Mucopolysaccharidoses (MPS) and Mucolipidoses (ML) are genetic lysosomal storage diseases (LSDs) caused by the body’s inability to produce specific enzymes. Normally, the body uses enzymes to break down and recycle materials in cells. In individuals with MPS or ML, the missing or insufficient enzyme prevents the proper recycling process, resulting in the storage of materials in virtually every cell of the body. As a result, cells do not perform properly and may cause progressive damage throughout the body, including the heart, bones, joints, respiratory system, and central nervous system. While the disease may not be apparent at birth, signs and symptoms develop with age as more cells become damaged by the accumulation of cell materials.

### Syndrome	 Eponym	 Enzyme Deficiency
MPS I Hurler, Scheie, Hurler-Scheie
MPS IIA Hunter
MPS IIB Sanfilippo A
MPS IIC Sanfilippo B
MPS IID Sanfilippo C
MPS IIE Sanfilippo D
MPS III A Sanfilippo A
MPS III B Sanfilippo B
MPS III C Sanfilippo C
MPS III D Sanfilippo D
MPS IV A Maroteaux-Lamy
MPS IV B Morquio A
MPS IV B Morquio B
MPS VII Sly
MPS IX  I-Cell, Pseudo-Hurler
ML II/III I-Cell, Pseudo-Hurler

**MISSION STATEMENT**

The National MPS Society exists to cure, support, and advocate for MPS and ML. We provide hope and support for individuals diagnosed with MPS or ML, as well as their families, by funding research, directing advocacy efforts, and driving awareness campaigns throughout the year.

Since 1974, we have been governed by a member-elected, fully volunteer Board of Directors, many of whom are parents of children with MPS or ML. We also benefit from the expertise of a Scientific Advisory Board, comprised of world-class physicians, researchers, and medical professionals throughout the world.

**HOW CAN YOU HELP?**

We need your support to help us educate others about our mission and to raise the money desperately needed for medical research, advocacy, and family support programs—the keys to longer, happier lives for those diagnosed with MPS or ML. You can help by donating whatever amount is possible, buying our logo wear to show your support, or hosting your own fundraiser in your hometown!

**NATIONAL MPS SOCIETY**

Support for Families, Research for a Cure.

mpssociety.org

Since 1974, the National MPS Society has existed to cure, support, and advocate for MPS and ML. Please join us in our efforts to fight these diseases. Only with your help can we continue funding all of the groundbreaking research and extensive family support programs that we manage.

**BECOME A DONOR TODAY.**

Please send an acknowledgement card to:

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State, ZIP ________________________

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☐ Payment online: www.mpssociety.org/donate

Mail to: National MPS Society, PO Box 14686,
Durham, NC 27709-4686

Donations are tax deductible. Tax ID number is 11-2734849

National MPS Society
P.O. Box 14686, Durham, NC 27709-4686

t: 877.MPS.1001 | p: 919.806.0101 | f: 919.806.2055

NATIONAL MPS SOCIETY IS A TAX-EXEMPT PUBLIC CHARITY UNDER IRS SECTION 501(C)3, DEDICATED TO INDIVIDUALS AFFECTED WITH MPS AND ML.
WHAT ARE THE SYMPTOMS OF MPS AND ML?

While the symptoms of the disease vary from one syndrome to another, there are similarities. Affected individuals often have cognitive impairment, cloudy corneas, short stature, stiff joints, incontinence, speech and hearing issues, chronic runny nose, hernia, heart disease, hyperactivity, depression, pain, and a dramatically shortened life span.

HOW ARE THESE DISEASES INHERITED?

MPS diseases are hereditary. In nearly all cases, a child receives a recessive gene from each parent. MPS II is the only exception, where the gene may be passed from a mother to her male children. A couple’s chance of having another child with one of these diseases is 1 in 4 with each new pregnancy. Unaffected siblings may possess the recessive gene and be carriers of the disease. The occurrence of MPS in the general population is thought to be one in 25,000 births.

WHAT TESTING IS AVAILABLE FOR MPS OR ML?

Individuals with an MPS or ML condition may be identified through newborn screening or blood/urine testing. Prenatal testing is also available for those who suspect their unborn child may have the disorder. Tests are also available to determine if individuals are carriers of an MPS or ML gene. To learn more about these tests, contact your doctor, nearest genetic center, or the Society.

HOW IS RESEARCH HELPING TODAY’S FAMILIES?

Although there is currently no cure for MPS or ML, research is making great strides. Carrier detection, newborn screening, enzyme replacement therapy, and gene therapy are among today’s research themes and treatment options. In MPS I, stem cell transplantation may also be an option in certain cases. In the last few years, we’ve made major advancements in research thanks to the direct fundraising efforts of the Society and the self-initiated fundraising efforts of its members.

OUR MISSION STATEMENT—
TO CURE, SUPPORT, AND ADVOCATE
FOR MPS AND ML

To Cure MPS and ML. The Society and our members raise money to support groundbreaking research through fellowships, grants, and projects at numerous prestigious universities and other centers. We collaborate extensively with other lysosomal storage disease organizations and patient support groups. Although amazing accomplishments have been made, it is more important than ever to accelerate the funding of the incredible research that is bringing therapies—and ultimately cures—to children and adults with MPS and ML.

To Support Families. Our family support programs provide a wide-range of resources and information to families. Through our ever-growing series of resource guides dealing with specific syndromes and treatments, our weekly email newsletter, and our bi-annual print magazines, we provide a constant flow of up-to-date information on everything related to MPS and ML. We connect families through personal contact at our annual conferences and regional social events, as well as support families through their first year after diagnosis with personal contact through our Pathways program. In addition, we provide financial support through grants that assist families in obtaining durable medical equipment, attending family conferences, out of town medical appointments, and continuing educational opportunities, pursuing life-enriching extraordinary experiences, and offsetting bereavement expenses.

To Advocate. The Society sponsors public events, issues press releases, publishes syndrome and treatment materials, and maintains a current and dynamic website. To Advocate.

Our legislative committee advocates enhanced research in the pursuit of treatments for MPS and ML; and the establishment of newborn screening. We cultivate working relationships with congressional offices and government agencies and advocate for enhancements to federal and state programs.