

Table 2. Characteristics of the participants

Code RD-	Respondent (race)	Patient age (year)	Type of rare disease	Year of diagnosis/ treatment	Treatment	Funding	Clinic visit/ month	Distance from home
C1	Parent (M)	12	MPS II	2010/2015	Idursulfase (Elaprase)	Society (MLDA)	4	50–100 km
C2	Parent (M)	6	MPS I	2012/2014	Laronidase (Aldurazyme)		4	<50 km
C3	Parent (M)	6	MPS I	2011/2015	Laronidase (Aldurazyme)		4	<50 km
C4	Patient (M)	32	MERRF	2013/2013	Co–enzyme Q10 L– carnitine	MOH	Every 3 months	<50 km
C5	Parent (M)	5	MPS I	2013/2014	Laronidase (Aldurazyme)	MOH	4	50–100 km
C6	Parent (M)	13	Metabolic urea cyclic disorder	2003/2003	Special diet and milk	MOH	Eery 3 months	>100 km
C7	Patient (C)	26	MPS IVA	1992/2016	Elosulfase (Vimizim)	Society (MLDA)	4	<50 km
C8	Parent (M)	17	MPS II	2008/2011	Idursulfase (Elaprase)	MOH	4	<50 km
C9	Parent (I)	11	MPS VI	2008/2011	Galsulfase (Naglazyme)	MOH	4	>100 km
C10	Parent (C)	28	Pompe	2012/2015	Alglucosidase (Myozyme)	Society (MLDA)	2	>100 km
C11	Parent (M)	29	MPS VI	2011/2014	Galsulfase (Naglazyme)	MOH	4	>100 km
C12	Parent (I)	20	Fragile X	2004/NA	no treatment	NA	NA	NA
C13	Patient (M)	33	SMA	2006/NA	no treatment	NA	NA	NA
C14	Parent (M)	11	MPS II	2011/2014	Idursulfase (Elaprase)	ICAP	4	<50 km
C15	Parent (C)	16	MPS II	2006/2012	Idursulfase (Hunterase)	MOH	4	>100 km
C16	Parent (C)	7	MPS II	2011/2015	Idursulfase (Elaprase)		4	<50 km
C17	Parent (M)	3	MPS II	2013/2013	Idursulfase (Elaprase)	ICAP	4	>100 km

Note: Race: C, Chinese; I, Indian; M, Malay.

ICAP, International Compassionate Assistance Program; MERRF, Myoclonic epilepsy associated with ragged red fibers; MLDA, Malaysia Lysosomal Disease Association; MPS, Mucopolysaccharidosis; NA, not applicable.

This underscores a pivotal message for all healthcare providers. By capitalizing on their role as the primary source of information, healthcare providers can engage in collaborative efforts with patient associations. This partnership is instrumental in delivering comprehensive health information to rare disease patients, fostering a deeper understanding and positively influencing their illness perceptions.

Supplementary material. The supplementary material for this article can be found at <http://doi.org/10.1017/S0266462324000333>.

Funding statement. This research received no funding from any agency, commercial, or not-for-profit sectors.

Competing interest. All authors report that they have no potential conflicts of interest.

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