U.S. SENATOR BOB CASEY

CHAIRMAN Special Committee on Aging

Chairman Bob Casey's Opening Statement
"Unlocking Hope: Access to Therapies for People with Rare, Progressive, and Serious Diseases"

Good morning, the Senate Special Committee on Aging will come to order. Welcome everybody, welcome to the Committee's eighth hearing of the 118th Congress, about drug development for rare and serious diseases. As is the tradition of the Committee, the Aging Committee Ranking Member, Ranking Member, in this case will offer an opening statement.

Well, thank you, Ranking Member Braun and of course he worked to put this hearing together. His staff and our staff and I am grateful for your work on this. This is a topic that is obviously meaningful to everyone is this room and every member of the Committee and I look forward to a robust discussion today.

Rare diseases are those that affect fewer than 200,000 Americans, and over 7,000 rare diseases have been identified, seven thousand! While each individual rare disease may affect only a small number of people, collectively, it is estimated that some 25 to 30 million Americans are living with a rare disease. We have done a lot of research into and drug development for these diseases. In 1983, prior to the passage of the Orphan Drug Act, there were just 38 drugs approved to treat rare diseases. In the 40 years since, we have seen over 1,100, eleven hundred, new drugs or new indications for drugs approved for rare diseases.

I am proud to have worked on legislation, entitled the Creating Hope Act, to incentivize the development of drugs for rare pediatric diseases in particular, and other legislation to address some of the challenges faced by those living with, what you might call, ultra-rare diseases, which affect an even smaller number of people. My legislation, the Creating Hope Act, captures what we as legislators are trying to do: to give Americans living with rare diseases, hope for the future. And while we have made some progress, many challenges remain; as we will hear from our witnesses today, too many people living with—and dying from—rare diseases still do not have FDA-approved therapies to treat or mitigate their conditions.

I believe that every one of those individuals deserves access to an FDA-approved, safe, and effective therapy. So, we must make sure that research continues and that clinical trials are designed to allow effective therapies to reach patients as quickly as possible. Rare diseases, and

the research and clinical trials for drugs to treat them, are fundamentally different from more common conditions. We will explore some of those differences, and the ways in which they present challenges for drug development, through our witnesses and their testimony today.

It is important to note that my observations on the challenges facing the rare disease community, and the steps we might consider to address those challenges, don't necessarily apply to other, non-rare conditions. But as we will hear today, the challenges for drug development start from the earliest stages of development.

When patient populations are small, it is hard for clinicians and researchers to develop what is known as a "natural history" for the disease, or what the trajectory of the disease is when it is not treated. Natural histories are important for developing therapies, because if you don't understand how a disease progresses, it's hard to measure whether a new therapy is working. Rare diseases are often heterogeneous, meaning that not every patient's disease will progress in the same way, compounding the problems of poorly-understood natural histories.

Once a promising drug candidate makes it to clinical trials, there are further challenges. Drugs intended to treat common conditions are tested in hundreds or thousands of people. When your entire patient population is only a few thousand, or a few hundred, or a few dozen patients, it's simply not possible to do the same types of clinical trials. And when you have smaller trials, it is more difficult to demonstrate efficacy.

In recent years, the FDA has taken action to improve how it works with companies developing drugs for rare and serious conditions, and the agency has done a lot more work to hear directly from patients affected. More recently, the FDA has announced new initiatives specifically aimed at supporting drug development for rare diseases, such as the Support for clinical Trials Advancing Rare Disease Therapeutics, or START, the acronym START. This is a Pilot Program, to enable real-time communication between a drug sponsor and FDA staff on these development issues. Another important initiative at the FDA is the Rare Disease Endpoint Advancement pilot program, to support drug sponsors' ability to engage with the FDA around the development of so-called endpoints—the metrics of efficacy—for drugs for rare diseases.

In the last decade, the FDA has also done more to hear directly from patients, with Patient-Focused Drug Development meetings. These are meetings that give the FDA staff the opportunity to hear directly from patients about how their disease affects them, and what drug effects are meaningful to them in their daily lives. So, I support the agency's recent steps, but we should continue supporting and strengthening their authorities and to do more, to facilitate more approvals of safe and effective drugs.

So, I look forward to hearing recommendations from our witnesses about the steps we can take to lead to more FDA-approved treatments for rare diseases.