# ERROR CORRECTION FORM

Sequence Number: | | | | | | | | | | | | | | |

CIBMTR Recipient ID: | | | | | | | | | | |

Initials: | |

Today's Date: Month | | Day | | Year 2 0 | |

Infusion Date: Month | | Day | | Year 2 0 | |

CIBMTR Center Number: | | | | | |

CIBMTR®
CENTER FOR INTERNATIONAL BLOOD & MARROW TRANSPLANT RESEARCH

## Mucopolysaccharidosis and Other Storage Diseases Pre-HSCT Data

**Registry Use Only**

Sequence Number:

Date Received:

CIBMTR Center Number: | | | | | |

CIBMTR Recipient ID: | | | | | | | | | | |

Today's Date: Month | | Day | | Year 2 0 | |

Date of HSCT for which this form is being completed: 🕮 Month | | Day | | Year 2 0 | |

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

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

**This form must be accompanied by Form 2000 – Recipient Baseline Data. All information in the box above, including the date, should be identical with the corresponding Form 2000. Information should come from an actual examination by the Transplant Center physician, or the physician who is following the recipient pre-HSCT, or abstraction of the recipient's medical records.**

**If this is a report of a second or subsequent transplant, check here ☐ and continue with question 11.**

1. What was the date of diagnosis of Mucopolysaccharidosis / Other Storage Disease? Month | | Day | | Year | |
2. Did the diagnosis include a Sanfilippo-type enzyme deficiency?
   1 ☐ yes →
   2 ☐ no
   3. Specify enzyme:
      1 ☐ heparan N-sulfatase (Sanfilippo A – MPS IIIA)
      2 ☐ α-N-acetylglucosaminidase (Sanfilippo B – MPS IIIB)
      3 ☐ acetyl-Coα-glucosaminide acetyltransferase (Sanfilippo C – MPS IIIC)
      4 ☐ N-acetylglucosamine 6-sulfatase (Sanfilippo D – MPS IIID)
4. Did the diagnosis include a Morquio-type enzyme deficiency?
   1 ☐ yes →
   2 ☐ no
   5. Specify enzyme:
      1 ☐ N-acetyl-galactosamine-6-sulfatase (Morquio A – MPS IVA)
      2 ☐ β-galactosidase (Morquio B – MPS IVB)
6. Was the diagnosis neuronal ceroid lipofuscinosis (Batten disease)?
   1 ☐ yes →
   2 ☐ no
   7. Specify the disease subtype:
      1 ☐ neuronal ceroid lipofuscinosis enzyme — NCL 1 (infantile): PPT-palmitoyl protein thioesterase
      2 ☐ neuronal ceroid lipofuscinosis enzyme — NCL 2 (classic late infantile): transpeptidase

Specify the leukocyte enzyme activity levels at diagnosis:

8. Date enzyme levels tested: Month | | Day | | Year | |
9. Patient enzyme level: | | | . | | 1 ☐ nmol/hr/mg protein 2 ☐ pmol/hr/mg protein
10. Donor enzyme level: | | | . | | 1 ☐ nmol/hr/mg protein 2 ☐ pmol/hr/mg protein 3 ☐ unknown

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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 414-805-0714 or Minneapolis 612-627-5895).**

# ERROR CORRECTION FORM

Sequence Number: ☐☐☐☐☐☐☐☐☐☐☐☐☐☐☐

CIBMTR Recipient ID: ☐☐☐☐☐☐☐☐☐☐

Initials: ☐

Today's Date: ☐☐ Month ☐☐ Day 2 0 ☐☐ Year

Infusion Date: ☐☐ Month ☐☐ Day 2 0 ☐☐ Year

CIBMTR Center Number: ☐☐☐☐☐

CIBMTR Center Number: ☐☐☐☐☐ CIBMTR Recipient ID: ☐☐☐☐☐☐☐☐☐☐

11. Was treatment given for the disease between diagnosis and any time prior to the HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

Specify treatment(s):

12. 1 ☐ yes 2 ☐ no 3 ☐ unknown Enzyme replacement
13. 1 ☐ yes 2 ☐ no 3 ☐ unknown Gene transfer / gene therapy
14. 1 ☐ yes 2 ☐ no 3 ☐ unknown Substrate deprivation / inhibitor
15. 1 ☐ yes 2 ☐ no 3 ☐ unknown Other treatment → 16. If yes, specify: _____

## Clinical Status Prior to HSCT

17. Was cerebrospinal fluid (CSF) testing performed prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

18. Date of most recent test prior to HSCT: ☐☐ Month ☐☐ Day ☐☐☐☐ Year ☐ unknown

Report the results of the most recent test:

19. Opening pressure
    1 ☐ known → ☐☐☐ cm $H_2O$
    2 ☐ unknown
20. Total protein
    1 ☐ known → ☐☐☐.☐ 1 ☐ mg/dL 2 ☐ g/L
    2 ☐ unknown
21. Serum albumin
    1 ☐ known → ☐☐.☐ 1 ☐ mg/dL 2 ☐ g/L
    2 ☐ unknown
22. Serum IgG
    1 ☐ known → ☐☐.☐ 1 ☐ mg/dL 2 ☐ g/L
    2 ☐ unknown

23. Was magnetic resonance imaging (MRI) of the brain / spine performed prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

24. Date of most recent MRI prior to HSCT: ☐☐ Month ☐☐ Day ☐☐☐☐ Year ☐ unknown

Specify the location(s) of the abnormalities:

