

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:	

CIBMTR Center Number: CIBMTR Research ID:				
Repo	ort most recent findings SINCE DATE OF LAST REPORT unless otherwise specified.			
Leuk	odystrophies Post-Infusion Data			
4				
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)			
Enzyn	ne activity and / or enzyme substrate			
Recip	ient			
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			
	Li Abrioffiai			
	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			
Clinic	cal 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			

CIBMTR Center Number:		lumber: CIBMTR Research ID:
8.	Spe	cify total neurologic function scale score:
9.	Sele	ect 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 – <i>Go to question 13</i>
		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) – <i>Go to question 18</i>
		Spastic gait (needs assistance) – Go to question 19
		Wheelchair required – Go to question 20
		No voluntary movement – <i>Go to question 21</i>
		Episodes of urinary or fecal incontinency – Go to question 22
☐ Total uri		Total urinary or fecal incontinency – <i>Go to question 23</i>
☐ Nonfebrile		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	Whoolebair required:

CIBMTR Center Number:		r Nu	mber: CIBMTR Research ID:	
		2	1.	No voluntary movement:
		2	2.	Episodes of urinary or fecal incontinency:
		2	23.	Total urinary or fecal incontinency:
		2	4.	Nonfebrile seizures:
25.	Dic	d post	-infu	sion seizures attributed to the underlying disease occur?
		Yes	- Go	to question 26
		No -	Go	to question 27
		Unkı	nowr	n - Go to question 27
	26	5. V	/ere	any of the seizures considered nonfebrile?
) Y	es
) N	0
			J	nknown
27	۱۸/۵	0.005	abra	oning fluid (CSE) testing performed?
27.	_			spinal fluid (CSF) testing performed? to question 28
				to question 33
		Uliki	IOWI	n - Go to question 33
	28	s. D	ate	of most recent CSF testing
			ı K	nown - Go to question 29
		Г	l L	nknown - Go to question 30
		2	9.	Date of most recent CSF testing:
				YYYY MM DD
	30). S	peci	fy known CSF result(s) <i>(check all that apply)</i>
				ppening pressure – Go to question 31
] T	otal protein – <i>Go to question</i> 32
		3	31.	Opening pressure: • cm H ₂ O
		3	2.	Total protein: • □ mg/dL
				□ g/L

CIBN	/ITR Center Number:	_ CIBMTR Researc	:h ID:		·	
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	5				
	☐ Unknown - Go to question	36				
	35. Date of most recent MRI: _					
		YYYY	ММ	DD		
	36. Specify MRI results					
	□ Normal					
	☐ Abnormal					•
	37. Was gadolinium contrast used for	r this assessment?				
	☐ Yes – Go to question 38					
	□ No – Go to question 39					
	38. Was gadolinium enhancen	nent reported?				
	□ Yes					
	□ No					
	20.					
	39. Loes composite score:(AL	D recipients only)				
	40. Was documentation submitted to	the CIBMTR? (CIBM7	TR recom	mends at	taching th	e 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	ction velocities test:				
			YYYY		MM	DD

CIBMTR Center	Number: CIBMTR Research ID:				
	Normal				
	Abnormal				
44. Was documentation submitted to the CIBMTR? (CIBMTR recommends attaching the nerve conductive velocities tests)					
	Yes				
	No				
45. Was a neu	urocognitive test performed?				
□ Yes -	Also complete Neurocognitive Assessment Form 3503 - Go to question 46				
□ No - (Go to question 48				
☐ Unkno	own - Go to question 48				
46. Da	te of most recent neurocognitive test:				
	YYYY MM DD				
	as documentation submitted to the CIBMTR? (CIBMTR recommends attaching the neurocognitive ting report)				
	Yes				
	No				
48. Has there status)	been a change in the recipient's neurologic status? (Report clinical status, not neuropsychological				
□ Yes -	Go to question 49				
☐ Stable	e / unchanged – <i>Go to question 51</i>				
□ Unkno	own – Go to question 51				
49. Sp	ecify current neurologic status compared to previous report				
10. 5	Improved				
	Worsened				
50. Wa	as documentation submitted to the CIBMTR? (CIBMTR recommends attaching the physical exam or				
	urologic exam)				
	Yes				
	No				
Clinical Global	mpression (CGI) (neurologic assessment)				
51. Specify glo	obal improvement (select one)				
□ 0 = Not	assessed				

CIBN	ITR Ce	nter Number: CIBMTR Research ID:
	□ 1=	= Very much improved
	□ 2=	= Much improved
	□ 3=	= Minimally improved
	□ 4=	= No change
	□ 5=	= Minimally worse
	□ 6=	= Much worse
	□ 7=	= Very much worse
	□ Ur	nknown
52.	Speci	fy leukodystrophy-specific therapy given (check all that apply)
	□ N-	acetyl-L-cysteine (NAC) – <i>Go to question 54</i>
	□ G1	TE:GTO oil (Lorenzo's oil) – <i>Go to question 54</i>
	□ Ot	her therapy – Go to question 53
	□ No	one – Go to question 54
	53.	Specify other therapy:
Dise	ase Mo	difying Therapies
E4 \	Noro di	sease modifying therapies given? (excludes blood transfusions)
54.		es – Go to question 55
		o - Go to question 62
		Inknown - Go to question 62
		Miniswii Co to question oz
	If the	re is more than one therapy given copy questions 55- 61 for each therapy.
	55.	Specify the disease modifying therapy (check all that apply)
		☐ Leriglitazone – <i>Go to question 57</i>
		☐ 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

CIBMTR Center Number:				CIBMTR Rese	earch ID:		
☐ Unknown			nknown – Go to question	60			
		59.	Date therapy started:				□ Date estimated
				YYYY	MM	DD	
	60.	Date	therapy stopped				
		□ Kr	nown – Go to question 61	1			
		□ Uı	nknown - Go to question	62			
		□ No	ot applicable (still receiving	g therapy) - Go	o to questio	n 62	
		61.	Date therapy stopped:	YYYY			□ Date estimated
Marro	ow Evaluation	n					
Com	plete quest	ion 62 fo	or gene therapy infusions	s only			
62.	Was a mar	row aspi	irate and / or biopsy perfor	rmed?			
	□ Yes - A	lso con	nplete Laboratory Studie	s Form 3502	and Marrow	/ Surveill	lance Form 3506
	□ No						
	□ Unknow	/n					