

ERROR CORRECTION FORM

Sequence Number:

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CIBMTR Recipient ID:

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Initials:

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Today's Date:

		2	0		
Month	Day	Year			

Infusion Date:

		2	0		
Month	Day	Year			

CIBMTR Center Number:

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Mucopolysaccharidosis and Other Storage Diseases Post-HSCT Data

Registry Use Only

Sequence Number:

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Date Received:

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		2	0		
Month	Day	Year			

Date of HSCT for which this form is being completed:

Month	Day	Year			

HSCT type: autologous allogeneic, unrelated allogeneic, related syngeneic (identical twin)

Product type: marrow PBSC cord blood other product, specify: _____

Visit: 100 day 6 month 1 year 2 years > 2 years, specify: _____

To be completed in conjunction with a Form 2100 – 100 Days Post-HSCT Data, Form 2200 – Six Months to Two Years Post-HSCT Data, or Form 2300 – Yearly Follow-Up for Greater Than Two Years Post-HSCT Data. Information reported here should reflect the date of last contact as reported in the post-HSCT data collection form, or immediately prior to death.

1. Indicate the enzyme that was evaluated for activity level in the first 100 days post-HSCT. The enzyme reported on this form must correspond to the enzyme found deficient at diagnosis.

Mucopolysaccharidosis

- 1 α -L-iduronidase (Hurler / Scheie syndrome – MPS I)
- 2 iduronate sulfatase (Hunter syndrome – MPS II)
- 3 heparan N-sulfatase (Sanfilippo A – MPS IIIA)
- 4 α -N-acetylglucosaminidase (Sanfilippo B – MPS IIIB)
- 5 acetyl-CoA-glucosaminide acetyltransferase (Sanfilippo C – MPS IIIC)
- 6 N-acetylglucosamine 6-sulfatase (Sanfilippo D – MPS IIID)
- 7 N-acetylgalactosamine 6-sulfatase (Morquio A – MPS IVA)
- 8 β -galactosidase (Morquio B – MPS IVB)
- 9 N-acetylgalactosamine 4-sulfatase (Maroteaux-Lamy syndrome – MPS VI)
- 10 β -glucuronidase (Sly syndrome – MPS VII)

Other Storage Diseases

- 11 glucocerebrosidase (Gaucher disease)
- 12 acid sphingomyelinase (Niemann-Pick disease)
- 13 N-acetylglucosamine-1-phosphotransferase (mucopolipidosis II or I-cell)
- 14 acid lipase deficiency (Wolman disease)
- 15 α -fucosidase deficiency (fucosidosis)
- 16 neuronal ceroid lipofuscinosis enzyme — NCL 1 (infantile): PPT-palmitoyl protein thioesterase
- 17 neuronal ceroid lipofuscinosis enzyme — NCL 2 (classic late infantile): transpeptidase
- 18 α -mannosidase B deficiency (α -mannosidosis)
- 19 N-aspartyl- β -glucosaminidase (aspartylglucosaminuria)
- 20 hypoxanthine-guanine phosphoribosyltransferase deficiency (Lesch-Nyhan syndrome)
- 21 other storage disease → 2. Specify: _____

3. Specify the recipient's enzyme activity level:

				.	
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- 1 nmol/hr/mg protein
- 2 pmol/hr/mg protein

4. Date of test:

		2	0		
Month	Day	Year			

For 100-day follow-up reports, only questions 1–5 are required. Please sign below and submit only page 1 of this form. For all visits beyond 100 days post-HSCT, also continue with question 6.

5. Signed: (person completing form) _____

Please print name: _____

Phone number: (_____) _____

Fax number: (_____) _____

E-mail address: _____

CIBMTR Form 2138 (MUC) v1.0 (1–6) July 2007
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Mail this form to your designated campus (Milwaukee or Minneapolis). Retain the original at the transplant center.

Fax this form to your designated campus (Milwaukee 414-456-6165 or Minneapolis 612-627-5895).

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6. Was any treatment given for the disease since the date of the last report?

