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| Mucopo | olysaccharidosis and | Today's Date: 2 0 | | |
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| Sequence Number: | | HSCT type: autologous allogeneic, unrelated | • | al twin) |
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| 3 □ he 4 □ α-l | ıronate sulfatase (Hunter syndrome – Mi | | Saucher disease) | |
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| C · 6 □ N- IIII 7 □ N- IV/ 8 □ β-(9 □ N- Syl 10 □ R) Sy | N-acetylglucosaminidase (Sanfilippo B – etyl-Coα-glucosaminide acetyltransferas – MPS IIIC) acetylglucosamine 6-sulfatase (Sanfilippo D) acetylglucosamine 6-sulfatase (Morqui A) galactosidase (Morquio B – MPS IVB) acetylgalactosamine 4-sulfatase (Marote ndrome – MPS VI) glucuronidase (Sly syndrome – MPS VII) y the recipient's enzyme activity level: | A) 13 □ N-acetylglucosamine- (mucolipidosis II or I-c (Sanfilippo) 14 □ acid lipase deficiency 15 □ α-fucosidase deficiency (infantile): PPT-palmit 17 □ neuronal ceroid lipofu (classic late infantile): 18 □ α-mannosidase B defi 19 □ N-aspartyl-β-glucosam 20 □ hypoxanthine-guanine deficiency (Lesch-Nyt 21 □ other storage disease | e (Niemann-Pick disease ell) (Wolman disease) by (fucosidosis) scinosis enzyme — NCL byl protein thioesterase scinosis enzyme — NCL transpeptidase ciency (α-mannosidosis) hinidase (aspartylglucosal phosphoribosyltransfera an syndrome) Specify: | 1 2 minuria) se |
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| C · 6 □ N- IIII 7 □ N- IV 8 □ β-(9 □ N- Syl 10 □ β-(3. Specify 4. Date o For 100-d For all vis 5. Signed | N-acetylglucosaminidase (Sanfilippo B – etyl-Coα-glucosaminide acetyltransferas – MPS IIIC) acetylglucosamine 6-sulfatase (Sanfilippo D) acetylglucosamine 6-sulfatase (Sanfilippo D) acetylgalactosamine 6-sulfatase (Morqui A) galactosidase (Morquio B – MPS IVB) acetylgalactosamine 4-sulfatase (Marote ndrome – MPS VI) glucuronidase (Sly syndrome – MPS VII) y the recipient's enzyme activity level: If test: | A) 13 □ N-acetylglucosamine- (mucolipidosis II or I-o (sanfilippo) 14 □ acid lipase deficiency 15 □ α-fucosidase deficiency 16 □ neuronal ceroid lipofu (infantile): PPT-palmit 17 □ neuronal ceroid lipofu (classic late infantile): 18 □ α-mannosidase B defi 19 □ N-aspartyl-β-glucosam 20 □ hypoxanthine-guanine deficiency (Lesch-Nyh 21 □ other storage disease → 2. 1 □ nmol/hr/mg protein 2 □ pmol/hr/mg protein 2 □ pmol/hr/mg protein -5 are required. Please sign below and subicontinue with question 6. | e (Niemann-Pick disease ell) (Wolman disease) cy (fucosidosis) scinosis enzyme — NCL cyl protein thioesterase scinosis enzyme — NCL transpeptidase ciency (α-mannosidosis) ninidase (aspartylglucosar phosphoribosyltransfera an syndrome) Specify: | 1 2 minuria) se |
| C - 6 N- | N-acetylglucosaminidase (Sanfilippo B – etyl-Coα-glucosaminidase (Sanfilippo B – etyl-Coα-glucosaminide acetyltransferas – MPS IIIC) acetylglucosamine 6-sulfatase (Sanfilippo D) acetylgalactosamine 6-sulfatase (Morqui AA) galactosidase (Morquio B – MPS IVB) acetylgalactosamine 4-sulfatase (Marote ndrome – MPS VI) glucuronidase (Sly syndrome – MPS VII) y the recipient's enzyme activity level: f test: | A) 13 □ N-acetylglucosamine- (mucolipidosis II or I-o (sanfilippo) 14 □ acid lipase deficiency 15 □ α-fucosidase deficiency 16 □ neuronal ceroid lipofu (infantile): PPT-palmit 17 □ neuronal ceroid lipofu (classic late infantile): 18 □ α-mannosidase B defi 19 □ N-aspartyl-β-glucosam 20 □ hypoxanthine-guanine deficiency (Lesch-Nyh 21 □ other storage disease → 2. 1 □ nmol/hr/mg protein 2 □ pmol/hr/mg protein 2 □ pmol/hr/mg protein -5 are required. Please sign below and subicontinue with question 6. | e (Niemann-Pick disease ell) (Wolman disease) cy (fucosidosis) scinosis enzyme — NCL cyl protein thioesterase scinosis enzyme — NCL transpeptidase ciency (α-mannosidosis) ninidase (aspartylglucosa) phosphoribosyltransfera an syndrome) Specify: | 1 2 minuria) se |
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| Today's Date: | Infusion Date: CIBMTR Center Number: | Initials: |
| Month Day Year | Month Day Year | |
| CIBMTR Center Number: | CIBMTR Recipient ID: | |
| 6. Was any treatment give | en for the disease since the date of the last report? | |
| 1 □ yes | Specify disease treatment given since the date fo 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: | |
| | 3.074.03 | |
| Clinical Status Post- | | |
| <u> </u> | d (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 | |
| | 2 □ not known 2 □ g/L | |
| 19. Was Magnetic Resona 1 □ yes ——————————————————————————————————— | 20. Date of most recent MRI: 1 | |

