**Blood and Marrow Transplant Clinical Trials Network**

# 1507A (ENR)

**Web Version: 1.0;** 2.00; 03-02-18

## Haplo Sickle Cell Enrollment Form: Segment A

## Inclusion Criteria For Stratum 1: Children Ages 5.00 - 14.99 Years At Enrollment

1. Has the patient experienced a neurological event resulting in focal neurologic deficits that lasted ≥ 24 hours? *(HSCNEUIN)* ☐ 1 - Yes ☐ 2 - No
2. Date of most recent neurological event resulting in focal neurologic deficits lasting ≥ 24 hours: *(HSCNIDT)* _____ *(mm/dd/yyyy)*
3. Has the patient experienced a focal neurological event resulting in abnormalities on T2-weighted or FLAIR images using an MRI scan, indicative of an acute infarct, with no other reasonable medical explanation? *(HSCSTROK)* ☐ 1 - Yes ☐ 2 - No
4. Date of most recent focal neurological event resulting in abnormalities on T2-weighted or FLAIR images using an MRI scan: *(HSCSTRDT)* _____ *(mm/dd/yyyy)*
5. Was there clinical or radiologic evidence of a recent cerebral infarct by cerebral MRI/MRA within 30 days prior to enrollment? *(HSCRECNE)* ☐ 1 - Yes ☐ 2 - No
6. Was the patient previously deferred for ≥ 6 months due to clinical or radiologic evidence of a recent cerebral infarct by cerebral MRI/MRA within 30 days prior to enrollment? *(HSCDEFER)* ☐ 1 - Yes ☐ 2 - No
7. Record the date of the cerebral MRI/MRA with evidence of a cerebral infarct: *(HSCMRIDT)* _____ *(mm/dd/yyyy)*
8. Record the date of the repeat cerebral MRI/MRA with evidence of stabilization of the cerebral infarct: *(HSCRMRDT)* _____ *(mm/dd/yyyy)*

## Inclusion Criteria For Stratum 2: Adults Ages 15.00 - 45.99 At Enrollment

9. Has the patient experienced a neurological event resulting in focal neurologic deficits that lasted ≥ 24 hours? *(HSCNEURO)*
   1 - Yes
   2 - No
   3 - Not Available
10. Date of most recent neurological event resulting in focal neurologic deficits lasting ≥ 24 hours: *(HSCNEUDT)* _____ *(mm/dd/yyyy)*
11. Specify why neurological event information was not available: *(HSCNEUSP)* _____
12. Has the patient experienced a focal neurological event resulting in abnormalities on T2-weighted or FLAIR images using an MRI scan, indicative of an acute infarct, with no other reasonable medical explanation? *(HSCNEUEV)*
    1 - Yes
    2 - No
    3 - Not Available
13. Date of most recent focal neurological event resulting in abnormalities on T2-weighted or FLAIR images using an MRI scan: *(HSCNEDT)* _____ *(mm/dd/yyyy)*
14. Specify why focal neurological event information was not available: *(HSCFNESP)* _____
15. Does the patient have a history of two or more episodes of acute chest syndrome (ACS) in the two-year period preceding enrollment despite the institution of supportive care measures (i.e. asthma therapy and/or hydroxyurea)? *(HSCACS)*
    1 - Yes
    2 - No
    3 - Not Available
16. Date of ACS episodes: *(HSCAC1DT)* _____ *(mm/dd/yyyy)* *(HSCAC2DT)* _____ *(mm/dd/yyyy)*
17. Specify why ACS information was not available: *(HSCACSSP)* _____
18. Does the patient have a history of three or more severe vaso-occlusive pain crises per year in the two-year period preceding enrollment despite the institution of supportive care measures (i.e. a pain management plan and/or treatment with hydroxyurea); painful episodes related to priapism, osteonecrosis or any sickle-related complication? *(HSCVOC)*
    1 - Yes
    2 - No
    3 - Not Available

| | Start Date of Severe Pain Crisis #1 | Start Date of Severe Pain Crisis #2 | Start Date of Severe Pain Crisis #3 |
|---|---|---|---|
| 19. 2 Years Prior | *(HSC2P1DT)* _____ *(mm/dd/yyyy)* | *(HSC2P2DT)* _____ *(mm/dd/yyyy)* | *(HSC2P3DT)* _____ *(mm/dd/yyyy)* |
| 20. 1 Year Prior | *(HSC1P1DT)* _____ *(mm/dd/yyyy)* | *(HSC1P2DT)* _____ *(mm/dd/yyyy)* | *(HSC1P3DT)* _____ *(mm/dd/yyyy)* |

21. Specify why pain crises information was not available:*(HSCPCSP)*

22. Has the patient received ≥ 8 packed red blood cell (RBC) transfusions per year for ≥ 1 year in the 12 months before enrollment to prevent vaso-occlusive clinical complications (i.e. pain, stroke, and acute chest syndrome)?*(HSCRBC)*
- 1 - Yes
- 2 - No
- 3 - Not Available

| | Start Date of Transfusion |
|---|---|
| 23. RBC Transfusion #1: | *(HSCR1DT)* *(mm/dd/yyyy)* |
| 24. RBC Transfusion #2: | *(HSCR2DT)* *(mm/dd/yyyy)* |
| 25. RBC Transfusion #3: | *(HSCR3DT)* *(mm/dd/yyyy)* |
| 26. RBC Transfusion #4: | *(HSCR4DT)* *(mm/dd/yyyy)* |
| 27. RBC Transfusion #5: | *(HSCR5DT)* *(mm/dd/yyyy)* |
| 28. RBC Transfusion #6: | *(HSCR6DT)* *(mm/dd/yyyy)* |
| 29. RBC Transfusion #7: | *(HSCR7DT)* *(mm/dd/yyyy)* |
| 30. RBC Transfusion #8: | *(HSCR8DT)* *(mm/dd/yyyy)* |

31. Specify why RBC transfusion therapy information was not available: *(HSCRBCSP)*

32. Does the patient have an echocardiographic finding of tricuspid valve regurgitant jet velocity (TRJV) ≥ 2.7m/sec?*(HSCTRJV)* ☐ 1 - Yes ☐ 2 - No

33. Record TRJV:*(HSCTRJVA)* *(xx.x)* m/sec

34. Date echocardiograph was performed:*(HSCECODT)* *(mm/dd/yyyy)*

## Inclusion Criteria For Both Strata (Stratum 1 and Stratum 2)

35. Does the patient have Hemoglobin SS disease (HbSS) or Hemoglobin Sβ° (HbSβ°) Thalassemia?*(HSCHG)* ☐ 1 - Yes ☐ 2 - No

36. Record the type of SCD:*(HSCSCTYP)* ☐ 1 - Hemoglobin SS Disease (HbSS) ☐ 2 - Hemoglobin Sβ° (HbSβ°) Thalassemia

37. Performance status scale used to evaluate patient (Lansky for patients < 16 years old; Karnofsky for patients ≥ 16):*(HSCPSS)* ☐ 1 - Karnofsky ☐ 2 - Lansky

38. Record patient's performance status:*(HSCPSR)*
- 01 - 100 (Normal; No Complaints/Fully Active)
- 02 - 90 (Normal Activity/Minor Restriction in Strenuous Play)
- 03 - 80 (Normal Activity with Effort/Restricted in Strenuous Play)
- 04 - 70 (Unable to Carry On Normal Activity/Less Time Spent in Play)
- 05 - 60 (Requires Occasional Assistance/Minimal Active Play)
- *Additional Options Listed Below

**Cardiac Function**

39. Record the type of cardiac fraction test performed:*(HSCCFT)*
- 1 - Left Ventricular Ejection Fraction (LVEF)
- 2 - Shortening Fraction

*If the left ventricular ejection fraction is reported as a range, the lowest value should be recorded.*

40. Left ventricular ejection fraction:*(HSCLVEF)* *(xxx)* % Date LV ejection fraction performed:*(HSCLVEDT)* *(mm/dd/yyyy)*

*If the left ventricular shortening fraction is reported as a range, the lowest value should be recorded.*

41. LV Shortening fraction:*(HSCLVSF)* *(xxx)* % Date LV shortening fraction performed:*(HSCSFDT)* *(mm/dd/yyyy)*

**Pulmonary Function**

42. Record patient's $O_2$ saturation:*(HSCO2SAT)* *(xxx)* % Date $O_2$ saturation obtained:*(HSCO2DT)* *(mm/dd/yyyy)*

43. Record patient's DLCO value (corrected for hemoglobin):*(HSCDLCO)* *(xxx)* % Date DLCO value obtained:*(HSCDLCDT)* *(mm/dd/yyyy)*