25. 1 ☐ yes 2 ☐ no 3 ☐ unknown Odontoid hypoplasia
26. 1 ☐ yes 2 ☐ no 3 ☐ unknown Ventricular (hydrocephalus)
27. 1 ☐ yes 2 ☐ no 3 ☐ unknown Other abnormality → 28. If yes, specify: _____
29. Is a copy of the MRI report attached?
    1 ☐ yes
    2 ☐ no

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CIBMTR Recipient ID: ☐☐☐☐☐☐☐☐☐☐

Initials: ☐

Today's Date: ☐☐ Month ☐☐ Day 2 0 ☐☐ Year

Infusion Date: ☐☐ Month ☐☐ Day 2 0 ☐☐ Year

CIBMTR Center Number: ☐☐☐☐☐

CIBMTR Center Number: ☐☐☐☐☐ CIBMTR Recipient ID: ☐☐☐☐☐☐☐☐☐☐

30. Was mental development testing performed prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

31. Date of most recent test prior to HSCT: ☐☐ Month ☐☐ Day ☐☐☐☐ Year ☐ unknown

32. Specify the test instrument used:
    1 ☐ Bayley Scales of Infant Development
    2 ☐ Stanford Binet Intelligence Scale
    3 ☐ Wechsler Preschool and Primary Scale of Intelligence (WPPSI – Revised)
    4 ☐ Wechsler Intelligence Scale for Children – III (WISC – III)
    5 ☐ other test →

33. Specify other test instrument: _____

34. Full scale score: *(not percentile)* ☐☐☐ ☐ score unknown

35. Verbal score: *(not percentile)* ☐☐☐ ☐ score unknown

36. Performance score: *(not percentile)* ☐☐☐ ☐ score unknown

37. Were the Vineland Adaptive Behavior Scales performed at any time prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

38. Date of most recent test prior to HSCT: ☐☐ Month ☐☐ Day ☐☐☐☐ Year ☐ unknown:

39. Communication skills: ☐☐☐ ☐ score unknown

40. Daily living skills: ☐☐☐ ☐ score unknown

41. Socialization skills: ☐☐☐ ☐ score unknown

42. Was an eye exam performed at any time prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

43. Date of most recent exam prior to HSCT: ☐☐ Month ☐☐ Day ☐☐☐☐ Year ☐ unknown

Visual acuity (uncorrected vision only):

44. Right eye (OD): ☐☐ / ☐☐☐ ☐ unknown

45. Left eye (OS): ☐☐ / ☐☐☐ ☐ unknown

46. Binocular / both eyes (OU): ☐☐ / ☐☐☐ ☐ unknown

47. Was corneal clouding present?
    1 ☐ yes
    2 ☐ no
    3 ☐ unknown

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Today's Date: Month Day 2 0 Year

Infusion Date: Month Day 2 0 Year

CIBMTR Center Number:

CIBMTR Center Number: CIBMTR Recipient ID:

48. Was an ophthalmologic exam performed under anesthesia at any time prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

49. Date of most recent exam prior to HSCT: Month Day Year ☐ unknown

50. Specify exam results:
    1 ☐ normal
    2 ☐ abnormal / impaired
    3 ☐ unknown

51. Is a copy of the report attached?
    1 ☐ yes
    2 ☐ no

52. Was a hearing test performed at any time prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

53. Date of most recent test prior to HSCT: Month Day Year ☐ unknown

54. Specify test results:
    1 ☐ normal
    2 ☐ abnormal / impaired
    3 ☐ unknown

55. Is a copy of the report attached?
    1 ☐ yes
    2 ☐ no

56. Was pulmonary function evaluated at any time prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

57. Date of most recent test prior to HSCT: Month Day Year ☐ unknown

58. Oxygen saturation on room air: % ☐ unknown

59. Specify results of pulmonary evaluation:
    1 ☐ normal
    2 ☐ abnormal / impaired
    3 ☐ unknown

60. Is a copy of the report attached?
    1 ☐ yes
    2 ☐ no

61. Was an echocardiogram performed at any time prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

62. Date of most recent test prior to HSCT: Month Day Year ☐ unknown

Specify valvular insufficiency:

63. Aortic
    1 ☐ none
    2 ☐ mild or trivial
    3 ☐ moderate or severe
    4 ☐ valve replacement
    5 ☐ unknown

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CIBMTR Center Number:

CIBMTR Center Number: CIBMTR Recipient ID:

64. Mitral
    1 ☐ none
    2 ☐ mild or trivial
    3 ☐ moderate or severe
    4 ☐ valve replacement
    5 ☐ unknown

65. Pulmonary
    1 ☐ none
    2 ☐ mild or trivial
    3 ☐ moderate or severe
    4 ☐ valve replacement
    5 ☐ unknown

66. Tricuspid
    1 ☐ none
    2 ☐ mild or trivial
    3 ☐ moderate or severe
    4 ☐ valve replacement
    5 ☐ unknown

67. Was a cardiac contractility test performed at any time prior to HSCT?
    1 ☐ yes →
    2 ☐ no
    3 ☐ unknown

68. Date of most recent test prior to HSCT: Month Day Year ☐ unknown

69. Ejection fraction: % ☐ unknown

70. Shortening fraction: % ☐ unknown

71. Signed: _____
*Person completing form*

Please print name: _____

Phone: (_____) _____

Fax: (_____) _____

E-mail address: _____

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