Testimony before the Agriculture, Rural Development, FDA, and Related Agencies Subcommittee of the Senate Committee on Appropriations
June 23, 2010

Mr. Chairman, thank you for the opportunity to testify before you today. I am Diane Dorman and I am the vice president for public policy of the National Organization for Rare Disorders, or NORD. We were founded in 1983 to advocate for the enactment of the Orphan Drug Act, and we remain today the leading advocate for the 30 million American patients with the estimated 7,000 known rare diseases. In addition to our advocacy efforts, we also run patient assistance and patient support programs and have compiled the largest database of information about rare diseases in the world.

There are many federal and state agencies whose programs and policies affect people with rare diseases, but none is more important than the Food and Drug Administration. The FDA is the gatekeeper for the drugs, devices and medical foods that are needed by patients with rare diseases. The FDA sets the standards for studying new medical products and therefore plays a central role in research as well as product approvals.

NORD advocates for full funding of the FDA. We were instrumental in founding an organization now known as the Alliance for a Stronger FDA, which now includes more than 180 members representing all of FDA's stakeholders and which has the singular purpose of advocating for increased funding for the FDA. We have witnessed what happens when FDA is underfunded: the agency cannot meet its review times for new drugs and medical devices; cannot provide the guidance that researchers so desperately seek; and, cannot maintain the public's confidence in the regulatory system.

Delays in review times and lack of guidance affects our patient constituency especially hard, because despite the great advances in medicine, there are approved drugs for only about 200 of the estimated 7000 rare diseases. Many of our patients are treated with approved products being used off-label, and many are not being treated at all because there are no treatments.

NORD's top priority, in addition to the support services we provide to patients with rare diseases, is to advance medical research and the development and approval of new therapies for our patients.

It is with this perspective that we support in principle any steps that would advance medical research or provide FDA with the resources it needs to carry out its critical public health functions. We were encouraged by the creation earlier this year of a new position within the FDA's Center for Drug Evaluation and Research, a position dedicated exclusively to rare diseases.

We advocate for more training and support for FDA personnel who interface with the researchers who develop orphan products, and we support more transparency in the regulatory system so that investigators and drug and device manufacturers can make the right decisions as they develop new products.

The current leadership of the FDA has demonstrated its sensitivity to the vulnerability and special challenges of people with rare diseases. We understand the constraints on the budgets of federal agencies, but at the same time the FDA needs more resources if it is to fulfill the commitment it has made to the rare disease community.

Mr. Chairman, I would be pleased to answer any questions you have. Again, thank you for the opportunity to appear before you on behalf of the 30 million men, women and children with rare diseases.

Respectfully Submitted,

Diane Edquist Dorman
National Organization for Rare Disorders (NORD)
1779 Massachusetts Avenue NW, Suite 500
Washington, DC 20036
(202) 588-5700