

Form 2037 R2.0: Leukodystrophies Pre-HSCT Data

Center:

CRID:

Key Fields

Sequence Number: _____

CIBMTR Center Number: _____

Date Received: ____ - ____ - ____

CIBMTR Recipient ID: _____

Today's Date: ____ - ____ - ____

Date of HSCT for which this form is being completed: ____ - ____ - ____

HSCT type: (check all that apply)

- ☐ Autologous
- ☐ Allogeneic, unrelated
- ☐ Allogeneic, related
- ☐ Syngeneic (identical twin)

Product type: (check all that apply)

- ☐ Marrow
- ☐ PBSC
- ☐ Cord blood
- ☐ Other product

Specify: _____

If this is a report of a second or subsequent transplant, check here and continue with question 32.

Leukodystrophy Diagnosis

Questions: 1 - 31

1 What is the date of diagnosis of Leukodystrophy? ____ - ____ - ____

2 Specify the leukodystrophy subtype:

- ☐ globoid cell leukodystrophy (Krabbe Disease)
- ☐ metachromatic leukodystrophy (MLD)
- ☐ adrenoleukodystrophy (ALD)

Specify the leukocyte galactocerebrosidase enzyme activity at diagnosis:

3 Date recipient tested:

- ☐ Known
- ☐ Not known

4 ____ - ____ - ____

5 Recipient result:

- ☐ Known
- ☐ Not known

6 _____

☐ nmol/hr/mg protein ☐ pmol/hr/mg protein

7 Donor result:

- ☐ Known
- ☐ Not known

8 _____

☐ nmol/hr/mg protein ☐ pmol/hr/mg protein

Specify the leukocyte arylsulfatase A enzyme activity at diagnosis:

9 Date recipient tested:

- ☐ Known
- ☐ Not known

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10 _____

11 Recipient result:

☐ Known ☐ Not known

12 _____

☐ nmol/hr/mg protein ☐ pmol/hr/mg protein

13 Were the recipient's urinary sulfatides elevated at diagnosis?

☐ Yes ☐ No ☐ Not known

14 Donor result:

☐ Known ☐ Not known

15 _____

☐ nmol/hr/mg protein ☐ pmol/hr/mg protein

16 Mean fasting plasma very-long-chain fatty acid(VLCFA) C26:0 level at diagnosis?

☐ Known ☐ Not known

17 _____ µg/mL

18 Was the acid level measured within two weeks prior to the preparative regimen?

☐ yes ☐ no ☐ Unknown

19 Date recipient tested: _____

20 Plasma level: _____ µg/mL

21 Was treatment given for adrenal insufficiency between diagnosis and HSCT?

☐ yes ☐ no ☐ Unknown

Specify adrenal insufficiency:

22 Glucocorticoid

☐ yes ☐ no ☐ Unknown

23 Mineralocorticoid

☐ yes ☐ no ☐ Unknown

24 Was treatment given to lower plasma very-long-chain fatty acids at any time prior to HSCT?

☐ yes ☐ no ☐ Unknown

Specify treatments:

25 4-phenylbutyrate

☐ yes ☐ no ☐ Unknown

26 GTE:GTO oil (Lorenzo's oil)

☐ yes ☐ no ☐ Unknown

27 Lovastatin or related compound

☐ yes ☐ no ☐ Unknown

28 Other

☐ yes ☐ no ☐ Unknown

29 Specify: _____

30 Donor's mean fasting VLCFA C26:0 level:

☐ Known ☐ Not known

31 _____ µg/mL

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Clinical Status Prior to Preparative Regimen

Questions: 32 - 93

32 Is there a history of seizures at any time prior to the preparative regimen?

☐ yes ☐ no ☐ Unknown

33 Was cerebrospinal fluid(CSF) testing done prior to the preparative regimen?

☐ yes ☐ no ☐ Unknown

Specify results of the most recent testing:

