

FOLLOW-UP INSERT XXXIV
Mucopolysaccharidosis and
Other Storage Diseases

FOR REGISTRY USE ONLY:
 I.D. **U** - [] [] [] [] - [] [] [] [] [] []
 Date received: [] [] [] [] [] [] [] []

TEAM: [] [] [] [] IUBMID: [] [] [] [] [] [] [] []
(Institutional Unique Blood or Marrow Transplant Identification Number)

Registry (circle one): **IBMTR** **ABMTR** **EBMT**

Date of transplant for which this form is being completed: [] [] [] [] [] [] [] []
 Month Day Year

Date of report: [] [] [] [] [] [] [] []
 Month Day Year

Follow-up Information*

*** Report data for date of last contact as reported in Q.3 of Follow-up Care Form or immediately prior to death**

Record the leukocyte enzyme activity level at current evaluation:

1. Date tested: [] [] [] [] [] [] [] []
 Month Day Year
2. Patient enzyme activity level: [] [] [] [] [] nmol/hr/mg protein μmol/hr/mg protein Unknown

3. Was treatment given for the disease since last report?

- 1 Yes
 0 No
 8 Unknown

Yes No Unknown

4. Enzyme replacement
 5. Substrate deprivation/inhibitor
 6. Gene transfer/Gene therapy
 7. Other, specify _____

Clinical status posttransplant

8. Was cerebrospinal fluid (CSF) testing done since last report?

- 1 Yes
 0 No
 8 Unknown

9. Date of most recent test: [] [] [] [] [] [] [] [] Unknown
 Month Day Year

Results of most recent tests:
Yes No Unknown

10. Opening pressure [] [] [] [] [] [] [] [] cm H₂O
 11. Closing pressure [] [] [] [] [] [] [] [] cm H₂O
 12. Total protein [] [] [] [] [] [] [] [] mg/dL g/L

