

National Marrow Donor Program®
Insert XIII – Leukodystrophies

Registry Use Only

Sequence Number:

Date Received:

Unrelated

Recipient NMDP ID: - -

Recipient Last Name:

Recipient Local ID (optional):

Today's Date: / / TC Code:

Month Day Year

Date of Transplant for which this form is being completed: / /

Month Day Year

Product type: Marrow (Form 120) PBSC (Form 520) Cord blood (Form 620)

This form must be accompanied by Form 120, 520, 620 – Recipient Baseline and Transplant Data. All information in the box above, including the date, should be identical with the corresponding Form 120, 520, 620. Information should come from an actual examination by the Transplant Center physician, or the physician who followed the recipient post-transplant, or abstraction of the recipient's medical records.

1. For which type of leukodystrophy was the transplant performed?

Globoid Cell Leukodystrophy →

2. Report the leukocyte galactocerebrosidase enzyme activity at diagnosis:

Result: • 1 nmol/hr/mg protein 2 pmol/hr/mg protein

Date tested: / /

Month Day Year

3. Report the **donor's** leukocyte galactocerebrosidase level:

Result: • 1 nmol/hr/mg protein 2 pmol/hr/mg protein

Metachromatic Leukodystrophy →

4. Report the leukocyte arylsulfatase A enzyme activity at diagnosis:

Result: • 1 nmol/hr/mg protein 2 pmol/hr/mg protein

5. Report the urinary sulfatides at diagnosis:

Urinary level: g/mL

6. Report the **donor's** leukocyte arylsulfatase A level:

Result: • 1 nmol/hr/mg protein 2 pmol/hr/mg protein

Adrenoleukodystrophy →

7. Report the mean fasting plasma very-long-chain fatty acid (VLCFA) C26:0 as determined at diagnosis:

Plasma level: • μg/mL

Mail to NMDP Registry with Form 120, 520, 620. Retain a copy at the transplant center.

Recipient NMDP ID: - -

Recipient Last Name:

8. Was the mean fasting plasma very-long-chain-fatty acid level measured pre-transplant (within two weeks prior to conditioning for transplant)?

1 yes →
2 no
3 unknown

9. Specify:

Plasma level: • µg/mL

Date tested: Month Day Year

10. Was treatment given for adrenal insufficiency between diagnosis and transplant?

1 yes →
2 no
3 unknown

11. Specify:

a. Glucocorticoid 1 yes 2 no 3 unknown
b. Mineralocorticoid 1 yes 2 no 3 unknown

12. Was treatment given to lower plasma very-long-chain fatty acids at any time prior to transplant?

1 yes →
2 no
3 unknown

13. Specify:

a. GTE:GTO oil (Lorenzo's oil) 1 yes 2 no 3 unknown
b. Lovastatin or related compound 1 yes 2 no 3 unknown
c. 4-phenylbutyrate 1 yes 2 no 3 unknown
d. Other, specify: _____ 1 yes 2 no 3 unknown

Clinical Status Pre-Transplant

14. Is there a history of pre-transplant seizures?

- 1 yes
2 no
3 unknown

15. Was cerebrospinal fluid (CSF) testing done pre-transplant?

- 1 yes →
2 no
3 unknown

16. Report results of most recent tests:

a. Opening pressure → cm H₂O
1 yes →
2 no
3 unknown

b. Total protein → • 1 mg/dL 2 g/L
1 yes →
2 no
3 unknown

c. **Serum** albumin → • 1 mg/dL 2 g/L
1 yes →
2 no
3 unknown

d. **Serum** IgG → • 1 mg/dL 2 g/L
1 yes →
2 no
3 unknown

17. Date of most recent test: Month Day Year

Recipient NMDP ID: - -

Recipient Last Name:

18. Magnetic Resonance Imaging (MRI) pre-transplant:

- 1 normal
- 2 abnormal
- 3 unknown/
not done

19. Date of most recent report:
(If possible, attach a copy of the report.)

Month Day Year

20. Magnetic Resonance Spectroscopy pre-transplant:

- 1 normal
- 2 abnormal
- 3 unknown/
not done

21. Date of most recent test prior to transplant:
(If possible, attach a copy of the report.)

Month Day Year

22. Were nerve conduction velocities tested pre-transplant?

- 1 yes
- 2 no
- 3 unknown

23. Specify nerve conduction velocities:

a. Median nerve: m/sec

b. Peroneal nerve: m/sec

24. Date of most recent test prior to transplant:

Month Day Year

25. Was a Mental Development test done pre-transplant?

- 1 yes
- 2 no
- 3 unknown

26. Indicate test instrument and report results of test done closest to transplant; report score, not percentile:

- 1 Bayley Scales of Infant Development
- 2 Stanford-Binet Intelligence Scale 4th ed
- 3 Wechsler Preschool and Primary Scale of Intelligence (WPPSI – Revised)
- 4 Wechsler Intelligence Scale for Children – III (WISC – III)
- 5 Other, specify: _____

27. Date of test:

Month Day Year

28. Full scale score:

29. Verbal score:

30. Performance score:

31. Were the Vineland Adaptive Behavior Scales done pre-transplant?

- 1 yes
- 2 no
- 3 unknown

32. Score results:

a. Communication skills:

b. Daily living skills:

c. Socialization skills:

33. Date of test:

Month Day Year

Recipient NMDP ID: - -

Recipient Last Name:

34. Was visual acuity tested pre-transplant?

- 1 yes
 2 no
 3 unknown

35. Is patient blind?
 1 yes
 2 no

36. Visual acuity:
 a. Right eye: /
 b. Left eye: /

37. Date of test: / /
 Month Day Year

38. Was an audiologic evaluation (auditory brain stem or conditioned response) done pre-transplant?

- 1 yes
 2 no
 3 unknown

39. Tympanometry results:

	Normal	Retracted	Flat
a. Right ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>
b. Left ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>

40. Was the hearing loss (HL) in decibels (dB) assessed at the speech threshold for 500 hertz (HZ)?

- 1 yes
 2 no
 3 unknown

41. Speech Threshold results at 500 HZ:

	Normal - Mild	Moderate - Moderately Severe	Severe - Profound
a. Right ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>
b. Left ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>

See Degree of Hearing Loss chart below for scale ranges.

42. Was the hearing loss (HL) in decibels (dB) assessed at the speech threshold for 2000 hertz (HZ)?

- 1 yes
 2 no
 3 unknown

43. Speech Threshold results at 2000 HZ:

	Normal - Mild	Moderate - Moderately Severe	Severe - Profound
a. Right ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>
b. Left ear:	1 <input type="checkbox"/>	2 <input type="checkbox"/>	3 <input type="checkbox"/>

See Degree of Hearing Loss chart below for scale ranges.

Degree of Hearing Loss: Pure tones and speech testing

Normal:	0-20 dB HL	Moderately Severe:	60-70 dB HL
Mild:	25-40 dB HL	Severe:	75-90 dB HL
Moderate:	45-55 dB HL	Profound:	> 90 dB HL