**Renal Function**

| | Most Recent Value | Institutional ULN for Age | Date Sample Obtained |
|---|---|---|---|
| 44. Serum Creatinine (mg/dL) | *(HSCSC)* *(x.xx)* | *(HSCSCULN)* *(x.xx)* | *(HSCSCDT)* *(mm/dd/yyyy)* |
| 45. Creatinine Clearance (mL/min/1.73m$^2$) | *(HSCCRCL)* *(xxx.x)* | | *(HSCCRDT)* *(mm/dd/yyyy)* |

**Hepatic Function**

46. Does the patient have hyperbilirubinemia?*(HSCHYPER)* ☐ 1 - Yes ☐ 2 - No

47. If 'Yes', is the hyperbilirubinemia a result of hyperhemolysis or a severe drop in hemoglobin post blood transfusion?*(HSCHYPHE)* ☐ 1 - Yes ☐ 2 - No

| | Most recent value | Institutional ULN for Age | Date Sample Obtained |
|---|---|---|---|
| 48. Direct Bilirubin (mg/dL) | *(HSCBILI)* *(x.x)* | *(HSCBILIU)* *(x.x)* | *(HSCBILDT)* *(mm/dd/yyyy)* |
| 49. ALT (units/L) | *(HSCALT)* *(xxx)* | *(HSCALULN)* *(xxx)* | *(HSCALTDT)* *(mm/dd/yyyy)* |
| 50. AST (units/L) | *(HSCAST)* *(xxx)* | *(HSCASULN)* *(xxx)* | *(HSCASTDT)* *(mm/dd/yyyy)* |

51. Is the patient currently receiving ≥ 8 packed red blood cell transfusions per year for ≥ 1 year or has the patient received ≥ 20 packed red blood cell transfusions (lifetime cumulative)?*(HSCBRBC)* ☐ 1 - Yes ☐ 2 - No

52. Was a liver MRI performed using a validated methodology per institutional preference (T2* or R2* or by ferriscan [R2 MRI]) for estimation of hepatic iron content?*(HSCLMRI)* ☐ 1 - Yes ☐ 2 - No

53. Record date of liver MRI:*(HSCLMRDT)* *(mm/dd/yyyy)*

54. Does the patient have significant clinical evidence of iron overload (estimated hepatic iron content ≥10mg Fe/g liver dry weight)?*(HSCFEOV)* ☐ 1 - Yes ☐ 2 - No

55. Was a liver biopsy performed?*(HSCLIVBX)* ☐ 1 - Yes ☐ 2 - No

56. Record date of liver biopsy:*(HSCLBXDT)* *(mm/dd/yyyy)*

57. Did the gastroenterology/hepatology consultation and histological examination document the absence of cirrhosis, bridging fibrosis, and active hepatitis?*(HSCCIRRH)* ☐ 1 - Yes ☐ 2 - No

58. Does the patient have a first-degree related HLA-haploidentical donor who is willing and able to donate bone marrow?*(HSCHAPLO)* ☐ 1 - Yes ☐ 2 - No

59. Date confirmatory typing completed:*(HSCHLADT)* *(mm/dd/yyyy)*

## Exclusion Criteria

60. Does the patient have an HLA-matched sibling who is able and willing to donate bone marrow?*(HSCHLAMS)* ☐ 1 - Yes ☐ 2 - No

61. Has the patient experienced an uncontrolled bacterial, viral, or fungal infection in the 6 weeks before enrollment (currently taking medication with evidence of progression of clinical symptoms or radiologic findings)?*(HSCINFEC)* ☐ 1 - Yes ☐ 2 - No

62. Does the patient have evidence of human immunodeficiency virus (HIV) infection or known HIV positive serology?*(HSCHIV)* ☐ 1 - Yes ☐ 2 - No

63. Has the patient received a previous hematopoietic stem cell transplant (HCT)? *(HSCHCT)* ☐ 1 - Yes ☐ 2 - No

64. Has the patient received a prior solid organ transplant?*(HSCORTXP)* ☐ 1 - Yes ☐ 2 - No

65. Has the patient participated in another clinical trial in which the patient received an investigational or off-label use of a drug or device within 3 months of enrollment? *(HSCCLTR)* ☐ 1 - Yes ☐ 2 - No

