Concomitant Medication (CM1)

Web Version: 1.0; 1.00; 08Dec20

Segment *(PROTSEG)*:A
CM Number *(CMSPID)*:

Only enter medications on this form taken on the day of enrollment.
NOTE: Hydroxyurea treatment taken within 30 days of enrollment is an exclusion criterion for this study.

Medication:*(CM1TRT)*

Start date:*(CM1STDT)*

(ddMMMyyyy) *(CM1STEST)*

0-Exact date
3-Day only unknown
2-Day and month unknown
1-Day, month, and year unknown

Indication:*(CM1INDC)*

Stop date:*(CM1ENDT)*

(ddMMMyyyy) *(CM1ENEST)*

(CM1ONGO) 9-Ongoing

Additional Selection Options for CM1

CM Number *(CMSPID)* (key field):

001-1
002-2
003-3
004-4
005-5
006-6
007-7
008-8
009-9
010-10
011-11
012-12
013-13
014-14
015-15
016-16
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033-33
034-34
035-35
036-36
037-37
038-38
039-39
040-40
041-41
042-42
043-43
044-44
045-45
046-46
047-47
048-48
049-49
050-50

Demographics (DEM)

Web Version: 1.0; 1.00; 08Dec20

Gender:*(GENDER)* ☐ M-Male ☐ F-Female

Date of birth:*(BIRTHDT)* _____ *(ddMMMyyyy)*

Ethnicity:*(ETHNIC)*

1-Hispanic or Latino
2-Not Hispanic or Latino
3-Not reported
4-Unknown

Indicate No or Yes for each race listed below. Yes may be checked for more than one race.

Check this box if the participant refused to identify his or her race:*(RACEREF)* ☐ 1-Refused

American Indian or Alaskan Native:*(AMERIND)* ☐ N-No ☐ Y-Yes

Asian:*(ASIAN)* ☐ N-No ☐ Y-Yes

Native Hawaiian or other Pacific Islander:*(PACIFIC)* ☐ N-No ☐ Y-Yes

Black or African American:*(BLACK)* ☐ N-No ☐ Y-Yes

White:*(WHITE)* ☐ N-No ☐ Y-Yes

Upon saving the screen, 'Race' will be populated based on the subject's response to the individual race questions above.

Race:*(RACE)*

00-UNKNOWN
99-MULTIPLE
01-AMERICAN INDIAN OR ALASKA NATIVE
02-ASIAN
03-NATIVE HAWAIIAN OR OTHER PACIFIC ISLANDER
*Additional Options Listed Below

Additional Selection Options for DEM

Race:
04-BLACK
05-WHITE

MAN01A (ENR)

Web Version: 1.0; 1.00; 08Dec20

Participant:*(PATID)* _____ *(xxx-xxx-xxx)*

Enrollment date:*(STARTDT)* _____ *(ddMMMyyyy)*

Inclusion Criteria

*All answers in this section must be YES for the participant to be eligible. The Not assessed option is only for those participants who **fail to meet eligibility criteria**, to document which criteria were not assessed (i.e., evaluation discontinued once participant was identified as ineligible).*

1. Diagnosis of sickle cell disease with genotype HbSS, HbS/β^0 Thalassemia, HbSD, or HbSO.*(V101INC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
2. Age 18-45 years (35-45 year-old patients must be followed at BCH).*(V102INC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
3. Receiving regularly-scheduled blood transfusions or exchange transfusions as part of existing medical care.*(V103INC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
4. Adequate hematologic parameters including: ☐ N-No ☐ Y-Yes ☐ A-Not assessed
   a. White blood cell (WBC) count within the range of 2.5 - 25.0 x 10^9/L
   b. Hemoglobin within the range of 7 - 11 g/dL
   c. Platelet count within the range of 150 - 700 x 10^9/L

   (V104INC)
5. Adequate organ function and performance status: ☐ N-No ☐ Y-Yes ☐ A-Not assessed
   a. Karnofsky performance status ≥70%
   b. Serum creatinine </=1.5 times the upper limit of normal for age, and calculated creatinine clearence or GFR >/=60 mL/min/1.73m^2.

