Thalassemia Post-Infusion Data

Registry Use Only

Sequence Number:

Date Received:

CIBMTR Center Number: ___ ___ ___ ___ ___

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

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

               YYYY   MM   DD

Visit
- ☐ 100 day
- ☐ 6 months
- ☐ 1 year
- ☐ 2 years
- ☐ > 2 years, Specify: ___ ___

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

Report all findings SINCE DATE OF LAST REPORT unless otherwise specified

Post-Infusion Disease Testing

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

    ☐   Yes – *Go to question 2*

    ☐   No – *Go to question 16*

    ☐   Not applicable *(transfused within 4 weeks)* – *Go to question 16*

    ☐   Unknown – *Go to question 16*

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

   Specify the hemoglobin allele types based on the sample tested above

3. Hb A

    ☐ Yes – *Go to question 4*

    ☐ No – *Go to question 5*

    4.   Hb A: ___ ___ %

5. Hb A2

    ☐ Yes – *Go to question 6*

    ☐ No – *Go to question 7*

    6.   Hb A2: ___ ___ %

7. Hb C

    ☐ Yes – *Go to question 8*

    ☐ No – *Go to question 9*

    8.   Hb C: ___ ___ %

9. Hb F

    ☐ Yes – *Go to question 10*

    ☐ No – *Go to question 11*

    10.   Hb F: ___ ___ %

11. Hb E

☐ Yes – *Go to question 12*

☐ No – *Go to question 13*

12. Hb E: ___ ___ %

13. Other thalassemia related hemoglobin allele type

☐ Yes- *Go to question 14*

☐ No – *Go to question 16*

14. Specify: _____

15. Level: ___ ___ %

Transfusion Therapy

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

☐ Yes– *Go to question 17*

☐ No – *Go to question 18*

17. Date of last transfusion: ___ ___ ___ ___ — ___ ___ — ___ ___
<div align="center">YYYY   MM   DD</div>

Treatment

18. Was the recipient on iron chelation?

☐ Yes – **Go to question 19**

☐ No – **Go to question 24**

☐ Unknown – **Go to question 24**

19. Was iron chelation previously reported?

☐ Yes – *Go to question 22*

☐ No - *Go to question 20*

20. Date started

☐ Known - *Go to question 21*

☐ Unknown - *Go to question 22*

21. Date started: ___ ___ ___ ___ — ___ ___ — ___ ___   ☐ Date estimated
<div align="center">YYYY   MM   DD</div>

22.   Is iron chelation ongoing?

   ☐   Yes – *Go to question 24*

   ☐   No – *Go to question 23*

      23.   Date of last dose: ___ ___ ___ ___ — ___ ___ — ___ ___   ☐   Date estimated

                          YYYY   MM   DD

24.   Was phlebotomy performed?

   ☐   Yes – *Go to question 25*

   ☐   No – *Go to question 30*

      25.   Was phlebotomy previously reported?

         ☐   Yes – *Go to question 28*

         ☐   No - *Go to question 26*

         26.   Date started

            ☐ Known - *Go to question 27*

            ☐ Unknown - *Go to question 28*

            27.   Date started: ___ ___ ___ ___ — ___ ___ — ___ ___   ☐   Date estimated

                                 YYYY   MM   DD

      28.   Is phlebotomy ongoing?

         ☐   Yes – *Go to question 30*

         ☐   No – *Go to question 29*

         29.   Date completed: ___ ___ ___ ___ — ___ ___ — ___ ___   ☐   Date estimated

                             YYYY   MM   DD

Splenic Assessments

30.   Did the recipient have a splenectomy?

   ☐   Yes - **Go to question 32**

   ☐   No – **Go to question 31**

   ☐   Not applicable *(prior splenectomy previously reported)* - **Go to question 32**