66. Is the patient pregnant or breastfeeding?*(HSCPREG)* ☐ 1 - Yes ☐ 2 - No ☐ 3 - Not Applicable

67. Is the patient pregnant or breastfeeding?*(HSCPREG)* ☐ 1 - Yes ☐ 2 - No ☐ 3 - Not Applicable

68. Does the patient have a clinically significant, uncontrolled autoimmune disease requiring active medical management (immunosuppressive therapy or chemotherapy), which, in the judgment of the local Principal Investigator, indicates that the patient could not tolerate transplantation?*(HSCAIMM)* ☐ 1 - Yes ☐ 2 - No

69. Is the patient a female of childbearing potential (FCBP) (all females > 10 years of age, unless post-menopausal for a minimum of 1 year before the time of consent or surgically sterilized)?*(HSCFCBP)* ☐ 1 - Yes ☐ 2 - No

70. If 'Yes', does the patient agree either (1) to practice 2 effective methods of contraception at the same time, or (2) to practice true abstinence when this is in line with the preferred and usual lifestyle of the subject, from the time of signing of informed consent through 12 months post-transplant?*(HSCCONTR)* ☐ 1 - Yes ☐ 2 - No

71. Does the patient (even if surgically sterilized) agree to practice effective barrier contraception, or agree to practice true abstinence from the time of signing informed consent through 12 months post-transplant?*(HSCMCONT)* ☐ 1 - Yes ☐ 2 - No

72. Is there a presence of anti-donor specific HLA antibodies?*(HSCANTI)* ☐ 1 - Yes ☐ 2 - No

*HLA antibody presence and specificity will be determined by solid phase immunoassays. An anti-donor specific HLA antibody will be considered positive when the mean fluorescence intensity (MFI) is higher than the cut-off defined by each institution. Recommended cut-off values are MFI >1000 for donor specific antibody to HLA-A, -B, and DRB1 and MFI >2000 for HLA-C, DQB1 and DPB1.*

73. Date solid phase immunoassay performed to determine HLA antibody presence and specificity:*(HSCIMMDT)* *(mm/dd/yyyy)*

74. Was MFI >1000 for donor specific antibody to HLA-A, -B, DRB1 and/or MFI >2000 for HLA-C, DQB1 and DPB1?*(HSCMFI)*
    1 - Yes
    2 - Yes, Approved by Protocol Chair and/or Protocol Officer
    3 - No

75. Date of Protocol Chair and/or Protocol Officer approval:*(HSCAPDT)* *(mm/dd/yyyy)*

## Donor Inclusion Criteria

76. Is the donor willing to donate bone marrow?*(HSCDWILL)* ☐ 1 - Yes ☐ 2 - No

77. Does the donor meet institutional and protocol-specified criteria for donation? *(HSCDCRIT)* ☐ 1 - Yes ☐ 2 - No

## Donor Exclusion Criteria

78. Does the donor have a clinically significant hemoglobinopathy?*(HSCHEM)* ☐ 1 - Yes ☐ 2 - No

## Consent for Use of Blood Samples for Optional Study-Specific Research

79. Did the patient give consent to provide blood samples for optional study-specific research?*(HSCPSMPL)* ☐ 1 - Yes ☐ 2 - No

80. Date patient consented to optional study-specific research samples: *(HSCPSMDT)* *(mm/dd/yyyy)*

## Donor Consent for Use of Blood Samples for Optional Study-Specific Research

81. Did the donor give consent to provide blood samples for optional study-specific research?*(HSCDSMPL)* ☐ 1 - Yes ☐ 2 - No

82. Date donor consented to optional study-specific research samples:*(HSCDSMDT)* *(mm/dd/yyyy)*

Comments:*(HSCCOMM)*

## Additional Selection Options for ENR

**Record patient's performance status:**
06 - 50 (Requires Considerable Assistance/No Active Play)
07 - 40 (Disabled/Able to Initiate Quiet Activities)
08 - 30 (Severely Disabled/Needs Assistance for Quiet Play)
09 - 20 (Very Sick/Limited to Very Passive Activity)
10 - 10 (Moribund; Completely Disabled)