

Supplemental Table 1. Clinical results at most recent follow-up.

	Patient (current age in months)					
	1	2	3	4	5	6
Current age/age at most recent follow-up (in months)	58/53	57/52	52/44	43/45	36/35	30/24
Height, weight, head circumference (in percentiles)	< 1%, < 1%, 76%	< 1%, < 1%, 3%	67%, 97%, 87% (HC at 29mo)	10%, 40%, 50%	3%, 10%, 99%	1%, 2%
Method of Nutrition	By mouth; G-tube supplemental feeds	Primarily G-tube, purees by mouth	By mouth	By mouth	By mouth; supplemental G-tube	By mouth
Fine motor	Drinks from cup; Self feeds crackers (pincer); Stacks 5 blocks; Scribbles; Plays on tablet	Drinks from cup; Holds toys with both hands & puts into container/take out; Plays on tablet	Drinks from cup; Self feeds: crackers (pincer) & utensils; Scribbles, Plays on tablet	Drinks from cup; Self feeds crackers (pincer) & utensils; Scribbles; Plays on tablet	Drinks from cup; Self feeds crackers (pincer); Stacks 2 blocks; Scribbles; Turns pages;	Waving bye; Reaches & holds toys with both hands; self-feeds
Speech/Language	Follows 2-3 step directions, learning colors; 1-2-words phrases, signs, ACC;	Approximates words; follows commands; nods & gestures;	Speaking 2-3 word sentences; uses 75 signs	Uses signs & gestures; limited words. Strong receptive, ACC	3 verbal words plus several signs; follows commands;	Saying several words, pointing to body parts
Ambulation	Wheelchair; limited walking with gait-trainer;	Wheelchair;	Self -propel wheelchair; Ride tricycle; Sits unsupported	Limited walking with gait-trainer; Sits unsupported; Crawling	Self -propel wheelchair; Crawls, but not pull up to stand	Walking independently, climbing
Tone	Central hypotonia; UE & LE hypertonia	Central hypotonia; LE hypertonia	Mild central & UE hypotonia; LE hypertonia	Central hypotonia; LE hypertonia	Low tone in trunk, increased in legs	Increased tone LE, no clonus

Spasticity management	Baclofen, braces, PT	Baclofen, braces, PT, surgery*, botulinum toxin	Baclofen, PT, braces	Baclofen, botulinum toxin, braces, PT	Braces	PT
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GALC, galactocerebrosidase; G-tube, gastrostomy tube; AAC, augmentative and alternative communication; LE, lower extremities; UE, upper extremities; PT, physical therapy. *Patient underwent bilateral intertrochanteric ostomy and hip adductor lengthening.

Supplemental Table 2. Neurologic Testing Results Before and After Transplant.

Study	Time	Patient No.					
		1	2	3	4	5	6
Psychosine in nmol/L (normal)	Pre-HCT	24 *	61 *	56	73	38	35
	6 months	ND	21 ^a	8.6	11	22	ND
	1-year	12 ^a	17 ^a	12	19	13.2 *	3.6
	2-year	9.4	ND	10	15	15.5 *	47 pmol/g
	Most recent (age, months)	ND	ND	3.7 (41)	16.2 (32)	9.8 (34)	NA
MRI	Pre-HCT	Abnormal	Normal	Abnormal	Normal	Normal	Abnormal
	6 months	Abnormal	Normal	Abnormal	Abnormal	Abnormal	Abnormal
	1-year	Abnormal	Abnormal	Abnormal	Abnormal	Abnormal	Abnormal
	2-year	Abnormal	Abnormal	Abnormal	Abnormal	Abnormal	Abnormal but improved
	Most recent (age, months)	Abnormal, stable (53)	Abnormal, stable (27)	ND	Abnormal but improved (35)	Abnormal, stable (32)	ND
VEP	Pre-HCT	Abnormal	Abnormal	Normal	Normal	Normal	ND
	6 months	Normal	Abnormal	Normal	Normal	Normal	ND
	1-year	Mixed	Abnormal (Sedated)	Normal	Normal	Normal	ND
	2-year	Normal	ND	Normal	ND	ND	ND
	Most recent (age, months)	Normal (36)	ND	ND	Borderline (36) ⁺	Abnormal (32)	ND

EEG	Pre-HCT	Excessive ESTs	Normal	Excessive ESTs	Excessive ESTs	Normal	Excessive ESTs
	6 months	Sedated	Normal	Normal	ND	Normal	ND
	1-year	Normal	Sedated	Normal	ND	Normal	ND
	2-year	Slowing	Slowing	Normal	ND	Normal	ND
	Most recent (age, months)	Slowing (53)	ND	ND	ND	Normal (32)	ND
BAER^c	Pre-HCT	Abnormal	Abnormal	Abnormal	Abnormal	Abnormal	ND
	6 months	Abnormal	Normal	Abnormal	Abnormal	ND	ND
	1-year	Abnormal	Abnormal	Abnormal	ND	ND	ND
	2-year [±]	Abnormal	ND	Abnormal	Normal (36)	NA	NA
NCS	Pre-HCT	Abnormal	Abnormal	Abnormal	Abnormal	Abnormal	ND
	6 months	ND	Abnormal	Abnormal	ND	Abnormal	ND
	1-year	Abnormal	Abnormal	Abnormal	Abnormal	Abnormal	ND
	2-year	Abnormal	Abnormal	Abnormal	ND	ND	ND
	Most recent (age, months)	Abnormal (36)	ND	ND	ND	Abnormal (32)	ND

HCT, hematopoietic cell transplant; ND, not done; NA, not applicable; MRI, magnetic resonance imaging; VEP, visual evoked potentials; EEG, electroencephalogram; ESTs, excessive sharp transients BAER, brainstem evoked potential responses; NCS, nerve conduction studies; *Psychosine normal values <2 nmol/L measured on dried blood spot. All other measurements performed on whole blood samples (normal <10 nmol/L). [±]Normal latencies but reduced amplitudes noted; [±]BAERs were not performed beyond 2-years in this cohort.