   (V105INC)
6. Adequate venous access or an existing venous access device.*(V106INC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed

Exclusion Criteria

*All answers in this section must be NO for the participant to be eligible. The Not assessed option is only for those participants who **fail to meet eligibility criteria**, to document which criteria were not assessed (i.e., evaluation discontinued once participant was identified as ineligible).*

1. Participants who have uncontrolled illness including, but not limited to: ☐ N-No ☐ Y-Yes ☐ A-Not assessed
   a. Ongoing or active infection.
   b. Emergency room admission or hospitalization for SCD-related reason in the past 30 days.
   c. Major surgery in the past 30 days.
   d. Medical/psychiatric illness/social situations that would limit compliance with study requirements as determined by the treating physician.

   (V101EXC)
2. Known myelodysplasia of the bone marrow or abnormal bone marrow cytogenetics.*(V102EXC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
3. Receipt of an investigational study drug or procedure within 90 days of study enrollment.*(V103EXC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
4. Pregnant or breastfeeding.*(V104EXC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
5. Known acute hepatitis or evidence of moderate or severe portal fibrosis or cirrhosis on prior biopsy.*(V105EXC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
6. Known left ventricular ejection fraction <40%.*(V106EXC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
7. Known DLCO (corrected for hemoglobin), FEV1, and/or FVC < 50% of predicted. *(V107EXC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
8. ALT or AST > 2.5 X upper limit of normal or direct bilirubin > 2.0 mg/dL.*(V108EXC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
9. On hydroxyurea treatment.*(V109EXC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed
10. Requires placement of central line for apheresis.*(V110EXC)* ☐ N-No ☐ Y-Yes ☐ A-Not assessed

Laboratory Results (LAB)

Web Version: 1.0; 1.00; 08Dec20

Segment *(PROTSEG)*:A
Visit Number *(VISNO)*:

Baseline Visit Assessments

Baseline collection date:*(LABBASDT)* *(ddMMMyyyy)*

Were Baseline assessments performed?*(LABBASYN)* ☐ N-No ☐ Y-Yes ☐ NA-N/A

If No, reason not done:*(LABBASNO)*

1-Subject unable to comply
2-Subject refusal
3-Technical problem
4-Clinic error
5-Laboratory error
*Additional Options Listed Below

If Other, specify:*(LABBNOSP)*

Chemistry

	Result	***Expected Range***
Creatinine	*(LABCRRES)* *(x.x)* mg/dl	0.3-1.7 mg/dl
Aspartate Aminotransferase (AST)	*(LABASRES)* *(xx)* u/L	2-40 u/L
Alanine Aminotransferase (ALT)	*(LABALRES)* *(xx)* u/L	3-30 u/L
Bilirubin (direct)	*(LABBDRES)* *(x.x)* mg/dl	0.0-0.4 mg/dl
Bilirubin (total)	*(LABBTRES)* *(x.x)* mg/dl	0.3-1.2 mg/dl

Infectious Disease Marker Testing

HIV 1 antigen	*(LABHIV1A)*	☐ 0-Negative	☐ 1-Positive
HIV 2 antigen	*(LABHIV2A)*	☐ 0-Negative	☐ 1-Positive
HBc core antigen	*(LABHBC)*	☐ 0-Negative	☐ 1-Positive
HBV surface antigen	*(LABHBRES)*	☐ 0-Negative	☐ 1-Positive
HCV surface antibody	*(LABHCRES)*	☐ 0-Negative	☐ 1-Positive
HTLV I	*(LABHTLV1)*	☐ 0-Negative	☐ 1-Positive
HTLV II	*(LABHTLV2)*	☐ 0-Negative	☐ 1-Positive
Chagas	*(LABCHAGA)*	☐ 0-Negative	☐ 1-Positive