34 Date of most recent testing: ____ - ____ - ____

35 Opening pressure:

☐ Known ☐ Not known

36 _____ cm H₂O

37 Closing pressure:

☐ Known ☐ Not known

38 _____ cm H₂O

39 Total protein:

☐ Known ☐ Not known

40 _____
☐ mg/dL ☐ g/L

41 Was Magnetic Resonance Imaging(MRI) performed at any time prior to the perparative regimen?

☐ yes ☐ no ☐ Unknown

42 Date of most recent test prior to the preparative regimen: ____ - ____ - ____

43 Specify MRI results:

☐ Normal ☐ Abnormal ☐ Not known

44 Is a copy of the MRI report attached?

☐ yes ☐ no

45 Was Magnetic Resonance Spectroscopy performed at any time prior to the preparative regimen?

☐ yes ☐ no ☐ Unknown

46 Date of most recent test prior to the preparative regimen: ____ - ____ - ____

47 Specify MRS results:

☐ Normal ☐ Abnormal ☐ Not known

48 Is a copy of the MRS report attached?

☐ yes ☐ no

49 Were nerve conduction velocities tested at any time prior to the preaprative regimen?

☐ yes ☐ no ☐ Unknown

50 Date of most recent test prior to the preparative regimen: ____ - ____ - ____

51 Specify results:

☐ Normal ☐ abnormal/impaired ☐ Not known

52 Is a copy of the report attached?

☐ yes ☐ no

53 Was a Mental Development test administered at any time prior to the preparative regimen?

☐ yes ☐ no ☐ Unknown

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54 Date of most recent test prior to the preparative regimen: ____ - ____ - ____

55 Specify test instrument administered:

- ☐ Bayley Scales of Infant Development
- ☐ Stanford Binet Intelligence Scale
- ☐ Wechsler Preschool and Primary Scale of Intelligence (WPPSI - Revised)
- ☐ Wechsler Intelligence Scale for Children - III (WISC - III)
- ☐ other test

56 Specify other test: _____

57 Full scale score: (not percentile)

☐ Known ☐ Not known

58 Score: _____

59 Performance score: (not percentile)

☐ Known ☐ Not known

60 Score: _____

61 Verbal score: (not percentile)

☐ Known ☐ Not known

62 Score: _____

63 Were the Vineland Adaptive Behavior Scales administered at any time prior to the preparative regimen?

☐ yes ☐ no ☐ Unknown

64 Date of most recent test prior to the preparative regimen: ____ - ____ - ____

65 Communication skills:

☐ Known ☐ Not known

66 Score: _____

67 Daily living skills

☐ Known ☐ Not known

68 Score: _____

69 Socialization skills

☐ Known ☐ Not known

70 Score: _____

71 Was visual acuity tested at any time prior to the preparative regimen?

☐ yes ☐ no ☐ Unknown

72 Is the recipient blind?

☐ yes ☐ no

73 Date of most recent exam prior to the preparative regimen: ____ - ____ - ____

74 Visual acuity of right eye(OD): (uncorrected vision)

☐ Known ☐ Not known

75 Score: _____ / _____

76 Visual acuity of left eye (OS): (uncorrected vision)

☐ Known ☐ Not known

77 Score: _____ / _____

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78 Visual acuity of both eyes (OU): (uncorrected vision)



Known



Not known

79 Score: _____ / _____

80 Did the recipient undergo an ophthalmologic exam under anesthesia at any time prior to the preparative regimen?



yes



no



Unknown

81 Date of most recent test prior to the preparative regimen: ____ - ____ - ____

82 Specify results:



Normal



abnormal/impaired



Not known

83 Is a copy of the report attached?



yes



no

84 Was the hearing loss (HL) in decibels (dB) assessed at the speech threshold for 500 hertz (HZ) at any time prior to the preparative regimen?



yes



no



Unknown

85 Date of most recent evaluation:



Known



Not known

86 ____ - ____ - ____

Specify tympanometry results:

87 Right ear



normal/mild (0-20 dB HL/25-40 dB HL)



moderate/moderately severe (45-55 dB HL/60-70 dB HL)



severe/profound (75-90 dB HL/>90 dB HL)

88 Left ear



normal/mild (0-20 dB HL/25-40 dB HL)



moderate/moderately severe (45-55 dB HL/60-70 dB HL)



severe/profound (75-90 dB HL/>90 dB HL)

89 Was the hearing loss (HL) in decibels (dB) assessed at the speech threshold for 2000 hertz (HZ) at any time prior to the preparative regimen?



yes



no



Unknown

90 Date of most recent evaluation:



Known



Not known

91 ____ - ____ - ____

Specify tympanometry results:

92 Right ear



normal/mild (0-20 dB HL/25-40 dB HL)



moderate/moderately severe (45-55 dB HL/60-70 dB HL)



severe/profound (75-90 dB HL/>90 dB HL)

93 Left ear



normal/mild (0-20 dB HL/25-40 dB HL)



moderate/moderately severe (45-55 dB HL/60-70 dB HL)



severe/profound (75-90 dB HL/>90 dB HL)

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Center:

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First Name: Last Name:

Phone number: Fax number:

E-mail address: