Statement of Stephen Groft, Pharm.D.
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Thank you very much Commissioner Astrue for this opportunity to join you at these hearings on Compassionate allowances for patients with rare diseases. More importantly, I would like to express a sincere thank you for the willingness to address this concern. This need for special consideration was expressed by the DHHS National Commission on Orphan Diseases Report to Congress in 1989.

A review and discussion of rare diseases is extremely difficult. There are approximately 7000 inherited and acquired rare diseases affecting between 25 million to 30 million people in the United States. There is no one predictable pattern of progression of the disorders that may eventually lead to physical or emotional disability. However, for many individual rare diseases the progression can be predictable and dramatic. There are many distinguished presenters who have indicated a willingness to provide their experiences in the form of testimony during the next two days. I am sure you have heard from many more unable to join us today. When I used the term distinguished, it refers to the commitment and accomplishments these individuals have made to research and information development and dissemination activities to advance the knowledge about their rare disease. The patient advocacy groups are now considered to be partners with the research community in their quest for diagnostics and treatment interventions.

We will hear numerous suggestions for possible paths to follow as you determine the best choice to address the issue. I think I can say with a certain confidence that whatever path you choose, there will be numerous clinicians, research investigators, and patient advocacy groups supporting your position and assisting in ways beyond our imagination. They have done so during the past 25 years since the Orphan Drug Act was signed in 1983.

I would also like to complement the SSA staff who have worked diligently to address the issues surrounding the proposed program. They have generated the excitement that is necessary to sustain this initiative. We at NIH look forward to the opportunity to continue the collaborative efforts to help meet the needs of patients with rare diseases and their families who frequently are the primary caregivers for so many with rare diseases.