Sickle Cell Disease Pre-Infusion Data

CIBMTR®
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Registry Use Only

Sequence Number:

Date Received:

CIBMTR Center Number: ___ ___ ___ ___ ___

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

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

YYYY MM DD

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

For all questions, 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.

Subsequent Infusion

If this is a report of a second or subsequent transplant or cellular therapy infusion for the same disease subtype and this baseline disease insert has not been completed for the previous infusion (e.g., recipient was on TED track for prior HCT, prior HCT was autologous with no consent, prior cellular therapy was not reported to the CIBMTR), select "No" and begin the form at question two.

1. Is this a report of a second or subsequent infusion for the same disease?
   - ☐ Yes - ***Go to question 4***
   - ☐ No - ***Go to question 2***

Sickle Cell Diagnosis

2. What is the recipient's sickle cell disease genotype?
   - ☐ Hb SC – ***Go to question 4***
   - ☐ Hb SD - ***Go to question 4***
   - ☐ Hb SO Arab - ***Go to question 4***
   - ☐ Hb SS – ***Go to question 4***
   - ☐ Hb S beta^{+} thalassemia – ***Go to question 4***
   - ☐ Hb S beta^{0} thalassemia – ***Go to question 4***
   - ☐ Hb S delta beta^{0} thalassemia – ***Go to question 4***
   - ☐ Other genotype– ***Go to question 3***

3. Specify other genotype: _____

Transfusion Therapy

4. Were any red blood cell (RBC) transfusions administered?
   - ☐ Yes– ***Go to question 5***
   - ☐ No – ***Go to question 15***

5. Number of RBC transfusion events within the last 12 months: ___ ___

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

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7. Was a transfusion administered to PREVENT sickle cell related events? *(includes simple or exchange transfusions)*

- ☐ Yes – ***Go to question 8***
- ☐ No – ***Go to question 12***

8. Date transfusion started

- ☐ Known - ***Go to question 9***
- ☐ Unknown – ***Go to question 10***

9. Date transfusion started: ___ ___ ___ ___ — ___ ___ — ___ ___ ☐ Date estimated
YYYY MM DD

10. Date transfusion stopped

- ☐ Known ***– Go to question 11***
- ☐ Unknown – ***Go to question 12***
- ☐ Not applicable *(still receiving therapy)* – ***Go to question 12***

11. Date transfusion stopped: ___ ___ ___ ___ — ___ ___ — ___ ___ ☐ Date estimated
YYYY MM DD

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

- ☐ Yes
- ☐ No

13. Were RBC alloantibodies present?

- ☐ Yes – ***Go to question 14***
- ☐ No – ***Go to question 15***
- ☐ Unknown – ***Go to question 15***

14. Specify the number of alloantibodies

- ☐ 1
- ☐ ≥ 2

Therapy for Iron Overload

15. Was iron chelation therapy received at any time?

- ☐ Yes – ***Go to question 16***
- ☐ No – ***Go to question 18***
- ☐ Unknown – ***Go to question 18***

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16. Specify therapy *(check all that apply)*

- ☐ Deferasirox (Exjade / Jadenu) – ***Go to question 18***
- ☐ Deferiprone (Ferriprox) – ***Go to question 18***
- ☐ Deferoxamine (Desferal)– ***Go to question 18***
- ☐ Other – ***Go to question 17***

17. Specify other: _____

Pulmonary Assessments

18. Were pulmonary function tests (PFTs) performed? *(if PFT tests were conducted we ask that you attach the most recent report)*

- ☐ Yes – ***Go to question 19***
- ☐ No – ***Go to question 20***

19. Indicate if the PFT report is attached

- ☐ Attached
- ☐ Not available

20. For children unable to perform PFTs, was oxygen saturation in room air > 95%? *(only required to answer if children are of 5 years of age or less)*

- ☐ Yes
- ☐ No
- ☐ Not done

21. Was a 6-minute walk test performed?

- ☐ Yes – ***Go to question 22***
- ☐ No – ***Go to question 24***
- ☐ Unknown – ***Go to question 24***