   ☐   Unknown – **Go to question 31**

      31.   Did the recipient have splenomegaly?

         ☐   Yes

☐   No

Hepatic Assessments

32.   Does the recipient have hepatomegaly? *(≥ 2 cm below costal margin)*

☐   Yes– ***Go to question 33***

☐   No– ***Go to question 35***

☐   Unknown– ***Go to question 35***

33.   Is liver size known?

☐   Yes – ***Go to question 34***

☐   No – ***Go to question 35***

34.   Liver size as measured below the right costal margin at most recent evaluation: ___ ___ cm

35.   Was liver iron content (LIC) tested?

☐   Yes – ***Go to question 36***

☐   No – ***Go to question 38***

36.   Liver iron content: ___ ___ ___ ● ___   ☐   mg Fe/g liver dry weight

☐   g Fe/kg liver dry weight

☐   μmol Fe/g liver dry weight

37.   Method used to estimate LIC?

☐   T2*MRI *(T2* weighted magnetic resonance imaging)*

☐   SQUID MRI

☐   FerriScan

☐   Liver biopsy

☐   Other

38.   Was a liver biopsy performed?

☐   Yes – ***Go to questions 39***

☐   No – ***Go to questions 46***

39.   Date assessed

☐   Known **– *Go to question 40***

☐   Unknown **– *Go to question 41***

40.   Date assessed: ___ ___ ___ ___ — ___ ___ — ___ ___   ☐   Date estimated

YYYY   MM   DD

41.   Was there evidence of liver cirrhosis?

☐   Yes

☐   No

☐   Unknown

42.   Was there evidence of liver fibrosis?

☐   Yes – **Go to question 43**

☐   No – **Go to question 44**

☐   Unknown – **Go to question 44**

43.   Type of fibrosis

☐   Bridging

☐   Periportal

☐   Other

☐   Unknown

44.   Was there evidence of chronic hepatitis?

☐   Yes

☐   No

☐   Unknown

45.   Is documentation being attached? *(CIBMTR recommends attaching the liver biopsy report)*

☐   Yes

☐   No

Laboratory studies.

46.   Total serum bilirubin

☐   Known – **Go to question 47**

☐   Unknown – **Go to question 49**

47.   Total serum bilirubin: ___ ___ ___ ● ___ ☐ mg/dL

☐ µmol/L

48.   Upper limit of normal for your institution : ___ ___ ___ ● ___

49.   Direct bilirubin

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

    ☐   Known – *Go to question 50*

    ☐   Unknown – *Go to question 52*

50.   Direct bilirubin: ___ ___ ___ ● ___   ☐ mg/dL

                                                                       ☐ µmol/L

51.   Upper limit of normal for your institution: ___ ___ ___ ● ___

52.   AST (SGOT)

    ☐ Known – *Go to question 53*

    ☐ Unknown – *Go to question 54*

53.   AST (SGOT) : ___ ___ ___ ___ ● ___ ___ ☐ U/L

                                                                      ☐ µkat/L

54.   ALT (SGPT)

    ☐   Known – *Go to question 55*

    ☐   Unknown – *Go to question 56*

55.   ALT (SGPT) : ___ ___ ___ ___ ● ___ ___ ☐ U/L

                                                                      ☐ µkat/L

Cardiac Assessments

56.   Was an echocardiogram performed?

    ☐   Yes – *Go to question 57*

    ☐   No *– Go to question 63*

    ☐   Unknown *– Go to question 63*

57.   Was tricuspid regurgitant jet velocity (TRJV) measured?

      ☐   Yes – *Go to question 58*

      ☐   No – *Go to question 59*

58.   TRJV: ___ ___ ___ ●___ m/sec

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

      ☐   Yes – *Go to question 60*

      ☐   No – *Go to question 62*

60.   LVEF: ___ ___ %

61.   Left ventricular shortening fraction: ___ ___ %

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

☐   Yes

☐   No

63.   Was cardiac MRI performed?

☐ Yes **– *Go to question 64***

☐ No **– *Go to question 67***

64.   Is there evidence of abnormal cardiac iron deposition based on MRI of the heart?

☐   Yes

☐   No

65.   Cardiac iron T2 imaging

☐   Known – ***Go to question 66***

☐   Unknown – ***Go to question 67***

66.   Cardiac iron T2: ___ ___ msec

67. Was brain natriuretic peptide (BNP) assessed?

☐ Yes – ***Go to question 68***

☐ No – ***Go to question 69***

☐ Unknown – ***Go to question 69***

68.   BNP: __ __ ___ __ ___ •__ pg/mL

Renal Assessments

69.   Was proteinuria detected? (*excluding microalbuminuria*)

☐ Yes

☐ No

☐ Not done

70.   Serum creatinine

☐ Known – ***Go to questions 71***

☐ Unknown – ***Go to questions 72***

71.   Serum creatinine: ___ ___ ___ • ___ ___   ☐ mg/dL

☐ mmol/L

☐ µmol/L

72.   Glomerular filtration rate (GFR) *(if multiple, report the most recent tested)*

☐ Known– ***Go to question 73***

☐ Unknown – ***Go to question 74***

73.   GFR: ___ ___ ___ mL/min/1.73m^2 *(if the actual value cannot be reported use the Cockcroft- Gault equation to report the calculated value) (for pediatric recipients, use the "Bedside Schwartz" or Cystatin C-based equation)*

Avascular Necrosis

74.   Is there a new area affected by avascular necrosis?

☐ Yes – ***Go to question 75***

☐ No – ***Go to question 77***

☐ Unknown – ***Go to question 77***

75.   Specify joint(s) affected *(check all that apply)*

☐ Hip – ***Go to question 77***

☐ Knee – ***Go to question 77***

☐ Shoulder – ***Go to question 77***

☐ Other – ***Go to question 76***

76.   Specify other: _____

Other Symptoms

Answer the following questions since date of last report.