3. Was Magnetic Resonance Imaging (MRI) of the brain/spine done since last report?

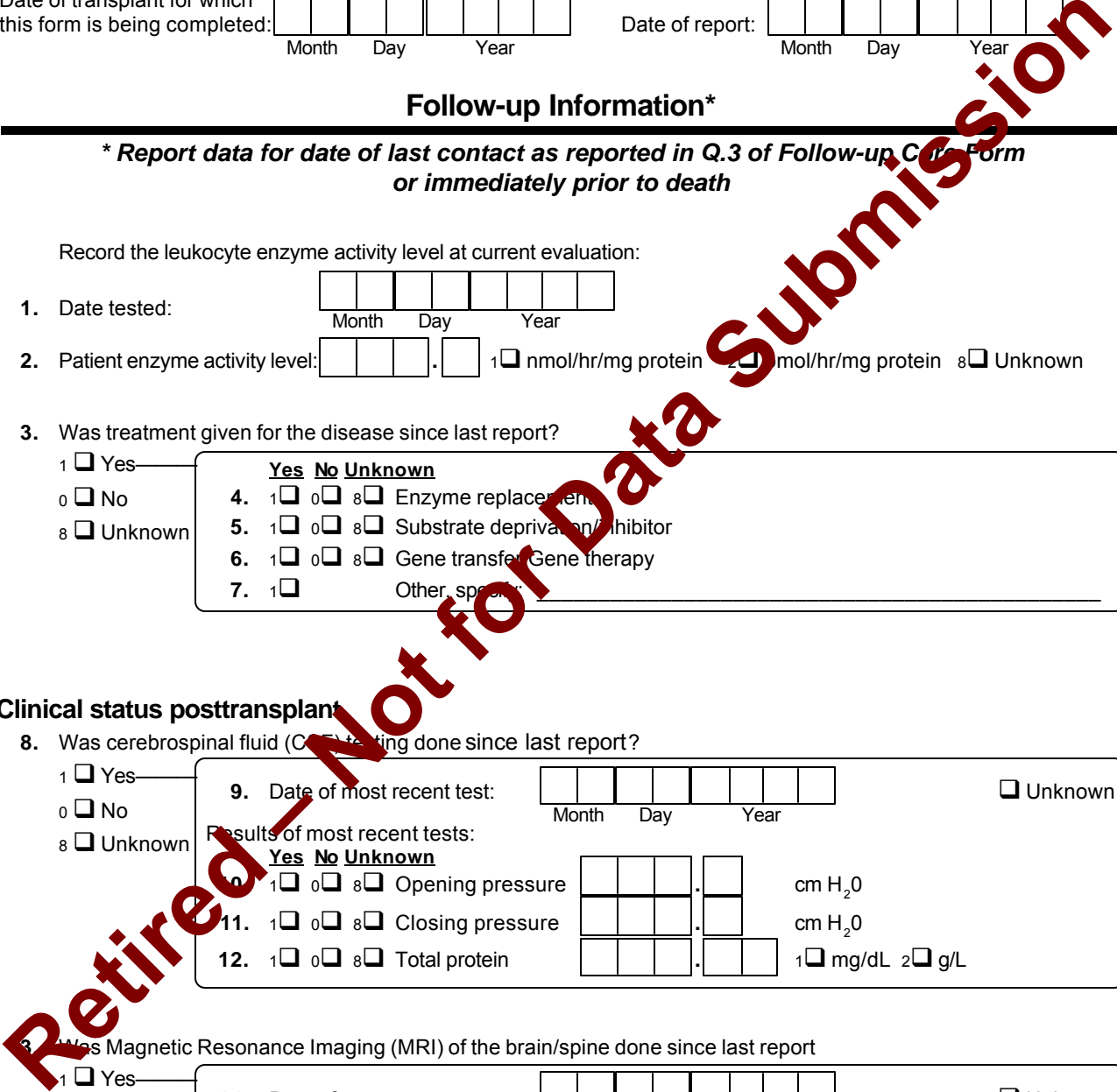
- 1 Yes
 0 No
 8 Unknown

14. Date of most recent test: [] [] [] [] [] [] [] [] Unknown
 Month Day Year

Location of abnormalities:
Yes No Unknown

15. Hydrocephalus
 16. Odontoid hypoplasia

If MRI Report is available, check here , attach copy and reference Q.13



TEAM:

IUBMID:

17. Was a Mental Development test done since last report?

- 1 Yes
- 0 No
- 8 Unknown

18. Date of most recent test:	<input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
	Month Day Year	
19. Indicate test instrument and standard score (<i>check only one</i>):		
1 <input type="checkbox"/> Bayley Scales of Infant Development		
2 <input type="checkbox"/> Stanford Binet Intelligence Scale 4th Edition		
3 <input type="checkbox"/> Wechsler Preschool and Primary Scale of Intelligence (WPPSI – Revised)		
4 <input type="checkbox"/> Wechsler Intelligence Scale for Children – III (WISC – III)		
7 <input type="checkbox"/> Other, specify: _____		
20. Full scale score (<i>not percentile</i>):	<input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
21. Verbal score (<i>not percentile</i>):	<input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
22. Performance score (<i>not percentile</i>):	<input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown

23. Were the Vineland Adaptive Behavior Scales done since last report?

- 1 Yes
- 0 No
- 8 Unknown

24. Date of most recent test:	<input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
	Month Day Year	
25. Communication skills:	<input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
26. Daily living skills:	<input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
27. Socialization skills:	<input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown

28. Was an eye exam done since last report?

- 1 Yes
- 0 No
- 8 Unknown

29. Date of most recent test:	<input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
	Month Day Year	
Visual acuity (uncorrected only):		
30. Right eye (OD):	<input type="text"/> <input type="text"/> / <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
31. Left eye (OS):	<input type="text"/> <input type="text"/> / <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
32. Binocular/both eyes (OU):	<input type="text"/> <input type="text"/> / <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
33. Was corneal clouding present?	1 <input type="checkbox"/> Yes 0 <input type="checkbox"/> No	8 <input type="checkbox"/> Unknown