- 1 yes
2 no
3 unknown

Specify disease treatment given since the date of the last report:

7. 1 yes 2 no enzyme replacement
8. 1 yes 2 no gene transfer / gene therapy
9. 1 yes 2 no substrate deprivation / inhibitor
10. 1 yes 2 no other

11. If yes, specify:

Clinical Status Post-HSCT

12. Was cerebrospinal fluid (CSF) testing performed since the date of the last report?

- 1 yes
2 no
3 unknown

Specify the results of most recent tests:

13. Date of most recent test:

- 1 known
2 not known
- / /
Month Day Year

14. Opening pressure:

- 1 known
2 not known
- cm H₂O

15. Closing pressure:

- 1 known
2 not known
- cm H₂O

16. Total protein:

- 1 known
2 not known
- . 1 mg/dL
2 g/L

17. Serum albumin:

- 1 known
2 not known
- . 1 mg/dL
2 g/L

18. Serum IgG:

- 1 known
2 not known
- . 1 mg/dL
2 g/L

19. Was Magnetic Resonance Imaging (MRI) of the brain and/or spine performed since the date of the last report?

- 1 yes
2 no
3 unknown

20. Date of most recent MRI:

- 1 known
2 not known
- / /
Month Day Year

Specify the location of any abnormalities detected on MRI:

21. 1 yes 2 no 3 unknown hydrocephalus
22. 1 yes 2 no 3 unknown odontoid hypoplasia
23. 1 yes 2 no 3 unknown other

24. Specify:

25. Is a copy of the MRI report attached?

- 1 yes
2 no

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26. Was a Mental Development test performed since the date of the last report?

- 1 yes →
- 2 no
- 3 unknown

27. Date of most recent test:

- 1 known →

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2	0		
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- 2 not known

28. Specify the test instrument used:

- 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 test →

29. Specify: _____

30. Full scale score (not percentile):

- 1 known →

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- 2 not known

31. Verbal score (not percentile):

- 1 known →

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- 2 not known

32. Performance score (not percentile):

- 1 known →

--	--	--	--
- 2 not known

33. Were the Vineland Adaptive Behavior Scales performed since the date of the last report?

- 1 yes →
- 2 no
- 3 unknown

34. Date of most recent test:

- 1 known →

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2	0		
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- 2 not known

35. Communication skills score:

- 1 known →

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- 2 not known

36. Daily Living skills score:

- 1 known →

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- 2 not known

37. Socialization skills score:

- 1 known →

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- 2 not known

38. Was the recipient's visual acuity tested since the date of the last report?

- 1 yes →
- 2 no
- 3 unknown

39. Date of most recent visual acuity test:

- 1 known →

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2	0		
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- 2 not known

40. Visual acuity (uncorrected) of right eye (OD):

- 1 known →

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- 2 not known

41. Visual acuity (uncorrected) of left eye (OS):

- 1 known →

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- 2 not known

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42. Visual acuity (uncorrected) of both eyes (OU):

1 known →

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2 not known

43. Was corneal clouding present?

1 yes
2 no

44. Did the recipient undergo an ophthalmologic exam under anesthesia since the date of the last report?

1 yes →
2 no
3 unknown

45. Date of most recent ophthalmologic exam:

1 known →

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2	0				
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Month Day Year

2 not known

46. Specify results:

1 normal
2 abnormal / impaired

47. Is a copy of the report attached?

1 yes
2 no

48. Was an audiologic evaluation (auditory brain stem or conditioned response) performed since the date of the last report?

1 yes →
2 no
3 unknown

49. Date of most recent audiologic evaluation:

1 known →

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2	0				
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Month Day Year

2 not known

Specify tympanometry results:

50. 1 normal 2 retracted 3 flat Right ear
51. 1 normal 2 retracted 3 flat Left ear

52. Was the hearing loss (HL) in decibels (dB) assessed at the speech threshold for 500 hertz (HZ) since the date of the last report?

1 yes →
2 no
3 unknown

53. Date of most recent evaluation:

1 known →

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2	0				
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Month Day Year

2 not known

Specify tympanometry results: (See Degree of Hearing Loss chart below for scale ranges.)

54. 1 normal / mild 2 moderate / moderately severe 3 severe / profound Right ear
55. 1 normal / mild 2 moderate / moderately severe 3 severe / profound Left ear

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

1 yes →
2 no
3 unknown

57. Date of most recent evaluation:

1 known →

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2	0				
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Month Day Year

2 not known

Specify tympanometry results: (See Degree of Hearing Loss chart below for scale ranges.)

58. 1 normal / mild 2 moderate / moderately severe 3 severe / profound Right ear
59. 1 normal / mild 2 moderate / moderately severe 3 severe / profound Left ear

Degree of Hearing Loss: Pure Tones and Speech Testing

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