Day -7 Visit Assessments

Day -7 collection date:*(LABM7DT)* *(ddMMMyyyy)*

Were Day -7 assessments performed?*(LAB7ASYN)* ☐ N-No ☐ Y-Yes ☐ NA-N/A

If No, reason not done:*(LABM7NO)*

1-Subject unable to comply
2-Subject refusal
3-Technical problem
4-Clinic error
5-Laboratory error
*Additional Options Listed Below

If Other, specify:*(LAB7NOSP)*

Hemoglobin Electrophoresis

	Result		Expected Range
Total Hemoglobin	*(LABHGTOT)*	*(xx)* g/dL	6-16 g/dL
Hemoglobin A1 (HbA1)	*(LABHGA1)*	*(xx.x)* %	95-97.9%
Hemoglobin A2 (HbA2)	*(LABHGA2)*	*(x.x)* %	2-3.5%
Hemoglobin F (HbF)	*(LABHGF)*	*(x.x)* %	0-2.1%
Hemoglobin C (HbC)	*(LABHGC)*	*(xx)* %	0-95%
Hemoglobin S (HbS)	*(LABHGS)*	*(xx)* %	0-95%

Chemistry

	Result		Expected Range
Lactic acid dehydrogenase (LDH)	*(LABLDH)*	*(xxxx)* U/L	100-1000 U/L
Ferritin	*(LABFTL)*	*(xxxxx)* ng/mL	10-10,000 ng/mL

Pregnancy Test

Was a pregnancy test performed?*(LABPRGYN)* ☐ N-No ☐ Y-Yes ☐ NA-N/A

If Yes, specimen type:*(LABPGSPC)* ☐ U-Urine ☐ S-Serum

Specimen collection date:*(LABPRGDT)* *(ddMMMyyyy)*

Result:*(LABPGRES)* ☐ 0-Negative ☐ 1-Positive

Day -1 Visit Assessments

Day -1 collection date:*(LABM1DT)* *(ddMMMyyyy)*

Were Day -1 assessments performed?*(LAB1ASYN)* ☐ N-No ☐ Y-Yes ☐ NA-N/A

If No, reason not done:*(LABM1NO)*

1-Subject unable to comply
2-Subject refusal
3-Technical problem
4-Clinic error
5-Laboratory error
*Additional Options Listed Below

If Other, specify:*(LAB1NOSP)*

Blood Grouping

ABO*(LABABO)*

Rh*(LABRH)* ☐ N-- (Negative) ☐ P-+ (Positive)

Syphilis Antibody-Rapid Plasma Reagin (RPR)*(LABRPR)* ☐ N-Negative ☐ R-Reactive

RBC Antibody screening*(LABRBC)* ☐ N-Negative ☐ P-Positive

Day 0 Visit Assessments

Day 0 collection date:*(LABD0DT)* *(ddMMMyyyy)*

Were Day 0 assessments performed?*(LAB0ASYN)* ☐ N-No ☐ Y-Yes ☐ NA-N/A

If No, reason not done:*(LABD0NO)*

1-Subject unable to comply
2-Subject refusal
3-Technical problem
4-Clinic error
5-Laboratory error
*Additional Options Listed Below

