



Leukodystrophies Post-Infusion

Registry Use Only

Sequence Number:

Date Received:

CIBMTR Center Number: _____

CIBMTR Research ID: _____

Event date: _____
 YYYY MM DD

Visit

- 100 day
- 6 months
- 1 year
- 2 years
- >2 years, Specify: _____

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: _____

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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
- Abnormal

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

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- 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 - **Also complete Neurocognitive Assessment Form 3503 - 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

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- 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 question 54**
- GTE:GTO oil (Lorenzo's oil) – **Go to question 54**
- Other therapy – **Go to question 53**
- None – **Go to question 54**

53. Specify other therapy: _____

Disease Modifying Therapies

54. Were disease modifying therapies given? (*excludes blood transfusions*)

- Yes – **Go to question 55**
- No - **Go to question 62**
- Unknown - **Go to question 62**

If there is more than one therapy given copy questions 55- 61 for each therapy.

55. Specify the disease modifying therapy (*check all that apply*)

- Leriglitazone – **Go to question 57**
- Other therapy – **Go to question 56**

56. Specify other therapy: _____

57. Was the date therapy started previously reported?

- Yes – **Go to question 60**
- No – **Go to question 58**

58. Date therapy started

- Known - **Go to question 59**

