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? 
   - Yes:  
   - No:  

3. Specify enzyme: 
   - hepanan N-sulfatase (Sanfilippo A – MPS IIIA) 
   - α-N-acetylgalactosaminidase (Sanfilippo B – MPS IIIB) 
   - acetyl-CoA-glucosamine acetyltransferase (Sanfilippo C – MPS IIIC) 
   - N-acetylglucosamine 6-sulfatase (Sanfilippo D – MPS IIID) 

4. Did the diagnosis include a Morquio-type enzyme deficiency? 
   - Yes:  
   - No:  

5. Specify enzyme: 
   - N-acetyl-galactosamine-6-sulfatase (Morquio A – MPS IVA) 
   - β-galactosidase (Morquio B – MPS IVB) 

6. Was the diagnosis neuronal ceroid lipofuscinosis (Batten disease)? 
   - Yes:  
   - No:  

7. Specify the disease subtype: 
   - neuronal ceroid lipofuscinosis enzyme — NCL 1 (infantile): PPT-palmitoyl protein thioesterase 
   - 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

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.

CIBMTR Form 2038 (MUC) v1.0 (1–5) July 2007
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Fax this form to your designated campus (Milwaukee 414-805-0714 or Minneapolis 612-627-5895).
<table>
<thead>
<tr>
<th>Question</th>
<th>Options</th>
<th>Notes</th>
</tr>
</thead>
<tbody>
<tr>
<td>16. If yes, specify:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>17. Was cerebrospinal fluid (CSF) testing performed prior to HSCT?</td>
<td>1. yes 2. no 3. unknown</td>
<td>Report the results of the most recent test: 18. Date of most recent test prior to HSCT: Month Day Year 19. Opening pressure: 1. known 2. unknown cm H₂O</td>
</tr>
<tr>
<td>23. Was magnetic resonance imaging (MRI) of the brain / spine performed prior to HSCT?</td>
<td>1. yes 2. no 3. unknown</td>
<td>Specify the location(s) of the abnormalities: 24. Date of most recent MRI prior to HSCT: Month Day Year 25. Odontoid hypoplasia 26. Ventricular (hydrocephalus) 27. Other abnormality</td>
</tr>
<tr>
<td>28. If yes, specify:</td>
<td></td>
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<tr>
<td>29. Is a copy of the MRI report attached?</td>
<td>1. yes 2. no</td>
<td></td>
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<tr>
<td>Question</td>
<td>1</td>
<td>2</td>
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<td>-------------------------------------------------------------------------</td>
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<tr>
<td>30. Was mental development testing performed prior to HSCT?</td>
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<tr>
<td>31. Date of most recent test prior to HSCT:</td>
<td></td>
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<tr>
<td>32. Specify the test instrument used:</td>
<td></td>
<td></td>
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<tr>
<td>1. Bayley Scales of Infant Development</td>
<td></td>
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<tr>
<td>2. Stanford Binet Intelligence Scale</td>
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<tr>
<td>3. Wechsler Preschool and Primary Scale of Intelligence (WPPSI – Revised)</td>
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<tr>
<td>4. Wechsler Intelligence Scale for Children – III (WISC – III)</td>
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<tr>
<td>5. other test</td>
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<tr>
<td>33. Specify other test instrument:</td>
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<tr>
<td>34. Full scale score: (not percentile)</td>
<td></td>
<td></td>
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<tr>
<td>35. Verbal score: (not percentile)</td>
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<tr>
<td>36. Performance score: (not percentile)</td>
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<tr>
<td>37. Were the Vineland Adaptive Behavior Scales performed at any time prior to HSCT?</td>
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<td>38. Date of most recent test prior to HSCT:</td>
<td></td>
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<td>39. Communication skills:</td>
<td></td>
<td></td>
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<td>40. Daily living skills:</td>
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<td>41. Socialization skills:</td>
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<tr>
<td>42. Was an eye exam performed at any time prior to HSCT?</td>
<td></td>
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<tr>
<td>43. Date of most recent exam prior to HSCT:</td>
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<tr>
<td>Visual acuity (uncorrected vision only):</td>
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<td>44. Right eye (OD):</td>
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<td>45. Left eye (OS):</td>
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<td>46. Binocular / both eyes (OU):</td>
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<tr>
<td>47. Was corneal clouding present?</td>
<td></td>
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<tr>
<td>1. yes</td>
<td></td>
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<td>2. no</td>
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<tr>
<td>3. unknown</td>
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</tbody>
</table>
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
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: _______________________________________

Fax this form to your designated campus (Milwaukee 414-805-0714 or Minneapolis 612-627-5895).