22. Distance walked: ___ ___ ___

- ☐ meters
- ☐ feet

23. Indicate if the 6-minute walk test report is attached

- ☐ Attached
- ☐ Not available

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Cardiovascular Assessments

24. Was left ventricular ejection fraction (LVEF) or left ventricular shortening fraction reported by echocardiogram?
- ☐ Yes – ***Go to question 25***
- ☐ No – ***Go to question 27***

25. LVEF: ___ ___ %

26. Left ventricular shortening fraction: ___ ___ %

27. Is there evidence of pulmonary hypertension?
- ☐ Yes – ***Go to question 28***
- ☐ No – ***Go to question 31***

28. What method was used to diagnose pulmonary hypertension? *(check all that apply)*
- ☐ Echocardiogram **– *Go to question 29***
- ☐ Right heart catheterization **– *Go to question 30***

29. Indicate if the echocardiogram report is attached
- ☐ Attached
- ☐ Not available

30. Indicate if the right heart catheterization report is attached
- ☐ Attached
- ☐ Not available

31. Cardiac iron T2 imaging *(found on MRI results)*
- ☐ Known – ***Go to question 32***
- ☐ Unknown – ***Go to question 34***

32. Cardiac iron T2: ___ ___ msec

33. Indicate if the cardiac MRI report is attached
- ☐ Attached
- ☐ Not available

34. Was brain natriuretic peptide (BNP) assessed?
- ☐ Yes – ***Go to question 35***
- ☐ No – ***Go to question 37***

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☐ Unknown – ***Go to question 37***

35. BNP: __ __ __ __ __ •__ pg/mL

36. Date BNP collected: ___ ___ ___ ___ — ___ ___ — ___ ___
YYYY MM DD

37. N-terminal pro b-type natriuretic peptide (NT-proBNP)

☐ Known – ***Go to question 38***

☐ Unknown – ***Go to question 40***

38. NT-proBNP: __ __ __ __ __ •__ pg/mL

39. Date NT-proBNP collected: ___ ___ ___ ___ — ___ ___ — ___ ___
YYYY MM DD

40. Was an electrocardiogram performed?

☐ Yes – ***Go to question 41***

☐ No – ***Go to question 42***

41. Indicate if the electrocardiogram report is attached

☐ Attached

☐ Not available

Renal Assessments

42. Blood pressure (BP)

☐ Known – ***Go to question 43***

☐ Unknown – ***Go to question 44***

43. BP: ___ ___ ___ / ___ ___ ___ mmHg

44. Urine albumin

☐ Known– ***Go to question 45***

☐ Unknown – ***Go to question 46***

45. Urine albumin: ___ ___ ___

☐ µg/mL

☐ mg/L

☐ mg/mmol

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46. Urine creatinine *(one time urine)*

- ☐ Known– ***Go to question 47***
- ☐ Unknown – ***Go to question 48***

47. Urine creatinine: ___ ___ ___

- ☐ mg/dL
- ☐ µmol/L

Splenic Assessments

48. Was splenic function assessed?

- ☐ Yes – ***Go to question 49***
- ☐ No – ***Go to question 53***
- ☐ Not applicable ***– Go to question 53***
- ☐ Unknown – ***Go to question 53***

49. Select which splenic test was completed

- ☐ Complete red blood cell count – ***Go to question 50***
- ☐ Pitted RBC score – ***Go to question 51***
- ☐ Splenic scan – ***Go to question 52***

50. Complete RBC: ___ ___ ___ ___ • ___ ___
- ☐ x 10^3/µL (x 10^9/L) (x 10^3/mm^3)
- ☐ x 10^6/µL (x 10^{12}/L)

51. Pitted RBC score: ___ ___ • ___ %

52. Splenic scan results

- ☐ Normal *(radionuclide uptake)*
- ☐ Abnormal *(no radionuclide uptake)*

Acute Chest Syndrome

53. Has acute chest syndrome (ACS) occurred in the last two years?

- ☐ Yes – ***Go to question 54***
- ☐ No ***– Go to question 58***
- ☐ Unknown ***– Go to question 58***

