



National MPS Society Talking Points for Legislative Meetings

MPS diseases are rare genetic diseases that affect both children and adults. 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.

While the talking points below should help guide your conversations with members of Congress and their staff, do NOT feel that you need to cover all of the information. In fact, you will not get nearly enough time in your meeting to cover all of it. You have fact sheets as leave-behind materials for the information you don't have time to address. Also, remember that one of THE most important goals of your visits with members and their staff is to get to know them and have them get to know you. Don't be afraid to be conversational and spend time making "small talk". Also, don't be shy about asking questions to get to know them a bit better as well. Do they know anyone with a rare disease? Do they have a Facebook page and Twitter handle? What do they think are the most important issues facing constituents in their district?

It is perfectly acceptable to bring notes to your meeting – and to take notes as well!

Describing the National MPS Society

- The National MPS Society supports research to find cures for Mucopolysaccharidoses (MPS) and related diseases, and provides hope and support for affected individuals and their families through research, advocacy and awareness of these devastating disorders.
- Nonprofit advocacy 501(c)3 /support organization that is more than 47 years old with a four-star rating from Charity Navigator, representing excellent performance.
- Supports a membership of more than 2000 households.
- Awards approximately \$600,000 for research annually.
- Supports many different diseases, each of which is defined by a specific enzyme deficiency.

Explaining MPS

- The body uses enzymes to break down and recycle materials in cells.
- People with MPS 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 profound intellectual disabilities, heart disease, vision loss, speech and hearing impairment, short stature, stiff joints, and pain, among others.
- There are many types of MPS diseases, each defined by a specific enzyme deficiency, with a range of severity and impacts 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 may require significant time in the hospital.
- Currently, there are no cures for MPS diseases.

How MPS Progresses

- MPS diseases are devastating for children and families, largely due to the progressive nature of the diseases.
- Babies with MPS diseases are often born looking perfectly healthy. It is only later, as cell damage progresses, that parents receive this heartbreaking diagnosis.
- MPS diseases end up affecting nearly every part of the body, damaging the heart, bones, joints, respiratory system and central nervous system.
- All MPS diseases are terminal. Although there are adults living with MPS diseases, the life expectancy of those with MPS is short, with most children not living beyond their teenage years.
- MPS is a progressive disease – it is always changing. This means that families know that the detrimental health impacts will get worse over time.
- While every day with a child living with MPS disease is a gift, it is also filled with profound and complex medical concerns and fear for the future.

How MPS Impacts Families

- Caring for children with MPS is a full-time job. They cannot care for themselves.
- 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 often need specialized medical equipment, such as 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 are astronomical.
- The emotional and social impacts are also great. Some children with MPS diseases have significant behavioral problems and sleep infrequently. Siblings are affected in significant ways. School issues impact many children with MPS. Vacations and other “normal” family activities all require special planning and accommodations. Having a child with MPS 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 diseases.
- There are very few treatments, and some MPS diseases have no treatment options at all.
- There are only a few researchers in the world who are dedicated to MPS and related diseases, and these few researchers are heading into retirement.
- There is very little funding available from the NIH for new MPS research at this time, preventing young researchers from pursuing MPS research.

Talking about National MPS Society Legislative Priorities

While MPS diseases are heartbreaking, families hold on to hope for a future where MPS can be treated and cured. Your support can help children with MPS and their families realize that future.

Tips on Making an Ask

- Every visit should close with an ask – or a series of asks. If making more than one ask (as you will be doing during these visits), ask one at a time, in order of priority, and wait for a response to each question before moving on to the next.
- Asks should be clear and explicit.
- It is essential to wait for a response. If the lawmaker/aide does not commit with a clear “yes” or “no”, let them know you would like to follow up with them to get a clear answer and ask, “When might I be back in touch with you?”
- Write down responses so you will clearly remember what was said and be sure to report those responses back to the Legislative Advocacy Committee after the meeting.

Wrapping up your meeting

- Be sure to thank the lawmaker/aide for his/her time and attention and for their support of MPS diseases, your child, and your family.