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# WHAT ARE MPS AND ML?

# Q:

Mucopolysaccharidosis (MPS) and mucopolysaccharidosis (ML) are rare, lysosomal storage disorders. These are caused by the body's inability to produce or transport specific enzymes into the cells to break down materials. As a result, cells do not perform properly and progressive damage may occur to the heart, bones, joints, respiratory system, and central nervous system.



[www.mpssociety.org](http://www.mpssociety.org)



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# WHAT ARE CHARACTERISTICS OF MPS AND ML?

# Q:

Symptoms vary between syndromes, but may include: short stature, bone and joint issues, speech and hearing impairment, upper respiratory disease, hernia, heart disease, developmental delay, hyperactivity, eye disease, chronic pain, and shortened lifespan.



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# WHAT DOES MUCOPOLYSACCHARIDOSIS MEAN?

# Q:

Mucopolysaccharidosis (MPS) can be broken up into three parts. “Muco” refers to the thick, jelly-like consistency of the molecules that build up. “Poly” means “many.” “Saccharide” is a general term for a sugar molecule.





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# HOW COMMON ARE MPS AND ML?

# Q:

MPS and ML are rare diseases. Current estimates show that across all forms of MPS about 1 baby born out of every 25,000 will have one of these diseases.







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# ARE THERE TREATMENTS FOR MPS AND ML?

# Q:

Enzyme replacement therapy is approved for MPS I, II, IVA, VI, and VII. Transplantation through bone marrow or stem cells is used for treatment in some cases. Clinical trials are underway and explore options such as alternative ways to administer enzyme replacement therapies, different formulations of treatments, gene editing, and gene therapy.





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**WHAT IS THE  
MISSION OF  
THE NATIONAL  
MPS SOCIETY?**

**Q:**

The National MPS Society exists to cure, support, and advocate for MPS and ML. Working to achieve this mission involves supporting researching through funding fellowships and grants, supporting families through programs and an annual family conference, and increasing public and professional awareness through advocacy efforts.

