

**U.S. SENATE COMMERCE COMMITTEE HEARING  
TESTIMONY OF MRS. SHELBI OPPENHEIMER  
April 23, 2002**

Thank you, Mr. Chairman and distinguished Committee members.

My name is Shelbie Oppenheimer. I am grateful to have been invited here today to share what I think is an important perspective on the issue of generic drugs versus innovative drugs. To me, this debate is more important than policy, law and politics. To me, this is very personal. To me, drug development is less about the science of chemistry or biology or the complex economics involved or the enormous financial stakes. It's about the reality of life and health. Let me be very straightforward. I have a disease that cannot be cured today. I have Amyotrophic Lateral Sclerosis – ALS, also known as Lou Gehrig's Disease. It is a progressive disorder that occurs when motor nerve cells in the nervous system cease functioning and die. Muscle control becomes completely lost, resulting in paralysis.

The life expectancy of an ALS patient averages about two to five years from the time of diagnosis and there is no known cause, prevention or cure. ALS can strike anyone. There is just one drug available that may extend life expectancy for some ALS patients for a few months, but that drug – as significant as it is—is not the answer for my condition. While I recognize the critical importance of the basic scientific research being done by the National Institutes of Health and The ALS Association, hope for me ...and for others dealing with ALS...today is the discovery or development of better therapies and, perhaps, one day soon, even a cure.

Research, drug development and innovation are the answer for people with ALS. Like many other neurological disorders, ALS is a difficult disease to understand. It's causes and mechanisms are complex and therefore treatment is a maddening, multi-layered puzzle. ALS is not a disease that affects millions of people. At any given time there are about 30,000 people with ALS. So, a drug for my condition will not be a so-called “blockbuster” on the marketplace. A pharmaceutical research and development project

directed to finding new drug treatments for ALS is viewed as costly and difficult, and a very high risk for a company.

The market will never be huge. So, the chance of a big return on investment is a question mark. As I see it, a patent on a new drug is one thing a company can count on to justify its investment.

Mr. Chairman, I am a realistic person. I know that innovation in medicines is not only an intellectual exercise. It is also a business decision. I know that if a project to develop a drug for high blood pressure is weighed against the choice of developing a drug for ALS, I will lose. The size of the patient population, the ultimate potential profit, and patent protection are key components in that decision. I want, perhaps I should say I need, ALS drug development to be competitive in a business environment. I want innovative companies to have the desire to apply their skills to ALS drug development and I want their business considerations to be protected so ALS drugs can be worthwhile to bring to market. They'll certainly be worthwhile to me ... to my family.

Unfortunately, drug innovation is not a walk in the park. As an ALS patient who has seen many potential products fail, I want to stress and repeat what many before me have said, "there are very

few initial drug candidates that ever reach patients.”

There are multiple reasons for this, but one of them is just simply that drug research and development is a risky expensive business.

Sometimes, a company with very good intentions simply can't afford to go out on a limb to develop a "maybe" product that may help very few people. I am interested in any legislation that affects pharmaceutical research and development. I don't want to see legislation that would put people like me at risk of facing a future without incentives for innovation.

Companies that develop brand –name drugs are good at research. Companies manufacturing generic drugs essentially don't do research. They both make positive contributions to health care and are essential factors in economic considerations on many levels. I am simply asking you to be sure any legislation being considered is about patients- all patients, including those who have diseases that are relatively rare and those who are disabled, not just those whose conditions are treated by huge best seller drugs.

Today, I am asking you to be careful and fair. There are some tempting headlines and sound bites here. But I urge your thoughtful

consideration because vote-driven legislation in this case can hurt patients like me.

I am asking you please not to go for what may seem like an easy answer. Instead, think of the effect changes will have on my future.

The drugs that will combat ALS, that will treat very rare cancers, which will truly change our world may only be dreams or vague ideas or they may be right around the corner. We don't know. We do know that people and companies must desire to pursue them and make them a reality. Incentives for companies to develop these drugs must be preserved and must be part of policy.

I am not an expert in the legislative process; I come before you as a person living with ALS. Please don't do anything, however well-intended, that will discourage the pursuit of a treatment and eventually a cure for my horrific disease.

Although I devote myself every day to caring for, loving, and nurturing my daughter Isabel, and not wasting days consumed by what may be, sometimes I can't help but worry... which muscle will fail me next and how will that effect my ability to take care of her? When will my physical limitations become too big to hide from her? Will she need to feed me as I once fed her?

Instead of thinking about a career, weekend plans, what to serve for dinner, and which school for my daughter to attend, I can't help but be angry that I must think about slowly fading away physically and being completely aware of it mentally. I cry at the thought of losing my ability to speak and not being able to tell my daughter and my husband Jeff that I love them. I weep at the thought of not knowing if I will be able to dance at my daughter's wedding. This is my future. This future can change if the right drug is available for me.

Thank you for listening, Mr. Chairmen and Members of the Committee. I would be happy, along with my colleague, Steve Gibson from The ALS Association, to answer any questions you might have.

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