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| Sequence Number: | CIBMTR Recipient ID: |
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| Today's Date: | Infusion Date: CIBMTR Center Number: Initials: |
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| Month Day Year | Month Day Year |
| CIBMTR Center Number: | CIBMTR Recipient ID: |
| 26. Was a Mental Developn | nent test performed since the date of the last report? |
| 1 □ yes → 2 □ no 3 □ unknown | 27. Date of most recent test: 1 |
| | 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 |
| | 2 ☐ not known 32. Performance score (not percentile): 1 ☐ known → |
| | otive Behavior Scales performed since the date of the last report? |
| 1 □ yes | 34. Date of most recent test: 1 □ known → □ □ □ □ □ □ □ □ □ □ □ □ □ □ □ □ □ □ |
| 3 ☐ unknown | 1 ☐ known 2 ☐ not known Month Day Year |
| | 35. Communication skills score: |
| | 1 ☐ known — ➤ ☐ ☐ ☐ 2 ☐ not known |
| | 36. Daily Living skills score: |
| | 1 ☐ known — ➤ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ |
| | 37. Socialization skills score: |
| | 1 ☐ known — ➤ 2 ☐ not known |
| 38 Was the recipient's visu | al acuity tested since the date of the last report? |
| 1 □ yes ———— | 39. Date of most recent visual acuity test: |
| 2 □ no 3 □ unknown | 1 known |
| | 40. Visual acuity (uncorrected) of right eye (OD): 1 □ known → |
| | 41. Visual acuity (uncorrected) of left eye (OS): 1 □ known |
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| FRF | ROR CORRECTION FORM Visit: |
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| Month Day Year | Month Day Year |
| CIBMTR Center Number: | CIBMTR Recipient ID: |
| | 42. Visual acuity (uncorrected) of both eyes (OU): 1 known |
| | 43. Was corneal clouding present? 1 ☐ yes 2 ☐ no |
| 44. Did the recipient underg | o an opthalmologic exam under anesthesia since the date of the last report? |
| 1 □ yes | 45. Date of most recent opthalmologic exam: |
| 3 ☐ unknown | 1 known |
| | Month Day Year 46. Specify results: 1 □ normal 2 □ abnormal / impaired 47. Is a copy of the report attached? 1 □ yes 2 □ no |
| 40.14 | |
| 46. was an audiologic evalu 1 □ yes ——————————————————————————————————— | uation (auditory brain stem or conditioned response) performed since the date of the last report? 49. Date of most recent audiologic evaluation: 1 □ known ——————————————————————————————————— |
| 3 LI UIIKIIOWII | 1 Ll 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 (H | IL) 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 |
| | 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 |
| | IL) 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 |
| | 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 |
| | 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 |

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Moderately Severe: 60-70 dB HL

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| CIBMTR Center Number: | CIBMTR Recipient ID: | |
| | ge in the recipient's neurologic status since the date of the last report? not neuropsychological status.) | |
| 1 □ yes | 61. Date of most recent evaluation: | |
| unchanged 3 □ unknown | 1 | |
| | 62. Specify current neurologic status compared to previous report: 1 □ improved 2 □ worsened | |
| | 63. Is a copy of the physical exam or neurologic exam attached? 1 □ yes 2 □ no | |
| 64. Was a pulmonary evalu | uation performed since the date of the last report? | |
| 1 □ yes — | 65. Date of most recent pulmonary evaluation: | |
| 2 □ no 3 □ unknown | 1 known | |
| | 66. Specify oxygen saturation on room air: % | |
| | 67. Specify the results of the most recent pulmonary function test: 1 □ normal 2 □ abnormal / impaired | |
| | 68. Is a copy of the pulmonary evaluation report attached? 1 □ yes 2 □ no | |
| 60. Was an achocardiograf | n performed since the date of the last report? | |
| 1 □ yes — ➤ | 70. Date of most recent echocardiogram: | |
| 2 □ no 3 □ unknown | 1 known Day Pear | |
| | Specify the findings for valvular insufficiency: 71. 1 none 2 mild / trivial 3 moderate / severe 4 valve replacement Aortic 72. 1 none 2 mild / trivial 3 moderate / severe 4 valve replacement Mitral 73. 1 none 2 mild / trivial 3 moderate / severe 4 valve replacement Pulmonary 74. 1 none 2 mild / trivial 3 moderate / severe 4 valve replacement Tricuspid | |
| 75. Was cardiac contractilit | y examined since the date of the last report? | |
| 1 □ yes | 76. Date of most recent exam: 1 known 2 not known Month Day Year | |
| | 77. Specify the method used to assess left ventricle performance: | |
| | 1 ☐ ejection fraction → 78. Specify fraction: % 2 ☐ shortening fraction → 78. | |

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| | 1 🗆 <u>1</u> 2 🗖 1 | yes | 80. Date of most recent dobutant | nine stress echocardiogra Day Day Year st recent test: | | |
| | 1 🗆 <u>1</u> 2 🗆 1 | yes | y performed since the date of the late of the late of most recent orthoper 1 known | dic surgery: Day 2 Vear | | |