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Statement of
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Chair, Committee on Federal Legislation
On Behalf of the
National MPS Society
For the
Social Security Administration
Hearing on
Compassionate Allowance Outreach Hearing on Rare Diseases
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Thank you Commissioner Astrue for the opportunity to represent the National MPS Society and to talk with you about my family and our experiences navigating the disability determination approval process for Social Security. I feel strongly that children with MPS can teach all of us the important role the government plays in caring for people with severe disabilities and I appreciate the opportunity to share my thoughts with you today.

My name is Sissi Langford. I am an elected Director on the Board of the National MPS Society where I serve as Chair of the Committee on Federal Legislation. This committee acts as a liaison between Congress and relevant government agencies and our general membership. The MPS Society is a non-profit 501c3 family support organization. Our goal is to ultimately find a cure for MPS disorders by supporting research, providing support to individuals and their families affected by MPS, and promoting public and professional awareness. Due to the often short life expectancy of people affected by MPS the majority of our affected members are children and their families. Our membership also includes a small percentage of adults who suffer from MPS. The vast majority of people with MPS experience significant medical problems. These problems vary among the affected individuals. Some children are considered mildly affected while others seek treatments which have drastically improved their quality of life. These treatments are not cures and are not available for many MPS children. I recognize that these factors make it difficult to set policy for the Social Security Administration benefit approval process. I hope that this discussion will provide the Office with some insight on MPS disorders.

The Mucopolysaccharidosis(MPS) disorders are genetically determined lysosomal storage disorders (LSD), resulting in the body's inability to produce certain enzymes. This lack of enzyme production results in the interruption of the usual breakdown of specific normal molecules (complex carbohydrates) that are stored in virtually every cell in the body. Storage causes progressive cell damage in multiple systems within the body including respiratory, bones, internal organs, heart and central nervous system. The results of this damage include mental retardation, short stature, corneal damage, joint stiffness, loss of mobility, speech and hearing impairment, heart disease, hyperactivity,

chronic respiratory and digestive problems and most importantly, drastically shortened life span.

My twelve-year-old son, Joe and my eleven-year-old daughter, Maggie suffer from Sanfilippo syndrome or MPSIII, a result of a genetic mutation in both mine and my husband's DNA. There is a one in four chance that each one of our children will have the mutation that causes MPS III. Because of the extensive central nervous system involvement there are no treatments available for children with MPS III.

We live on Johns Island, South Carolina where we receive good state and county-level services and have access to a medical university in nearby Charleston for management of this disorder. Many other MPS families do not have the level of care we have here in South Carolina. Improvements to the disability determination process will benefit MPS families.

Joe was born in February, 1995 and developed normally except for frequent ear infections and frequent diarrhea. Maggie was born in August, 1996 and also appeared to be a normal, healthy child. Joe started dropping words at a time when he should have been gaining them and our pediatrician noticed that his spleen was enlarged which was an indication of a storage problem. Our pediatrician referred Joe to a geneticist for testing and to BabyNet in May, 1998. The diagnosis process took over a year and during that time we realized that Maggie was experiencing similar decline. Joe and Maggie were diagnosed with Sanfilippo in June of 1999. My husband and I researched MPS and we were devastated by what we learned. Joe and Maggie seemed mostly normal so we could not imagine they would decline as described.

Now, Joe and Maggie depend on wheelchairs for mobility. They are no longer verbal and have lost the ability to swallow. They do not laugh or cry. They both rely completely on feeding tubes for nutrition. They have heart defects and are treated for seizures. They also take medications to sleep and to control agitation and involuntary movement. They are seen regularly by cardiologists, neurologists, gastroenterologists, orthopedists and other specialists. Joe and Maggie are now developmentally about 12 months old and will continue to decline as MPS causes more medical problems. Children with MPS III have a life expectancy of an average of fourteen years. It is our hope that Joe and Maggie will live longer than average and that they can continue to enjoy an acceptable quality of life.