If Other, specify:*(LAB0NOSP)*

PRE-Collection

CBC/Differential	Result	Expected Range
Hematocrit	*(LABHEMA1)* _____ *(xx)* %	20-51%
Reticulocytes	*(LABRETC1)* _____ *(xx)* %	0-30%
Platelets	*(LABPLAT1)* _____ *(xxx)* x10^3/microliter	100-750 x10^3/microliter
WBC	*(LABWBC1)* _____ *(xx.x)* x10^3/microliter	2.0-20.0 x10^3/microliter
Neutrophils (Abs)	*(LABNEU1)* _____ *(xx)* x10^3/microliter	1-10 x10^3/microliter
Lymphocytes (Abs)	*(LABLYMP1)* _____ *(x)* x10^3/microliter	1-5 x10^3/microliter
Basophils (Abs)	*(LABBASO1)* _____ *(x)* x10^3/microliter	0-2 x10^3/microliter
Eosinophils (Abs)	*(LABEOSI1)* _____ *(x)* x10^3/microliter	0-6 x10^3/microliter
Monocyte (Abs)	*(LABMONO1)* _____ *(xx)* x10^3/microliter	0-20 x10^3/microliter
Peripheral CD34+ count	*(LABCD341)* _____ *(xxx)* cells/microliter	0-200 cells/microliter

Start of Collection

CBC/Differential	Result	Expected Range
Hematocrit	*(LABHEMA2)* _____ *(xx)* %	20-51%
Reticulocytes	*(LABRETC2)* _____ *(xx)* %	0-30%
Platelets	*(LABPLAT2)* _____ *(xxx)* x10^3/microliter	100-750 x10^3/microliter
WBC	*(LABWBC2)* _____ *(xx.x)* x10^3/microliter	2.0-20.0 x10^3/microliter
Neutrophils (Abs)	*(LABNEU2)* _____ *(xx)* x10^3/microliter	1-10 x10^3/microliter
Lymphocytes (Abs)	*(LABLYMP2)* _____ *(x)* x10^3/microliter	1-5 x10^3/microliter
Basophils (Abs)	*(LABBASO2)* _____ *(x)* x10^3/microliter	0-2 x10^3/microliter
Eosinophils (Abs)	*(LABEOSI2)* _____ *(x)* x10^3/microliter	0-6 x10^3/microliter
Monocyte (Abs)	*(LABMONO2)* _____ *(xx)* x10^3/microliter	0-20 x10^3/microliter
Peripheral CD34+ count	*(LABCD342)* _____ *(xxx)* cells/microliter	0-200 cells/microliter

Additional Selection Options for LAB

If No, reason not done:
6-Investigator decision
99-Other

CD34 Negative Selection (NEG)

Web Version: 1.0; 1.01; 08Dec20

Segment *(PROTSEG)*:A
Visit Number *(VISNO)*:

Visit Date:*(NEGVISDT)* _____ *(ddMMMyyyy)*

CD34 Negative Selection

Name of sample processor:*(NEGPROC)* _____ *(First Last)*

Total number of cells processed:*(NEGTOTCL)* _____ *(xxx)* x 10^6 cell/mL

% of CD34 negative cells in collection:*(NEGCD34)* _____ *(xx)* %

Are the CD34 negative selection FACS plots available for upload to eClinical? *(NEGFACSA)* ☐ N-No ☐ Y-Yes

If Yes, were the plots uploaded? ☐ N-No ☐ Y-Yes

To upload the plot, use the [Upload file] button at the bottom of the screen. Select [Browse] and choose the file you intend to upload. Include a short description of the file (ex.: ParticipantID_NegSelect). Select [Upload]. *(NEGFACSU)*

Hematology	*Result*	*Expected Range*
Platelets	*(NEGPLT)* _____ *(xxx)* x10^3/microliter	100-750 x10^3/microliter
WBC	*(NEGWBC)* _____ *(xx.x)* x10^3/microliter	2.0-20.0 x10^3/microliter
Neutrophils (Abs)	*(NEGANC)* _____ *(xx)* x10^3/microliter	1-10 x10^3/microliter
Lymphocytes (Abs)	*(NEGLYM)* _____ *(xx)* x10^3/microliter	1-5 x10^3/microliter
Basophils (Abs)	*(NEGBASO)* _____ *(xx)* x10^3/microliter	0-2 x10^3/microliter
Eosinophils (Abs)	*(NEGEOS)* _____ *(xx)* x10^3/microliter	0-6 x10^3/microliter
Monocyte (Abs)	*(NEGMONO)* _____ *(xx)* x10^3/microliter	0-20 x10^3/microliter

