Thalassemia Pre-Infusion Data

CIBMTR Center Number: ___ ___ ___ ___ ___

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

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

                YYYY   MM   DD

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

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

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

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)*

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

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

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)

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*

☐ Hypogonadism / gonadal dysfunction – *Go to question 96*

☐ Hypothyroidism requiring replacement therapy - *Go to question 96*

☐ Osteonecrosis – *Go to question 96*

☐ Osteopathies (porosis, penia) – *Go to question 96*

☐ 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, at last evaluation.

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

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

☐ No

☐ Unknown