
**Testimony Submitted by the National Organization for Rare Disorders**
**Before the**
**Social Security Administration**
**Public Hearing on Compassionate Allowances**
**December 4, 2007**

Good morning. My name is Diane Edquist Dorman, Vice President for Public Policy for the National Organization for Rare Disorders. On behalf of NORD and the millions of Americans affected by rare diseases, I want to thank you for the opportunity to speak before you today regarding the Social Security Administration's desire to improve the rules for Compassionate Allowances for individuals affected by a rare disease.

As mentioned in our comments submitted to the Social Security Administration this past September, the men, women and children seriously affected by rare diseases, many of which are severely debilitating and/or life-threatening, are routinely denied Social Security Disability insurance and are forced to go through the lengthy and often expensive appeals process. This is because nearly 100 percent of the rare diseases are not in the SSA *Listing of Impairments*, nor are they included in the U.N's *International Classification of Diseases*.

In many cases, initial denials of benefits is reversed following appeals but not before patients and their families have lost precious time and spent thousands of dollars on legal assistance.

Rather than discussing the course of individual rare diseases, for the most part, I would like to provide a panoramic, 10,000 foot view.

- According the Office of Rare Diseases at the National Institutes of Health, there are approximately 7,000 known rare diseases, each of which affects far fewer than 200,000 people in the U.S. The NIH estimates that, in the aggregate, between nine and ten percent of the U.S. population has been diagnosed with one of these rare diseases, disorders or syndromes.

- Eighty-five to 90 percent of known rare diseases are chronic, serious or life-threatening.

- Approximately 80 percent of these diseases are genetic. Consequently, I make the assumption that children are inordinately impacted. This is evidenced by the fact that about 50% of the over 300 orphan products are approved for pediatric use.

- The diagnosis of a rare disease often takes years. Little evidence is available to support a diagnosis. Experts in the field are few and far between, and doctors know little of these diseases. As a result, patients and their families are shuffled from one specialist to another.

- In a survey NORD conducted with Sarah Lawrence College several years ago, 42% of the respondents said they were prevented from working because of their disease. (Survey sent to 15,000 people. Response rate was 9%)

- In same survey, 77% of the respondents said their rare disease had caused them or their families a financial burden. 32% characterized that burden as "extreme."

- Many rare disorders have no surrogate endpoints, markers or tests. Some are diagnosed based on clinical observations only, others by genetic tests.

When Commissioner Astrue spoke at the NORD Annual Conference in September, he emphasized that the impetus for change comes from SSA and not from external sources. In other words, SSA truly desires to provide better service to claimants who are currently being subjected to unnecessary, emotionally draining, and costly delays. NORD applauds this pro-active approach and wants to assure all involved that we and our medical advisors will support SSA in the effort to improve its service for patients with rare diseases in any way that we can.

Thank you.