Leukodystrophies Post-Infusion

Report most recent findings SINCE DATE OF LAST REPORT unless otherwise specified.

Leukodystrophies Post-Infusion Data

1. For which type of leukodystrophy was the infusion performed?
   - [ ] Krabbe Disease (globoid cell leukodystrophy)
   - [ ] Metachromatic leukodystrophy (MLD)
   - [ ] Adrenoleukodystrophy (ALD)
   - [ ] Hereditary diffuse leukoencephalopathy with spheroids (HDLS)
Enzyme activity and/or enzyme substrate

Recipient

2. Was enzyme activity and/or enzyme substrate tested?
   □ Yes – Go to question 3
   □ No – Go to question 6
   □ Unknown – Go to question 6

3. Date recipient tested: ___ ___ ___ ___ — ___ ___ — ___ ___
   YYYY MM DD

4. Recipient result
   □ Normal
   □ Abnormal

5. Was documentation submitted to the CIBMTR? (e.g., enzyme activity and/or enzyme substrate testing) (CIBMTR recommends attaching the enzyme activity and/or enzyme substrate testing)
   □ Yes
   □ No

Clinical Status Post-Infusion

6. Was the total neurologic function scale (NFS) score obtained? (ALD recipients only)
   □ Yes – Go to question 7
   □ No – Go to question 25

7. Specify date of NFS score: ___ ___ ___ ___ — ___ ___ — ___ ___
   YYYY MM DD

8. Specify total neurologic function scale score: ___ ___

9. Select known domain clinical score(s) (check all that apply)
   □ Hearing / auditory processing problems – Go to question 10
   □ Aphasia / apraxia – Go to question 11
   □ Loss of communication – Go to question 12
   □ Vision impairment / fields cut – Go to question 13
   □ Cortical blindness – Go to question 14
   □ Swallowing difficulty or other central nervous system dysfunction – Go to question 15
□ Tube feeding – Go to question 16
□ Running difficulties / hyperreflexia – Go to question 17
□ Walking difficulties / spasticity / spastic gait (no assistance) – Go to question 18
□ Spastic gait (needs assistance) – Go to question 19
□ Wheelchair required – Go to question 20
□ No voluntary movement – Go to question 21
□ Episodes of urinary or fecal incontinency – Go to question 22
□ Total urinary or fecal incontinency – Go to question 23
□ Nonfebrile seizures– Go to question 24

10. Hearing / auditory processing problems: ___ ___

11. Aphasia / apraxia: ___ ___

12. Loss of communication: ___ ___

13. Vision impairment / fields cut: ___ ___

14. Cortical blindness: ___ ___

15. Swallowing difficulty or other central nervous system dysfunction: ___ ___

16. Tube feeding: ___ ___

17. Running difficulties / hyperreflexia: ___ ___

18. Walking difficulties / spasticity / spastic gait (no assistance): ___ ___

19. Spastic gait (needs assistance): ___ ___

20. Wheelchair required: ___ ___

21. No voluntary movement: ___ ___

22. Episodes of urinary or fecal incontinency: ___ ___

23. Total urinary or fecal incontinency: ___ ___

24. Nonfebrile seizures: ___ ___

25. Did post-infusion seizures attributed to the underlying disease occur?
   □ Yes - Go to question 26
26. Were any of the seizures considered nonfebrile?
   □ Yes
   □ No
   □ Unknown

27. Was cerebrospinal fluid (CSF) testing performed?
   □ Yes - Go to question 28
   □ No - Go to question 33
   □ Unknown - Go to question 33

28. Date of most recent CSF testing
   □ Known - Go to question 29
   □ Unknown - Go to question 30

29. Date of most recent CSF testing: ___ ___ ___ ___ — ___ ___ — ___ ___
    YYYY                        MM                  DD

30. Specify known CSF result(s) (check all that apply)
    □ Opening pressure – Go to question 31
    □ Total protein – Go to question 32

31. Opening pressure: _____ • ___ cm H₂O

32. Total protein: _____ • ___ □ mg/dL
   □ g/L

33. Was magnetic resonance imaging (MRI) performed?
    □ Yes - Go to question 34
    □ No - Go to question 41
    □ Unknown - Go to question 41

34. Date of most recent MRI
    □ Known - Go to question 35
    □ Unknown - Go to question 36

35. Date of most recent MRI: ___ ___ ___ ___ — ___ ___ — ___ ___
    YYYY                        MM                  DD
36. Specify MRI results
   □ Normal – Go to question 39
   □ Abnormal – Go to question 37

37. Was gadolinium contrast used for this assessment?
   □ Yes – Go to question 38
   □ No – Go to question 39

38. Was gadolinium enhancement reported?
   □ Yes
   □ No

39. Loes composite score: ___ ___ (ALD recipients only)

40. Was documentation submitted to the CIBMTR? (CIBMTR recommends attaching the MRI report)
   □ Yes
   □ No

41. Were nerve conduction velocities tested?
   □ Yes - Go to question 42
   □ No - Go to question 45
   □ Unknown - Go to question 45

42. Date of most recent nerve conduction velocities test: ___ ___ ___ ___ — ___ ___ — ___ ___
    YYYY MM DD

43. Specify results
   □ Normal
   □ Abnormal

44. Was documentation submitted to the CIBMTR? (CIBMTR recommends attaching the nerve conduction velocities tests)
   □ Yes
   □ No

45. Was a neurocognitive test performed?
   □ Yes - Go to question 46
   □ No - Go to question 48
   □ Unknown - Go to question 48
46. Date of most recent neurocognitive test: ________ — ________ — ________
   YYYY MM DD

47. Was documentation submitted to the CIBMTR? *(CIBMTR recommends attaching the neurocognitive testing report)*
   ☐ Yes
   ☐ No

48. Has there been a change in the recipient’s neurologic status? *(Report clinical status, not neuropsychological status.)*
   ☐ Yes - Go to question 49
   ☐ Stable / unchanged – Go to question 51
   ☐ Unknown – Go to question 51

49. Specify current neurologic status compared to previous report
   ☐ Improved
   ☐ Worsened

50. Was documentation submitted to the CIBMTR? *(CIBMTR recommends attaching the physical exam or neurologic exam)*
   ☐ Yes
   ☐ No

**Clinical Global Impression (CGI) (neurologic assessment)**

51. Specify global improvement *(select one)*
   ☐ 0 = Not assessed
   ☐ 1 = Very much improved
   ☐ 2 = Much improved
   ☐ 3 = Minimally improved
   ☐ 4 = No change
   ☐ 5 = Minimally worse
   ☐ 6 = Much worse
   ☐ 7 = Very much worse
   ☐ Unknown

52. Specify leukodystrophy-specific therapy given *(check all that apply)*
   ☐ N-acetyl-L-cysteine (NAC) – Go to First Name
   ☐ GTE:GTO oil (Lorenzo’s oil)– Go to First Name
   ☐ Other therapy – Go to question 53
None – Go to First Name

53. Specify other therapy: ______

First Name: _____________________________________________________________
Last Name: _____________________________________________________________
Email address: ___________________________________________________________
Date: ___________ ___________ ___________
        YYYY     MM     DD