



# 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: \_\_\_\_\_

**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: \_\_\_\_\_ %

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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: \_\_\_\_\_

YYYY                      MM                      DD

### 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

YYYY                      MM                      DD

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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? ( $\geq 2$  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
- $\mu$ mol 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

CIBMTR Center Number: \_\_\_\_\_ CIBMTR Research ID: \_\_\_\_\_

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

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- Known – **Go to question 50**
- Unknown – **Go to question 52**

50. Direct bilirubin: \_\_\_\_\_ • \_\_\_\_\_  mg/dL  
  $\mu\text{mol/L}$

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  
  $\mu\text{kat/L}$

54. ALT (SGPT)

- Known – **Go to question 55**
- Unknown – **Go to question 56**

55. ALT (SGPT) : \_\_\_\_\_ • \_\_\_\_\_  U/L  
  $\mu\text{kat/L}$

**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: \_\_\_\_\_ %

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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



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$\mu\text{mol/L}$

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<sup>2</sup> *(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**

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79. Serum iron: \_\_\_\_\_ • \_\_\_\_\_   $\mu\text{g/dL}$

$\mu\text{mol/L}$

80. Total iron binding capacity (TIBC)

Known – **Go to question 81**

Unknown – **Go to question 82**

81. TIBC: \_\_\_\_\_ • \_\_\_\_\_   $\mu\text{g/dL}$

$\mu\text{mol/L}$

82. Serum ferritin

Known – **Go to questions 83**

Unknown – **Go to questions 84**

83. Serum ferritin: \_\_\_\_\_ ng/mL ( $\mu\text{g/L}$ )

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 ( $\mu\text{g/L}$ )



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- Hypersplenism– **Go to question 103**
- Hypothyroidism requiring replacement therapy – **Go to question 103**
- Osteopathies (porosis, penia) – **Go to question 103 & 104**
- 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

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

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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

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