34. Did the patient undergo an ophthalmologic exam under anesthesia since last report?

- 1 Yes
- 0 No
- 8 Unknown

35. Date of most recent test:	<input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/> <input type="text"/>	<input type="checkbox"/> Unknown
	Month Day Year	
36. Results of test (<i>check only one</i>):		
0 <input type="checkbox"/> Normal		
1 <input type="checkbox"/> Abnormal/Impaired		
8 <input type="checkbox"/> Unknown		

**If the report is available,
check here , attach copy and reference Q.34**

Retired - Not for Data Submission

TEAM:

IUBMID:

37. Was the patient's hearing tested since last report?

- 1 Yes
- 0 No
- 8 Unknown

38. Date of most recent test:

Month Day Year

Unknown

39. Results of hearing test (check only one):

- 0 Normal
- 1 Abnormal/Impaired
- 8 Unknown

*If the report is available,
check here , attach copy and reference Q.37*

40. Did neurologic status change since precondition evaluation?

- 1 Yes
- 0 Stable/Unchanged
- 8 Unknown

41. Date of most recent test:

Month Day Year

Unknown

Status:

42. 1 Improved
2 Worsened

*If Physical Examination or Neurologic
Examination Reports are available,
check here , attach copy and reference Q.40*

43. Was a pulmonary evaluation done since last report?

- 1 Yes
- 0 No
- 8 Unknown

44. Date of most recent test:

Month Day Year

Unknown

45. Oxygen saturation on room air:

%

Unknown

46. Results of pulmonary evaluation (check only one):

- 0 Normal
- 1 Abnormal/Impaired
- 8 Unknown

*If Pulmonary Function Report is available,
check here , attach copy and reference Q.43*

47. Was an echocardiogram done since last report?

- 1 Yes
- 0 No
- 8 Unknown

48. Date of most recent test:

Month Day Year

Unknown

Valvular insufficiency:

49. Tricuspid (check only one):

- 0 None
- 1 Mild or trivial
- 2 moderate or severe
- 3 valve replacement
- 8 Unknown

50. Mitral (check only one):

- 0 None
- 1 Mild or trivial
- 2 moderate or severe
- 3 valve replacement
- 8 Unknown

51. Aortic (check only one):

- 0 None
- 1 Mild or trivial
- 2 moderate or severe
- 3 valve replacement
- 8 Unknown

52. Pulmonary (check only one):

- 0 None
- 1 Mild or trivial
- 2 moderate or severe
- 3 valve replacement
- 8 Unknown

53. Was the cardiac contractility tested since last report?

- 1 Yes
- 0 No
- 8 Unknown

54. Date of most recent test:

Month Day Year

Unknown

55. Ejection fraction (EF): %

~ or ~

56. Shortening fraction (SF): %

Unknown

Unknown

Retired - Not for Data Submission

TEAM:

IUBMID:

57. Was orthopedic surgery performed since last report?

- 1 Yes
- 0 No
- 8 Unknown

58. Date of most recent orthopedic surgery:

<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>
Month	Day	Year			

Unknown

Specify site(s) (check all that apply):

Yes No Unknown

- 59. 1 0 8 Knees
- 60. 1 0 8 Hips
- 61. 1 0 8 Spine
- 62. 1 0 8 Fingers
- 63. 1 0 8 Wrist (carpal tunnel syndrome)
- 64. 1 0 8 Other, specify: _____

65. Was a stress dobutamine test performed since last report?

- 1 Yes
- 0 No
- 8 Unknown

66. Date of most recent test:

<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>	<input type="text"/>
Month	Day	Year			

Unknown

67. Results of test (check only one):

- 0 Normal
- 1 Abnormal/Impaired
- 8 Unknown

If Report is available, check here, attach copy and reference Q.65

Retired – Not for Data Submission