



National MPS Society Talking Points for Legislative Meetings

MPS and ML diseases are rare genetic disorders that primarily affect children. They cause progressive damage to cells in the body, resulting in severe disability and early death. There are few treatments and no cures. But there is hope.

The following talking points should provide enough information for you to feel comfortable talking to lawmakers. You can pull from these talking points as needed to conduct your meetings, but remember that you do NOT need to cover all of these points!

Describing the National MPS Society

- The National MPS Society supports research to find cures for mucopolysaccharidoses (MPS) and mucopolysaccharidosis (ML), and provides hope and support for affected individuals and their families through research, advocacy and awareness of these devastating disorders.
- Nonprofit advocacy 501C3 /support organization that is nearly 40 years old with a four-star rating from Charity Navigator, representing excellent performance.
- Supports a membership of more than 800.
- Raised \$3.3 million for research since FY2002.
- Supports many different diseases, each of which is defined by a specific enzyme deficiency.

Explaining MPS and ML

- The body uses enzymes to break down and recycle materials in cells.
- People with MPS or ML are not able to produce certain enzymes at the right levels or at all.
- This leads to build-up of excess materials in almost every cell of the body, causing cell damage that increases over time as more and more materials accumulate.
- This results in severe problems, including, heart disease, vision loss, speech and hearing impairment, short stature, stiff joints, pain, and profound intellectual disabilities among others.
- There are seven types of MPS diseases and one type of ML, each defined by a specific enzyme deficiency, with a range of severity both between and within each disease.
- There are very few treatments, and those that exist are expensive and difficult for patients and families because they are invasive and require significant time in the hospital.
- Currently, there are no cures for MPS or ML diseases.

How MPS Progresses

- MPS and ML diseases are devastating for children and families, largely due to the progressive nature of the diseases.
- Babies with MPS or ML diseases are often born looking perfectly healthy. It is only later, as cell damage becomes worse, that parents receive this heartbreaking diagnosis.
- MPS and ML diseases end up affecting nearly every part of the body, damaging the heart, bones, joints, respiratory system and central nervous system.
- All MPS and ML diseases are terminal. Although there are adults living with MPS and ML, the life expectancy of those with MPS and ML is shortened, with most children not living beyond their teenage years.

- MPS and ML are not “static” diseases – they are always changing and progressing. This means that families know that the detrimental health impacts will get worse over time.
- While every day with a child living with MPS or ML is a gift, it is also filled with medical concerns and fear for the future.

How MPS Impacts Families

- Caring for children with MPS or ML is a full-time job. They often cannot care for themselves as they get older.
- Parents need to coordinate with dozens of medical experts, including pediatricians, neurologists, geneticists, ENTs, orthopedists, cardiologists, audiologists, dentists and oral surgeons as well as physical, speech and occupational therapists and home nursing aides.
- Children with MPS or ML often need specialized medical equipment, like oxygen pumps, gastrointestinal feeding equipment, therapeutic beds, braces, wheelchairs, and other specialty medical items.
- For these reasons, the costs to care for children with MPS or ML are astronomical.
- The emotional and social impacts are also great. Some children with MPS or ML diseases have significant behavioral problems and sleep infrequently. Siblings are affected in significant ways. School issues impact many MPS/ML children. Vacations and other “normal” family activities all require special planning and accommodations. Having a child with MPS or ML disease affects nearly every aspect of a family’s daily living.

Lack of MPS Treatment Options

- There are **no cures** for any of the MPS or ML diseases.
- There are very few treatments, and some MPS diseases and all of the ML diseases have no treatment options at all.
- There are only a few researchers in the world that are dedicated to MPS and ML, and these few researchers are heading into retirement.
- There is very little funding available from the NIH for new MPS or ML research at this time, preventing young researchers from pursuing MPS or ML research.

Talking about National MPS Society Legislative Priorities

The National MPS Society advocates for policies that will advance treatments and one day a cure for MPS and ML diseases. There are several specific policies we advocated for this year, including:

- **Insertion of MPS-related language into the 2013 Appropriations Bill.**
 - This is language that encourages the National Institutes of Health (NIH) to prioritize MPS and ML-related research
 - MPS and ML researchers get almost **all** of their research funding from the NIH, so encouraging the NIH to allocate funding for MPS and ML-related diseases is absolutely critical.
 - Although there are very few treatments for MPS or ML, the ones we do have are a result of NIH-funded research.
 - Prominent researchers in this field believe that there is real hope for more effective treatments and one day cures though these are not currently available. Much more research is needed to get us to that reality. Encouraging the NIH to pursue MPS research this year is crucial.

- **Legislation to create an accelerated approval process for treatments for ultra-rare diseases including MPS**
 - Parents of children with MPS or ML are in a race against time. They realize that they have limited time for the discovery of treatments that can help or save their children.
 - The FDA has a way to accelerate the approval of treatments for life-threatening diseases that do not currently have treatments or cures - but ultra-rare diseases like MPS or ML do not qualify for this accelerated approval track.
 - This makes it nearly impossible and extremely costly for private organizations to pursue research on ultra-rare diseases, leaving those affected with MPS or ML without options.
 - This will positively impact all rare diseases.
 - Opening the accelerated approval pathway gives parents of children with MPS or ML hope that an effective treatment may be found during their child's lifetime that will improve his or her quality of life dramatically.
 - This would NOT by-pass clinical trials – it would simply get new therapies to the clinical trial phase more quickly. Basically, it gets possible treatments to the starting line faster.
 - Every day we wait to test new therapies, our children's disease progresses.
 - Several pieces of legislation in Congress this year (FAST, ULTRA, and TREAT) addressed this and we are hopeful that we have begun to move the issue forward.

- **Passage National MPS Awareness Day Resolution**
 - The May 15th National MPS Awareness Day Resolution helps us raise awareness about these devastating diseases. This can result in more interest among young researchers to enter this field, new donors to support MPS and ML research, more community support for MPS/ML patients and their families, and much more.
 - There are many in the medical and research communities who are not knowledgeable about MPS or ML - a national awareness day helps us educate these important groups.
 - Acknowledging MPS and ML as a nation gives families whose children are suffering from MPS or ML the sense that their struggle is recognized and the lives of their children are valued. The impact of this cannot be underestimated!

For more information about the legislative policy priorities of the National MPS Society, refer to the Fact Sheet in the Toolkit.

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