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
Specify the leukocyte arylsulfatase A enzyme activity at diagnosis:

9 Date recipient tested:
   Known [ ] Not known [ ]

10 [ ] [ ] [ ] __ __ __ __

11 Recipient result:
   Known [ ] Not known [ ]

12 [ ] [ ] [ ] __ __ __ __

13 Were the recipient’s urinary sulfatides elevated at diagnosis?
   Yes [ ] No [ ] Not known [ ]

14 Donor result:
   Known [ ] Not known [ ]

15 [ ] [ ] [ ] __ __ __ __

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 [ ]
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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 [ ]
   __________________________ μg/mL

31 Specify results of the most recent testing:

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 [ ]

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

35 Opening pressure:
   Known [ ] Not known [ ]
   __________________________ cm H₂O

36 Closing pressure:
   Known [ ] Not known [ ]
   __________________________ cm H₂O

37 Total protein:
   Known [ ] Not known [ ]
   __________________________ mg/dL [ ] g/L [ ]

38 Was Magnetic Resonance Imaging (MRI) performed at any time prior to the preparative regimen?
   yes [ ] no [ ] Unknown [ ]

39 Date of most recent test prior to the preparative regimen: __ __ __ __ - __ __ __ __

40 Specify MRI results:
   Normal [ ] Abnormal [ ] Not known [ ]

41 Is a copy of the MRI report attached?
   yes [ ] no [ ]
Was Magnetic Resonance Spectroscopy performed at any time prior to the preparative regimen?

- yes
- no
- Unknown

Date of most recent test prior to the preparative regimen: __ __ __ __

Specify MRS results:

- Normal
- Abnormal
- Not known

Is a copy of the MRS report attached?

- yes
- no

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

- yes
- no
- Unknown

Date of most recent test prior to the preparative regimen: __ __ __ __

Specify results:

- Normal
- abnormal/impaired
- Not known

Is a copy of the report attached?

- yes
- no

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

- yes
- no
- Unknown

Date of most recent test prior to the preparative regimen: __ __ __ __

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

Specify other test: _______________________

Full scale score: (not percentile)

- Known
- Not known

Score:

Performance score: (not percentile)

- Known
- Not known

Score:

Verbal score: (not percentile)

- Known
- Not known

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

- yes
- no
- Unknown

Date of most recent test prior to the preparative regimen: __ __ __ __

Communication skills:

- Known
- Not known

Score: ___________________

Daily living skills

- Known
- Not known

Score: ___________________

Socialization skills

- Known
- Not known

Score: ___________________

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

- yes
- no
- Unknown

Is the recipient blind?

- yes
- no

Date of most recent exam prior to the preparative regimen: __ __ __ __

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

- Known
- Not known

Score: ___________________ / ___________________

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

- Known
- Not known

Score: ___________________ / ___________________

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

- Known
- Not known

Score: ___________________ / ___________________

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

- yes
- no
- Unknown

Date of most recent test prior to the preparative regimen: __ __ __ __

Specify results:

- Normal
- abnormal/impaired
- Not known

Is a copy of the report attached?

- yes
- no

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
Date of most recent evaluation:

Known \[\checkmark\] Not known

Specify tympanometry results:

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)

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)

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 \[\checkmark\] No \[\checkmark\] Unknown

Date of most recent evaluation:

Known \[\checkmark\] Not known

Specify tympanometry results:

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)

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)