54. Total number of episodes within the last two years

- ☐ Known – ***Go to question 55***

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☐ Unknown **– *Go to question 56***

55. Number of episodes: ___ ___

56. Were red blood cell transfusions required for treatment of ACS in the last two years? *(report any simple or exchange transfusions)*

☐ Yes

☐ No

☐ Unknown

57. Was intubation / mechanical ventilation required for treatment of ACS in the last two years?

☐ Yes

☐ No

☐ Unknown

Pain

58. Have vaso-occlusive pain episodes occurred that required hospitalization or treatment within the last 2 years? *(treatment that is in a hospital or clinic setting)*

☐ Yes – ***Go to question 59***

☐ No **– *Go to question 61***

☐ Unknown **– *Go to question 61***

59. Total number of episodes within the last two years

☐ Known – ***Go to question 60***

☐ Unknown – ***Go to question 61***

60. Number of episodes: ___ ___

61. Were opioids prescribed for the treatment of pain?

☐ Yes

☐ No

Avascular Necrosis

62. Has avascular necrosis occurred?

☐ Yes – ***Go to question 63***

☐ No **– *Go to question 67***

☐ Unknown **– *Go to question 67***

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

- ☐ Hip **– *Go to question 65***
- ☐ Knee **– *Go to question 65***
- ☐ Shoulder **– *Go to question 65***
- ☐ Other – ***Go to question 64***

64. Specify other: _____

65. Was a musculoskeletal MRI performed?

- ☐ Yes **– *Go to question 66***
- ☐ No **– *Go to question 67***

66. Indicate if the musculoskeletal MRI report is attached

- ☐ Attached
- ☐ Not available

67. Did the recipient undergo joint replacement?

- ☐ Yes – ***Go to question 68***
- ☐ No – ***Go to question 70***

68. Joint replaced *(check all that apply)*

- ☐ Hip – ***Go to question 70***
- ☐ Knee – ***Go to question 70***
- ☐ Shoulder – ***Go to question 70***
- ☐ Other – ***Go to question 69***

69. Specify other joint replaced: _____

Central Nervous System

70. Was transcranial doppler velocity assessed? *(pediatric only)*

- ☐ Yes – ***Go to question 71***
- ☐ No – ***Go to question 74***
- ☐ Unknown – ***Go to question 74***

71. Date of most recent transcranial doppler: ___ ___ ___ ___ — ___ ___ — ___ ___
YYYY MM DD

72. Transcranial doppler velocity: ___ ___ ___ cm/sec

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73. Indicate if the transcranial doppler velocity report is attached
    - ☐ Attached
    - ☐ Not available

74. Was there a sickle cell central nervous system (CNS) complication?
    - ☐ Yes – ***Go to question 75***
    - ☐ No ***– Go to question 80***
    - ☐ Unknown ***– Go to question 80***

If there is more than one central nervous event copy questions 75- 79 for each instance.

75. Specify type of CNS complication
    - ☐ Cerebral venous thrombosis
    - ☐ Hemorrhagic stroke
    - ☐ Ischemic stroke
    - ☐ Moyamoya
    - ☐ Overt stroke
    - ☐ Posterior reversible encephalopathy syndrome (PRES)
    - ☐ Seizure
    - ☐ Silent stroke
    - ☐ Transient ischemic stroke

76. Year of onset
    - ☐ Known – ***Go to question 77***
    - ☐ Unknown – ***Go to question 78***

77. Year of onset: ___ ___ ___ ___

78. Was an MRI / MRA of the brain performed for the diagnosis of this reported CNS complication?
    - ☐ Yes – ***Go to question 79***
    - ☐ No ***– Go to question 80***
    - ☐ Unknown ***– Go to question 80***

79. Indicate if the MRI / MRA report is attached
    - ☐ Attached
    - ☐ Not available

If there is more than one central nervous event copy questions 75- 79 for each instance.

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Other Symptoms

80. Did one or more episodes of priapism occur? *(to be answered for males only)*

- ☐ Yes – ***Go to question 81***
- ☐ No ***– Go to question 82***
- ☐ Unknown ***– Go to question 82***

81. Was surgery performed at any time to correct blood flow?

- ☐ Yes
- ☐ No
- ☐ Unknown

82. Has sickle cell retinopathy developed?

- ☐ Yes
- ☐ No
- ☐ Unknown

83. Have chronic leg ulcers developed?

- ☐ Yes
- ☐ No
- ☐ Unknown

84. Is there a diagnosis of asthma or a reactive airway disease?

- ☐ Yes
- ☐ No
- ☐ Unknown

85. Has a venous thromboembolism developed?

- ☐ Yes – ***Go to question 86***
- ☐ No – ***Go to question 87***
- ☐ Unknown – ***Go to question 87***

86. Was it associated with an indwelling catheter?

- ☐ Yes
- ☐ No

87. Has a pulmonary embolism developed?

- ☐ Yes – ***Go to question 88***

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☐ No – ***Go to question 89***

☐ Unknown – ***Go to question 89***

88. Was it associated with an indwelling catheter?

☐ Yes

☐ No

89. Number of splenic sequestration events: __ __

90. Date of last event: ___ ___ ___ ___ — ___ ___ — ___ ___
YYYY MM DD

Existing Organ Impairments

Copy and complete questions 91 - 102 to report multiple impairments.

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

☐ Amenorrhea - ***Go to question 92***

☐ Cardiomyopathy - ***Go to question 92***

☐ Cholelithiasis- ***Go to question 92***

☐ Growth hormone deficiency / short stature - ***Go to question 92***

☐ Hypersplenism- ***Go to question 92***

☐ Hypogonadism / gonadal dysfunction - ***Go to question 92***

☐ Hypothyroidism requiring replacement therapy - ***Go to question 92***

☐ Osteonecrosis - ***Go to question 92***

☐ Osteopathies (porosis, penia) – ***Go to question 92***

☐ Retinal changes - ***Go to question 92***

☐ Thrombosis - ***Go to question 92***

☐ None - ***Go to question 103***

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

93. 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 94***

☐ Quantitative CT – ***Go to question 98***

☐ Unknown - ***Go to question 103***

94. DEXA scan vertebral

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☐ Known – ***Go to question 95***

☐ Unknown – ***Go to question 96***

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

96. DEXA scan hip

☐ Known – ***Go to question 97***

☐ Unknown – ***Go to question 98***

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

98. Quantitative CT vertebral

☐ Known – ***Go to question 99***

☐ Unknown – ***Go to question 100***

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

100. Quantitative CT hip

☐ Known – ***Go to question 101***

☐ Unknown – ***Go to question 102***

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

102. Indicate if the DEXA scan report is attached

☐ Attached

☐ Not available

Copy and complete questions 91 - 102 to report multiple impairments.

Infection

103. Does the recipient have a history of clinically significant bacterial bloodstream infection? *(any prior clinically significant infection, e.g., sepsis)*

☐ Yes

☐ No

104. Does the recipient have a history of clinically significant bacterial bone infection? *(any prior clinically significant infection, e.g., osteomyelitis)*

☐ Yes

☐ No

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Disease Modifying Therapies

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

- ☐ Yes – ***Go to question 106***
- ☐ No – ***Go to question 112***
- ☐ Unknown – ***Go to question 112***

If there is more than one therapy given copy questions 106 - 111 for each therapy.

106. Specify the disease modifying therapy

- ☐ Crizanlizumab (Adakveo) – ***Go to question 108***
- ☐ Hydroxyurea – ***Go to question 108***
- ☐ L-Glutamine (Endari) – ***Go to question 108***
- ☐ Voxelotor (Oxbryta) – ***Go to question 108***
- ☐ Other – ***Go to question 107***

107. Specify other: _____

108. Date therapy started

- ☐ Known - ***Go to question 109***
- ☐ Unknown – ***Go to question 110***

109. Date therapy started: ___ ___ ___ ___ — ___ ___ — ___ ___ ☐ Date estimated
YYYY MM DD

110. Date therapy stopped

- ☐ Known – ***Go to question 111***
- ☐ Unknown – ***Go to question 112***
- ☐ Not applicable *(still receiving therapy)* – ***Go to question 112***

111. Date therapy stopped: ___ ___ ___ ___ — ___ ___ — ___ ___ ☐ Date estimated
YYYY MM DD

If there is more than one therapy given copy questions 106 - 111 for each therapy.

Other Laboratory Studies

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

- ☐ Yes – **Go to question 113**

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☐ No – **Go to question 127**

☐ Not applicable *(transfused within the last month)* – **Go to question 127**

☐ Unknown – **Go to question 127**

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

Specify the hemoglobin allele types based on the sample tested in question 113.

114. Hb A1

☐ Yes – ***Go to question 115***

☐ No – ***Go to question 116***

115. Hb A1: ___ ___ %

116. Hb A2

☐ Yes – ***Go to question 117***

☐ No – ***Go to question 118***

117. Hb A2: ___ ___ %

118. Hb C

☐ Yes – ***Go to question 119***

☐ No – ***Go to question 120***

119. Hb C: ___ ___ %

120. Hb F

☐ Yes – ***Go to question 121***

☐ No – ***Go to question 122***

121. Hb F: ___ ___ %

122. Hb S

☐ Yes – ***Go to question 123***

☐ No – ***Go to question 124***

123. Hb S: ___ ___ %

124. Other sickle related hemoglobin allele type

☐ Yes – ***Go to question 125***

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☐ No – ***Go to question 127***

125. Specify: _____

126. Level: ___ ___ %

127. Were absolute reticulocyte counts tested?

☐ Yes – ***Go to question 128***

☐ No – ***Go to question 130***

☐ Unknown – ***Go to question 130***

128. Absolute reticulocyte count: ___ ___ ___ ___ • ___ ___ ___ ___ ☐ x 10^3/ µL (x 10^9/L) (x 10^3/mm^3)
☐ x 10^6/ µL (x 10^{12}/L)

129. Date sample drawn: __ __ __ __ — __ __ — __ __ ☐ Date estimated
YYYY MM DD

130. Were soluble transferrin receptors (sTfR) tested?

☐ Yes – ***Go to question 131***

☐ No – ***Go to question 133***

☐ Unknown – ***Go to question 133***

131. sTfR: ___ ___ • ___ mg/L

132. Date sample drawn: __ __ __ __ — __ __ — __ __ ☐ Date estimated
YYYY MM DD

133. Was an erythropoietin (EPO) level tested?

☐ Yes – ***Go to question 134***

☐ No – ***Go to question 136***

☐ Unknown – ***Go to question 136***

134. EPO level: ___ ___ ___ IU/L

135. Date sample drawn: __ __ __ __ — __ __ — __ __ ☐ Date estimated
YYYY MM DD

136. Was a D-dimer level tested?

☐ Yes – ***Go to question 137***

☐ No – ***Go to question 138***

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☐ Unknown – ***Go to question 138***

137. D-dimer level: __ __ __ . __ __ ☐ ng/mL
☐ µg/mL
☐ mg/mL

Reason for Infusion

138. What was the primary reason for infusion?

☐ Acute chest syndrome **– *Go to question 140***
☐ Central nervous system event **– *Go to question 140***
☐ Priapism **– *Go to question 140***
☐ Pulmonary hypertension **– *Go to question 140***
☐ Vaso-occlusive pain **– *Go to question 140***
☐ Other– ***Go to question 139***

139. Specify other: _____

Marrow Evaluation at Last Evaluation

Complete question 140 for gene therapy infusions only

140. 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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