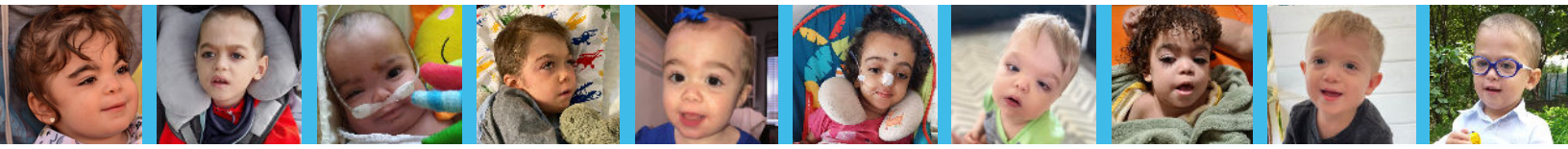
UNITED MSD FOUNDATION

MULTIPLE SULFATASE DEFICIENCY



MSD101

Multiple Sulfatase Deficiency

WHAT IS IT?

MSD is an inherited genetic disease that inhibits the body's natural ability to dispose of cellular waste.

ULTRA-RARE

affecting **1 in 500,000** worldwide

Statistically, there are more than 16,000 global MSD patients.

HOW DOES IT WORK?

It all starts with the ***SUMF1*** **GENE.** It is responsible for encoding a protein called formylglycine-generating enzyme (FGE).

MSD patients have problems with both copies of the *SUMF1* gene functioning properly.

FGE enzyme

SUMF1 **ENCODES** the **FGE enzyme** which is responsible for **activating all 17** sulfatase enzymes in our body.

The sulfatase enzymes break down and remove cellular waste produced by our bodies.

17

sulfatases [enzymes] get to work breaking down the trash that **every cell in the body makes everyday.**

These sulfatases are **crucial for life.**

Without this process,

THE BODY WILL NOT SURVIVE!

Waste builds up over time, clogging vital systems such as the brain, muscles, eyes and more preventing them from working properly.

Who is United MSD Foundation?

Founded in 2016, United MSD Foundation is a registered 501(c)(3) nonprofit serving an international community of Multiple Sulfatase Deficiency families, researchers, and care providers. We exist to bring awareness to MSD, fund research toward treatment, and support families through education, resources, and community.

Funding Critical Research

Since inception, our mission has been to cure MSD. To that end, we have funded a gene therapy mouse model and subsequent toxicology study, and a Natural History Study. We run a MSD Biobank and Patient Registry to provide researchers with access to patient data and samples. Currently, we are supporting first-in-human gene therapy trials with our partners at the Children's Hospital of Philadelphia.

Family Support

Everyone's journey with MSD is unique. Rare diseases often leave individuals and families feeling isolated, but as a part of the MSD community, we are here to embrace and support MSD families all along their journey. We support MSD families in a variety of ways, including through providing the following:

- A Comprehensive Patient Resource Guide
- Clinical Care Guidelines
- Monthly Family Support Meetings
- Family Mentor Program
- Access to a Private MSD Family Support Facebook Group
- Opportunities to Participate in Research
- Biennial MSD Scientific and Family Conference
- Interpreters