77.   Have chronic leg ulcers developed?

☐ Yes

☐ No

☐ Unknown

Additional Iron Overload Assessments

78.   Serum iron

☐ Known – ***Go to questions 79***

☐ Unknown – ***Go to questions 80***

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

79.   Serum iron: ___ ___ ___ • ___ ___   ☐ µg/dL
                                                       ☐ µmol/L

80.   Total iron binding capacity (TIBC)

    ☐ Known – *Go to question 81*

    ☐ Unknown – *Go to question 82*

    81.   TIBC: ___ ___ ___ • ___ ___   ☐ µg/dL
                                                           ☐ µmol/L

82.   Serum ferritin

    ☐ Known – *Go to questions 83*

    ☐ Unknown – *Go to questions 84*

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

84.   Soluble transferrin receptor (sTfR)

    ☐ Known – *Go to question 85*

    ☐ Unknown – *Go to question 87*

    85.   sTfR : ___ ___ • ___ mg/L

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

87.   Erythropoietin (EPO) level

    ☐ Known – *Go to question 88*

    ☐ Unknown – *Go to question 90*

    88.   EPO level: ___ ___ ___ IU/L

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

90.   Serum hepcidin level:

    ☐ Known – *Go to question 91*

    ☐ Unknown – *Go to question 93*

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

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

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

Additional Hematologic Labs

93.   Reticulocyte count

☐ Known – *Go to question 94*

☐ Unknown – *Go to question 96*

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

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

96.   Lactate dehydrogenase (LDH)

☐ Known – *Go to question 97*

☐ Unknown – *Go to question 99*

97.   LDH: ___ ___ ___ ___ ___ • ___ ___ ☐ U/L
                                              ☐ µkat/L

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

99.   Haptoglobin

☐ Known – *Go to question 100*

☐ Unknown – *Go to question 102*

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

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

Existing Organ Impairments

Copy and complete 102 - 113 to report multiple impairment

102.   Specify organ impairments or disorders developed

☐ Amenorrhea– *Go to question 103*

☐ Cardiomyopathy– *Go to question 103*

☐ Cholelithiasis– *Go to question 103*

☐ Growth hormone deficiency / short stature – *Go to question 103*

☐ Hypersplenism– **Go to question 103**

☐ Hypogonadism / gonadal dysfunction – **Go to question 103**

☐ Hypothyroidism requiring replacement therapy – **Go to question 103**

☐ Osteonecrosis **– Go to question 103**

☐ Osteopathies (porosis, penia) – **Go to question 103**

☐ Pulmonary hypertension– **Go to question 103**

☐ Retinal changes – **Go to question 103**

☐ Thrombosis – **Go to question 103**

☐ None - **Go to question 114**

103.   Was this organ impairment previously reported?

☐   Yes – **Go to question 105**

☐   No – **Go to question 104**

104.   Date of diagnosis: ___ ___ ___ ___ — ___ ___ — ___ ___ ☐   Date estimated

         YYYY   MM   DD

105.   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-score in patients older than 20) (check all that apply)

☐   Dual-energy X-ray absorptiometry (DEXA) scan – **Go to question 106**

☐   Quantitative CT– **Go to question 110**

☐   Unknown - **Go to question 114**

106.   DEXA scan vertebral

☐ Known **– Go to question 107**

☐ Unknown **– Go to question 108**

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

108.   DEXA scan hip

☐ Known **– Go to question 109**

☐ Unknown **– Go to question 110**

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

110.   Quantitative CT vertebral

☐   Known **– Go to question 111**

☐   Unknown **– Go to question 112**

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

112.   Quantitative CT hip

☐ Known – ***Go to question 113***

☐ Unknown – ***Go to question 114***

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

Copy and complete 102 - 113 to report multiple impairment

Disease Modifying Therapies

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

☐   Yes – ***Go to question 115***

☐   No - ***Go to question 122***

☐   Unknown - ***Go to question 122***

If there is more than one therapy given copy questions 115- 121 for each therapy.

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

☐   Hydroxyurea – ***Go to question 117***

☐   Luspatercept – ***Go to question 117***

☐   Other – ***Go to question 116***

116.   Specify other: _____

117.   Was the date therapy started previously reported?

☐   Yes – ***Go to question 120***

☐   No – ***Go to question 118***

118.   Date therapy started

☐ Known - ***Go to question 119***

☐ Unknown – ***Go to question 120***

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

YYYY   MM   DD

120.   Date therapy stopped

☐   Known – ***Go to question 121***

☐   Unknown - ***Go to question 122***

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

☐   Not applicable *(still receiving therapy)* - **Go to question 122**

121.   Date therapy stopped: ___ ___ ___ ___ -___ ___ - ___ ___   ☐   Date estimated
                                              YYYY   MM   DD

Marrow Evaluation

Complete question 122 for gene therapy infusions only

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

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

☐ No

☐ Unknown