

## TESTIMONY FOR PATRICIA FURLONG

My name is Patricia Furlong, and I am the President of Parent Project Muscular Dystrophy (PPMD). We are focused on Duchenne muscular dystrophy. Our mission is to improve the treatment, quality of life and long-term outlook for all individuals affected by Duchenne muscular dystrophy (DMD) through research, advocacy, education and compassion. I am testifying on behalf of the PPMD's families in the United States, as an expert in Duchenne muscular dystrophy and as an individual who has experienced the effects that a DMD diagnosis has on a family and a community. My two boys were diagnosed in 1984. They died at ages 15 and 17. I appreciate the invitation to testify today and I want to especially thank the Social Security Administration for allowing me the opportunity to speak on behalf of the muscular dystrophy community.

There are several key points that I would like to make in my testimony today:

- Confusion related to programs within SSA
- Lack of information, knowledge and categorization of rare conditions within the system and with those processing claims
- Lack of knowledge within a given disease specific community related to specific programs within SSA
- Concern that SSA programs discourage independence

Muscular dystrophy is a broad term that refers to a number of primary diseases of muscle. These include dystrophinopathies (Duchenne and Becker), myotonic dystrophy, distal myopathies, Emery-Dreifuss, facioscapulohumeral, oculopharyngeal, and the limb girdle muscular dystrophies. Many aspects of these diseases are not understood, and treatments are either unavailable or minimally effective. The age of onset and severity of these diseases is highly variable.

Mention muscular dystrophy and Duchenne muscular dystrophy (DMD) is the one that most people think of, whether or not they know its name. Images of these young boys don't easily leave your mind. DMD is the most severe of all of the muscular dystrophies. It is the world's most common and catastrophic form of genetic childhood disease. DMD represents 90% of all childhood onset muscular dystrophy cases, and is characterized by rapidly progressive muscle weakness that results in death, generally by 20 years of age. One in 3500 male children will be born with the disease, about a third of them into families with no previous history of the disease. These boys will lose the ability to walk by age 10, and will gradually lose their ability to breathe, until they die in their late teens or early twenties.

Individuals who apply for benefits for a child diagnosed with Duchenne muscular dystrophy are often applying for Medicaid or the Medicaid Waiver program through their local Department of Health/Family and Human Services. Some are aware of both programs, others have no knowledge whatsoever. The application process required in most states uses the same guidelines to meet the criteria for being 'disabled' and this results in confusion for families as they are often unaware of the requirements to qualify as 'disabled' and for Income and Asset limits. In some states, approval is granted within two to four months, while in other states, the approval process is as long as six to nine months. Long waits are the result of many factors to include families unfamiliar with the process. These families often fail to provide necessary materials and have difficulty obtaining supportive medical documentation to include appropriate clinical diagnosis. Families are often unable to provide sufficient documentation to fulfill the criteria for being "disabled". There is sometimes a significant difference of opinion and lack of 'evidence' in order to determine what is 'medical necessity' or 'educational necessity'.

Individuals with catastrophic and disabling diagnosis want and deserve to be independent. Developing systems that provide benefit while encouraging independence is essential.

Individuals who are able to work, albeit part-time, should not be denied benefits, thus discouraging independence in the disabled community.

The primary purpose of programs under SSA is to assist families and individuals living with disabling conditions. In Duchenne muscular dystrophy, the natural history is well known, though there is significant variability in rates of progression. Because SSA programs are developed to serve rare, disabling conditions and determine under what circumstances an individual meets or will soon meet eligibility presents a significant challenge. It seems important to develop a list of conditions in which patients will meet or will soon meet eligibility seems critical. Many disabling conditions require additional medical and living expenses for families prior to the individual meeting the specific definition of 'disabled'. Removing obstacles for obtaining benefits will necessitate educating both those processing claims and families or individuals in need.

Again, I commend SSA their desire to develop systems that serve those with catastrophic and disabling conditions. Improving access to such services will have a significant impact on the quality of life. Thank you for your time and compassion. I would be happy to answer any questions.