

Ultragenyx Pharmaceutical is looking for people living with MPS VII to participate in a disease monitoring program (DMP).

A **DMP** is a **long-term program** through which physicians and researchers collect information from people living with a disease in order to learn more about it.

A DMP may collect information on:

- How the disease changes over time
- How people may experience the disease differently
- The effectiveness and safety of medicines used to treat the disease



Why participate?

Participating offers many benefits – not just to you, but also to other patients who have been or will be diagnosed with MPS VII. Information learned from the program will help researchers better understand the disease. For you or your child, participating can offer:

- Access to **world-class medical facilities, highly specialized care**, and evaluation by leading physicians and researchers.
- A chance **to learn more about your disease**, treatment, or disease management options.
- **Regular, personalized reports**, including lab and exam results, that can be shared with the doctors and specialists you regularly see where you live.

Finding support and resources

Dedicated organizations support the MPS community throughout the world. Visit the organizations below to learn more about MPS, find resources, and connect with others.

Global	
International MPS Network (IMPSN)	impsnetwork.org.br
US	
National MPS Society	mpssociety.org
Argentina	
A.M.A Asociacion de Mucopolisacaridosis Argentina	facebook.com/MpsArgentina
Fundación ENHUE	fundacionenhue.org
Brazil	
Associação Catarinense de Doenças Raras	acdracamu.org
Vidas Raras	vidasraras.org.br/site
Casa Hunter	casahunter.org.br
France	
Vaincre les Maladies Lysosomales	vml-asso.org
Germany	
Gesellschaft für Mukopolysaccharidosen	mps-ev.de
Netherlands	
Volwassen, Kinderen en Stofwisselingsziekten	stofwisselingsziekten.nl
Spain & Portugal	
MPS España	mpsesp.org
Turkey	
MPS LH Dernegi	mpsturk.org

MPS VII

(Mucopolysaccharidosis Type VII)

Disease Monitoring Program



ultragenyx
pharmaceutical



The MPS VII DMP¹

Program goal: The DMP is designed to learn more about MPS VII, including how the disease presents (the signs and symptoms), how those symptoms change over time, and the long-term effectiveness and safety of vestronidase alfa, an enzyme replacement therapy for MPS VII.

Expected number of patients to be enrolled: 35 children and adults

Program locations: 15 sites worldwide

United States

- California
- Illinois
- New York
- Utah
- Washington
- Washington, D.C.

Other countries

- Argentina
- Brazil
- France
- Germany
- The Netherlands
- Portugal
- Spain
- Turkey



Who can participate?¹

Any person diagnosed with MPS VII can participate. You can participate whether or not you are taking any medication for MPS VII. The DMP will not provide any medication as part of the program.



What costs are involved in DMP participation?

If you need to travel to the program site, **your travel costs may be covered or you may be reimbursed for reasonable accommodations**, including meals, travel, and out-of-pocket costs that are directly related to your DMP participation. Check with your program site because reimbursement rules vary by country.

Program site visits¹

The DMP has a schedule of **regular visits and phone calls** over a 10-year period. All patients will visit the program site in-person at least once per year:

Children less than 5 years old

- 2 visits per year for the first 2 years
- 1 visit per year for the rest of the program

Children and adults 5 years or older

- 2 visits per year for the first year
- 1 visit per year for the rest of the program

All patients or caregivers will receive regular phone calls from the doctor and program team.



Program Tests¹

While you are a part of the DMP, doctors will perform a number of tests at regular intervals during your in-person visits to evaluate:

- How well your lung, heart, and brain are working
- The level of GAGs (complex sugars) in your urine
- Your spleen and liver size
- How far you can walk in six minutes
- How well you can hear

If you take vestronidase alfa, the doctor will also evaluate:

- The number of antibodies to vestronidase alfa in your body
- The safety of vestronidase alfa

Doctors will also ask patients and caregivers questions about symptoms, behavior, functioning, and health-related quality of life.

Contact Us

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1. Ultragenyx, data on file (MPS VII DMP Protocol)