It is an overwhelming challenge to provide our children with an acceptable quality of life due to their decline and the current level of care required to keep them as healthy as possible. Our family's ability to manage the care of Joe and Maggie is a direct result of the services we receive from the state of South Carolina which includes funding from the federal government. The process begins with the Social Security Administration's determination of eligibility. Many families fail to recognize the importance of determining eligibility early in their children's lives. MPS disorders are all degenerative which makes it difficult for parents, teachers and medical providers to accept that an active, vocal child will lose skills.

My husband and I were reluctant to apply to the Social Security Administration for disability determination for services because we had not accepted the reality of our children's condition and because we felt we should be able to handle our families' medical needs with our private insurance and personal resources. The early interventionists and other health professionals working with us recognized that we

struggled with this diagnosis and they consistently persuaded us to prepare for the future. We were strongly advised to pursue any services available to our family. The process was long and complicated. While Joe was accepted through the SSA for Medicaid and the Waiver, Maggie was declined because she was “not delayed enough”. I had to appeal this decision and attended a hearing in order to get Maggie on the waiting list for services. I had to learn my rights and how to get my doctors to write meaningful letters to describe our situation. This took several years. Our local agencies recommended other funding programs and encouraged us to apply for grants to pay for diapers (over 350 dollars per month) during the long delay to get through the approval process and off the waiting lists.

Description of state-provided services and budgets for Joe and Maggie:

- Joe and Maggie are both covered under the TEFRA (Katie Beckett Waiver) which is a category for children under age 19 who meet the SSI childhood disability definition and for which home care is more economical than a hospital or community home. It is based on the child’s income rather than family income. The child must meet institutional level of care and it must be appropriate to care for the child in the home.

- We applied for the TEFRA program in October 1999. Joe was made eligible in November 1999, but was placed on a Waiting List at slot # 12. Maggie was denied eligibility in May 2000. The hearing to appeal the denial was in August 2000. At that point Maggie was ruled eligible. Both children began getting services under the MR/MD Waiver in Feb 2003.

- Joe’s estimated budget total for 2006-2007 is $69,913.00 Maggie’s estimated budget total for 2006-2007 is $79,092.40. This does not reflect the amount our private insurance pays out.

- The children are eligible for additional nursing hours but it is hard to find more nursing care at this time. The budget reflects the nursing/personal care assistant hours we use, not the total for which they are eligible.

Even with this level of assistance we have completely altered our lifestyle to care for our children including moving to a home near a school that provides them with an appropriate educational experience. I can no longer work outside my home and have had to drastically reduce my professional work load to manage the care of my children. We will continue to do what we can to make sure Joe and Maggie are as healthy as they can be as they progress and eventually become unable to keep up with the devastating effects of MPS. It is impossible for me to express how hard these children fight for their health. Their ability to keep up this fight has given me the courage and inspiration to try to seek improvements for all MPS children.

The following points from the South Carolina Department of Disabilities and Special Needs (DDSN) 2006-2007 Accountability Report illustrate how services are provided in my state. The opportunities and barriers are in line with what other parents express as the problems we face in getting initial and ongoing approvals for services.

- DDSN receives/utilizes approximately $300 million in federal Medicaid funding to provide services.

- DDSN currently serves over 28,000 persons with mental retardation and related disabilities, autism, head injury and spinal cord injury. Approximately 82 percent of these individuals live at home with their families, which compares to only 60 percent nationally.
- Community residential services and in-home support services are provided through contracts with local disabilities and special needs boards and other community providers.

The report highlights the following Opportunities:

- Increase use of Medicaid funding to develop flexible in-home supports for increased individual/family independence and prevention of more costly out-of-home residential placements.
- Strengthen technology capacities to support self-determination initiatives and create efficiencies.
- Enhance service provider productivity and efficiency.

