative value

102. Quantitative CT vertebral

☐ Known – ***Go to question 103***

☐ Unknown – ***Go to question 104***

103. Z or T – score: __ __ . __ __ ☐ Negative value

104. Quantitative CT hip

☐ Known – ***Go to question 105***

☐ Unknown – ***Go to question 106***

105. Z or T – score: __ __ . __ __ ☐ Negative value

Copy and complete 95-105 to report multiple impairments

Disease Modifying Therapies

106. Were disease modifying therapies given? *(excludes blood transfusions)*

☐ Yes – ***Go to question 107***

☐ No - ***Go to question 113***

☐ Unknown - ***Go to question 113***

If there is more than one therapy given copy questions 107- 112 for each therapy.

107. Specify the disease modifying therapy *(check all that apply)*

☐ Hydroxyurea – ***Go to question 109***

☐ Luspatercept – ***Go to question 109***

☐ Other – ***Go to question 108***

108. Specify other: _____

109. Date therapy started

☐ Known - ***Go to question 110***

☐ Unknown – ***Go to question 111***

110. Date therapy started: ___ ___ ___ ___ -___ ___ - ___ ___ ☐ Date estimated

YYYY MM DD

111. Date therapy stopped

☐ Known – ***Go to question 112***

☐ Unknown ***- Go to question 113***

☐ Not applicable *(still receiving therapy)* - **Go to question 113**

112. Date therapy stopped: ___ ___ ___ ___ -___ ___ - ___ ___ ☐ Date estimated

YYYY MM DD

Marrow Evaluation

Complete question 113 for gene therapy infusions only.

113. Was a marrow aspirate and / or biopsy performed?

☐ Yes - **Also complete Laboratory Studies Form 3502 and Marrow Surveillance Form 3506**

☐ No

☐ Unknown