



**NORD**  
National Organization for Rare Disorders

**FOR IMMEDIATE RELEASE**

## **Can Our Society Afford to Provide Treatments for People with Rare Diseases?**

*A Medical Adviser to the National Organization for Rare Disorders (NORD) Responds to this Question*

WASHINGTON DC, Oct. 3, 2012---Can our society afford to provide costly medical care for patients with rare diseases, or would that money better be spent on treatments for more common conditions affecting larger numbers of people?

That was the question raised in a recent publication of the Hastings Center, a research institution dedicated to bioethics and the public interest.

Now a medical adviser to the National Organization for Rare Disorders (NORD) has responded to the report, writing that viewing rare diseases as "peripheral and unimportant" to the healthcare system would be a major mistake on several levels.

"Not only does it seem to justify abandoning millions of people but it also can undermine the integrity of the entire research enterprise," notes Doris T. Zallen, PhD, professor of science and technology in society at Virginia Tech and a long-time adviser to NORD. "It can reduce the chance of finding successful treatments for ALL diseases -- common and rare alike."

A rare disease is defined in the U.S. as one affecting fewer than 200,000 Americans. There are approximately 7,000 such diseases affecting nearly 30 million Americans, two-thirds of them children. Only a few hundred of these diseases have medical treatments, and only a small number are being studied by researchers to develop treatments.

The current investment in research on rare diseases is small, Dr. Zallen writes, and further reducing research on rare diseases by deciding that our society can't afford the cost of providing treatment would "harm the broad scientific enterprise" by discouraging young scientists from choosing that career path.

Because rare diseases often have a singular genetic basis, she writes, "the study of rare diseases has already provided fundamental understandings of genetic systems, biochemical pathways, and DNA-repair mechanisms that have helped elucidate the basis of, and improve treatments for, common diseases. The blockbuster drugs Botox and Viagra were originally developed to treat benign essential blepharospasm and pulmonary hypertension, two rare disorders."

Since there is little research funding for rare diseases, many patient groups try to raise money themselves, conducting car washes, bake sales and garage sales. Often, it takes many years for these small patient groups to raise enough money to fund a study.

At a recent meeting of NORD's medical advisers, Dr. Zallen writes, "we struggled to decide which of two excellent research grant submissions should receive the modest available funding. Even with all its hard work, the patient group providing the funding had raised only enough money for one seed grant."

People with rare diseases are not asking for "preference" when it comes to allocation of resources but only a "fair shake", Dr. Zallen adds. "The tax dollars used for government research programs come out of their pockets too, and society has an ethical duty not to punish minorities because of their small size. Not only they, but the wider community as well, would benefit -- since such research is essential to the health of the overall medical science enterprise."

Dr. Zallen has received numerous awards and honors for her teaching, research and outreach activities. An expert on topics related to bioethics, she helped write the federal guidelines to protect human subjects in gene therapy experiments and is the author of the book, *Does It Run in the Family? A Consumer's Guide to DNA Testing for Genetic Disorders*.

The full text of [Dr. Zallen's response](#) and a link to the [original article](#) are available on the NORD website ([www.rarediseases.org](http://www.rarediseases.org)).

Established in 1983, NORD is a nonprofit organization representing all patients and families affected by rare diseases and all seeking to help them. It provides programs of advocacy, education, research and patient services.

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