CD34 Positive Selection (POS)

Web Version: 1.0; 1.01; 08Dec20

Segment *(PROTSEG)*:A
Visit Number *(VISNO)*:

Visit Date:*(POSVISDT)* _____ *(ddMMMyyyy)*

CD34 Positive Selection

Name of sample processor:*(POSPROC)* _____ *(First Last)*

Total number of cells processed:*(POSTOTCL)* _____ *(xxx)* x 10^6 cell/mL

% of CD34 positive cells in collection:*(POSCD34)* _____ *(xx)* %

Are the CD34 positive selection FACS plots available for upload to eClinical? *(POSFACSA)* ☐ N-No ☐ Y-Yes

If Yes, were the plots uploaded? ☐ N-No ☐ Y-Yes

To upload the plot, use the [Upload file] button at the bottom of the screen. Select [Browse] and choose the file you intend to upload. Include a short description of the file (ex.: ParticipantID_PosSelect). Select [Upload]. *(POSFACSU)*

Hematology	*Result*	*Expected Range*
Platelets	*(POSPLT)* _____ *(xxx)* x10^3/microliter	100-750 x10^3/microliter
WBC	*(POSWBC)* _____ *(xx.x)* x10^3/microliter	2.0-20.0 x10^3/microliter
Neutrophils (Abs)	*(POSANC)* _____ *(xx)* x10^3/microliter	1-10 x10^3/microliter
Lymphocytes (Abs)	*(POSLYM)* _____ *(xx)* x10^3/microliter	1-5 x10^3/microliter
Basophils (Abs)	*(POSBASO)* _____ *(xx)* x10^3/microliter	0-2 x10^3/microliter
Eosinophils (Abs)	*(POSEOS)* _____ *(xx)* x10^3/microliter	0-6 x10^3/microliter
Monocyte (Abs)	*(POSMONO)* _____ *(xx)* x10^3/microliter	0-20 x10^3/microliter

Pre-CD34 Selection (PRE)

Web Version: 1.0; 1.01; 08Dec20

Segment *(PROTSEG)*:A
Visit Number *(VISNO)*:

Visit Date:*(PREVISDT)* *(ddMMMyyyy)*

Pre-CD34 Selection

Name of sample processor:*(PREPROC)* *(First Last)*

Total blood volume processed:*(PRETOTBV)* *(xxx)* mL

Total number of cells collected:*(PRETOTCL)* *(xxxx)* x 10^6 cell/mL

Final volume of cell suspension:*(PRECLSUS)* *(xxx)* mL

% of CD34 cells in cells collected:*(PRECD34)* *(xx)* %

Are the Pre-CD34 selection FACS plots available for upload to eClinical? *(PREFACSA)* ☐ N-No ☐ Y-Yes

If Yes, were the plots uploaded? ☐ N-No ☐ Y-Yes

To upload the plot, use the [Upload file] button at the bottom of the screen. Select [Browse] and choose the file you intend to upload. Include a short description of the file (ex.: ParticipantID_PreSelect). Select [Upload]. *(PREFACSU)*

Hematology	*Result*	*Expected Range*
Hematocrit	*(PREHCT)* *(xx)* %	20-51%
Platelets	*(PREPLT)* *(xxx)* x10^3/microliter	100-750 x10^3/microliter
WBC	*(PREWBC)* *(xx.x)* x10^3/microliter	2.0-20.0 x10^3/microliter
Neutrophils (Abs)	*(PREANC)* *(xx)* x10^3/microliter	1-10 x10^3/microliter
Lymphocytes (Abs)	*(PRELYM)* *(xx)* x10^3/microliter	1-5 x10^3/microliter
Basophils (Abs)	*(PREBASO)* *(xx)* x10^3/microliter	0-2 x10^3/microliter
Eosinophils (Abs)	*(PREEOS)* *(xx)* x10^3/microliter	0-6 x10^3/microliter
Monocyte (Abs)	*(PREMONO)* *(xx)* x10^3/microliter	0-20 x10^3/microliter