The report highlights the following Barriers:

- Turnover is very limited in the service system as severe disabilities are lifelong and many people are waiting for the essential services they need to be more independent.
- Waiting lists continue to grow. DDSN has 2,013 people waiting for residential services and a waiting list of 1,099 people for day and employment programs.
- The recruitment and retention of nurses continues to be extremely difficult in specific locations around the state. The unavailability of nurses caused by a nation-wide shortage of nurses is further complicated by competition from nursing homes, doctor's offices, school districts, and other providers.

It is encouraging to know that my state recognizes some of the same challenges parents see and it is my hope that South Carolina and other states will continue to look for solutions. It is also encouraging that Commissioner Michael J. Astrue is committed to improving the disability determination process for the Disability Insurance and Supplemental Security Income programs.

Understanding the problem:

Emotional Response to the Disorder: The majority of parents who have been through this process state that they feel the main problem for initial approval through the Social Security Administration was the lack of knowledge about MPS. Specifically, the degree and rate of decline these children face. Most parents eventually recognize that they must advocate for their child and that they must educate everyone involved with their child's care about the nature of MPS. The disorder is rare and is hard to understand. It is hard to pronounce, spell and remember (unless you have a child with it). It is degenerative and it is impossible to predict how quickly decline will occur. Unfortunately, many parents are not emotionally ready to take on the role of advocate soon after diagnosis when they should be working on getting approvals for services through the Social Security Administration. If MPS was on the Compassionate Allowance list it would certainly lessen the burden on the parent. It will allow the State to start helping the family as early as possible which will give the parent some time to acclimate to the important role of advocate.

Early Approval for services and equipment: It is important to recognize that success depends on the approval of services prior to need. Our physical therapist was adamant that we start the process for an adaptive van at least two years before we actually

needed it. She had to convince me to start the process for modifications even though my children were still walking. By the time the children were completely confined to wheelchairs we had the van and modifications in place. If we had waited until the children were no longer walking we would have faced huge transportation issues to medical appointments and to school. Our children each weigh over 55 pounds and need to be transported safely in their chairs. Our success was a result of an experienced professional who could predict our need and who went beyond the required scope of her job to get me on board.

Relevant Technical Training for Service Coordinators: Another problem MPS parents have experienced is a lack of technical training for service coordinators who must seek approvals for services. Parents state that service coordinators or case workers are not adequately trained to file paperwork that will not be denied or the approving agency is not trained to respond so problems can be corrected quickly. The ranking governing agency often returns declined paperwork with limited explanation for the denial. Parents request that this type of delay in approval for services be addressed by providing relevant, regular technical training for everyone involved with the approval process.

Identify strong Service Coordinators: It is not practical for all service coordinators to be trained in the unique challenges of a rare disorder. Identify and match strong service coordinators with families with rare diseases. Beyond me (the parent) our service coordinator is the most important person at the table. She has learned to plan ahead to keep up with our changing needs. She has learned to accept that the emotional impact of MPS can affect how I handle problems. She communicates with me often via email or a simple phone call. She recently visited us while my son was in the hospital for 24 days. While this level of attention may seem intense (since she has many other clients) it actually saves time in the long run because we are always up to date. Most importantly, she writes letters and assists medical professionals with letters that get approved. I had several other service coordinators with different, unsuccessful approaches. I had to learn to advocate for a service coordinator who can handle our situation.

Conclusions:

MPS parents and government agencies share the same goal: keep children at home while providing support that allows the family to continue to function. The parent and service coordinator must develop a working relationship to plan ahead for the challenges of caring for MPS children. The doctors and other health professionals must be open to writing descriptive letters that illustrate the disease process to allow the reviewer to understand the complex nature of MPS. The approving agencies must recognize that there is not a cookbook approach to approving the services needed to keep these children in their homes. Information describing the impact of the disorder would be helpful but a change in the philosophy for handling a rare, degenerative disorder like MPS will provide the greatest improvement.

Thank you for taking time to learn about MPS and Joe and Maggie. I appreciate the opportunity to share this with you and the fact that you value my views. I think this is an important step to improving the approval process.