

Supplementary Table 1: The Pre-Treatment Baseline Assessment for the Five Children with Fabry Disease

	Case 1	Case 2	Case 3	Case 4	Case 5	
Age at Assessment (years)	13.9	10.6	12.6	10.7	11.2	
Physical Examination	Weight (kg)	38.4	22.5	26	26.5	77
	Weight Percentile¹	P3-P10	<P3	<P3	P3-P10	>P97
	Height (cm)	155	130	142	136	163
	Height Percentile¹	P3-P10	<P3	P3-P10	P3-P10	>P97
	BMI (kg/m²)	15.9	13.3	12.9	14.3	29.0
Kidney	Urine Protein	Negative	Negative	Trace protein	Negative	Negative
	Urine Red Blood Cells (cells/HP)	Normal	Normal	Normal	Normal	Normal
	Blood Urea Nitrogen (mmol/L)	4.25	5.63	4.02	7.57	4.56
	Serum Creatinine (umol/L)	51.6	40.3	28	44	43.3
	Glomerular Filtration Rate (ml/min·1.73m²)	109.7	117.8	185.2	112.8	137.4
	Nutcracker Phenomenon (Left Renal Vein Entrapment)	Negative	Positive	Positive	Negative	Negative
	24-hour Urine Protein Quantification (mg)	48.0	62.0	49.0	64.0	38.0
Heart	Electrocardiograph	Accelerated atrial arrhythmia	Sinus rhythm	Sinus arrhythmia	Sinus rhythm	Sinus rhythm
	Ultrasonic Cardiogram	Normal upper limit of left atrial diameter; Aortic sinus diameter widened slightly	Normal	Normal	Normal	Normal

	Left Ventricular Mass Indexes (g/m²)²	43.6	48.9	42.5	50.9	49.1
Brain/Nervous System	Brain Magnetic Resonance Imaging	Pineal cyst	Punctate and linear high signals are seen in the sulci of bilateral parietal, occipital, and temporal lobes on FLAIR imaging, with prominent vascular markings; The walls of the left posterior cerebral artery and its branches show slight irregularity	Focal demyelination in the left frontal lobe	Normal	Normal
	Transcranial Doppler	Pulsatility Index: 0.53-0.67	Normal	Normal	Normal	Normal
	Electromyography	Normal	Normal	Normal	Normal	Normal
Lung	Pulmonary Function	Lung capacity: normal; Pulmonary ventilation function: normal	Lung capacity: normal; Pulmonary ventilation function: normal	NA	Lung capacity: reduced inspiratory capacity (IC); Pulmonary ventilation function: normal	Lung capacity: reduced inspiratory capacity (IC); Pulmonary ventilation function: reduced forced expiratory flow at 75% of vital capacity (FEF75)
Abdomen	Abdominal Ultrasound	Normal	Normal	Normal	Normal	Normal
Eyes	Fundus Examination	Normal	Normal	Normal	Normal	Normal
Ears	Pure Tone Test	Pass	Pass	Mild high-frequency bilateral hearing loss	Pass	Pass

Note: NA, not available. The patient experienced significant nausea and was unable to complete the examination.

Reference ranges: Blood urea nitrogen: 2.7-7 mmol/L; Serum creatinine: 27-66 µmol/L; 24-hour urine protein: <150 mg; Pulsatility Index: 0.7-1.1.

References:

¹Li H, Ji CY, Zong XN, et al. Height and weight standardized growth charts for Chinese children and adolescents aged 0 to 18 years. *Zhonghua Er Ke Za Zhi*. 2009;47(7):487-92. DOI: 10.3760/cma.j.issn.0578-1310.2009.07.003

²Díaz A, Zócalo Y, Bia D. Reference Intervals and Percentile Curves of Echocardiographic Left Ventricular Mass, Relative Wall Thickness and Ejection Fraction in Healthy Children and Adolescents. *Pediatr Cardiol*. 2019;40(2):283-301. doi:10.1007/s00246-018-2000-y.