Sickle Collection Intake (SCI)

Web Version: 1.0; 1.00; 08Dec20

Segment *(PROTSEG)*:A
Visit Number *(VISNO)*:

1.Visit date:*(SCIVISDT)* _____ *(ddMMMyyyy)*

Visit-Specific Information

2.Was the informed consent document signed and dated prior to any study procedures being performed?*(SCIICSD)* ☐ N-No ☐ Y-Yes

Participant-Specific Information

3.Sickle Cell Disease Genotype:*(SCISCDG)* ☐ 1-HbSS ☐ 2-HbSC ☐ 3-HbSβ^{0-} thalassemia ☐ 4-HbSβ^{+-} thalassemia ☐ 5-HbSD

4.Has the participant received chronic blood transfusions?*(SCIBLTFX)* ☐ N-No ☐ Y-Yes

a.If Yes, how many transfusions have been received in the past 12 months? *(SCITFNUM)* _____ *(xx)* transfusions

5.How many vaso-occlusive events has the participant experienced in the past 12 months?*(SCIVONUM)* _____ *(xx)* events

a.Number of these vaso-occlusive events which required narcotics or hospital admissions.*(SCIVONHA)* _____ *(xx)* events

6.Has the participant had a splenectomy?*(SCISPLEN)* ☐ N-No ☐ Y-Yes

a.If Yes, how was the procedure performed?*(SCISPPER)* ☐ 1-Laparoscopically ☐ 2-Surgically

7.Has the participant experienced any Central Nervous System (CNS) events? *(SCICNSEV)* ☐ N-No ☐ Y-Yes

a.If Yes, indicate all CNS event(s) which have occurred:*(SCISTROK)* ☐ 1-Stroke *(SCITIA)* ☐ 1-Transient ischemic attack *(SCISEIZR)* ☐ 1-Seizures *(SCICNSOT)* ☐ 1-Other

1.If Other, specify:*(SCICNSSP)* _____

8.Has the participant ever taken hydroxyurea (HU)?*(SCIHU)* ☐ N-No ☐ Y-Yes

a.If Yes, did they respond to taking HU?*(SCIHURES)* ☐ N-No ☐ Y-Yes

b.If Yes, are they presently taking HU?*(SCIHUPR)* ☐ N-No ☐ Y-Yes

9.What is the highest the participant's Hemoglobin F (HgF) has been?*(SCIHGF)* _____ *(xx)* % *(SCIHGUNK)* ☐ 1-Unknown

10.Has the participant undergone a Red Cell Exchange in the past year?*(SCIRCEX)* ☐ N-No ☐ Y-Yes

a.If Yes, when?*(SCIRCXDT)* _____ *(ddMMMyyyy)*

Visit-Specific Screening Information

11.Was blood drawn for screening hematology labs?*(SCIBLDHE)* ☐ N-No ☐ Y-Yes

12.Was blood drawn for screening serology tests (HIV, HBV, and HCV)?*(SCIBLDSE)* ☐ N-No ☐ Y-Yes

13.**Female participants only:** Was blood or urine collected for pregnancy testing? *(N/A if female of nonchildbearing potential)(SCIPRTST)* ☐ 1-No ☐ 2-Yes - blood ☐ 3-Yes - urine ☐ 4-N/A

Medications/Protocol Deviations

14.Is the participant currently taking any medications? ☐ N-No ☐ Y-Yes
If Yes, submit a Concomitant Medications form.(SCIMEDYN)

15.Has there been a protocol deviation?*(SCIPRDYN)* ☐ N-No ☐ Y-Yes