



Pulmonary Hypertension Association
Challenges and Opportunities for NIH
12/31/2008

Thank you for this opportunity to offer the perspective of the pulmonary hypertension community on some of the challenges and opportunities facing the National Institutes of Health and new leaders in the nation's health policy initiatives. Similarly to some previously interviewed organizations, the Pulmonary Hypertension Association strongly supports the President-elect's plan to double the NIH budget in the next ten years. In addition, we encourage health policy leaders to look beyond common, widely publicized conditions to the 25 million Americans struggling with rare disorders, particularly when research into these rare diseases presents opportunity for progress in multiple health fields.

Pulmonary Hypertension and the Pulmonary Hypertension Association

Pulmonary hypertension (PH) is high blood pressure between the heart and the lungs. It is deadly and currently incurable but increasingly treatable. PH can occur without a known cause or secondary to other diseases including sickle cell, lupus, scleroderma and HIV. Recent NIH research suggests that as many as 40% of sickle cell patients develop moderate to severe PH.

Symptoms of PH include shortness of breath, dizziness and bluish lips and skin. Since they are similar to the symptoms of asthma and other less fatal conditions, PH is often misdiagnosed. Approximately 30,000 people in the U.S. are currently diagnosed with PH. This is up from 3,000 in 2001, but many times this number remain misdiagnosed or undiagnosed.

Until 1994, there were no treatments specific to PH. Now, there are six FDA approved treatments, but a single PH therapy can cost as much as \$200,000 a year. In addition, due in part to a shortage of relevant research, patients face significant challenges receiving insurance coverage for the therapy combinations and dosage levels most often prescribed by physicians.

Twenty years ago, when three patients who were searching to end their own isolation founded the Pulmonary Hypertension Association (PHA), there were less than 200 diagnosed cases of this disease. It was virtually unknown among the general population and not well known in the medical community. They soon realized that this was unacceptable, and formally established PHA.

Today, PHA includes:

- over 9,500 patients, family members and medical professionals as members and an additional 34,000 supporters and friends;
- three research programs that, through partnerships with the National Heart Lung and Blood Institute and the American Thoracic Society, have directed more than \$6 million toward PH research;
- the first medical journal devoted to pulmonary hypertension, which is published quarterly and distributed to all cardiologists, pulmonologists and rheumatologists in the U.S.

**The NIH Budget: Investing in Our Future**

I was diagnosed with pulmonary hypertension in 2003. Thanks to new research and medicine I have been able to return to an active and fulfilling lifestyle. I now attend the University of Maryland at Baltimore, studying to be a nurse! Everything is more of a challenge and takes more planning because of all my medical needs, but I am persevering in hope and in faith that new discoveries are on the horizon for patients like me. I am literally counting on new research to save my life!

-Donna Pederson

Recently, PHA President, Rino Aldrighetti, had the opportunity to speak during a series of medical education sessions presented by the Pulmonary Hypertension Association of China. While in China, Rino sat near a professor and researcher at National Taiwan University. The professor mentioned that he used to work in the U.S. In fact, he had been an oncology researcher with the NIH. When Rino asked why he left, he replied that a lack of financial support for his work caused him to move to China, where research funding remains more plentiful.

Since PHA was incorporated, we have watched the NIH payline drop from 32% to 13%. This lack of investment stalls potentially lifesaving research. In addition, it creates long-term damage to the public research infrastructure of the United States. Intelligent, motivated students see little opportunity in medical research and pursue other career paths, or take their talent abroad to more welcoming environments. Significant, ongoing increases to the NIH budget are critical to the well being of the nation.

Rare Disease Research: Impact Beyond Numbers

I study what is happening in pulmonary hypertension as an example of what you can do with an orphan disease. With the combination of advocacy, industry involvement and state-of-the-art basic science, they came together in a perfect storm of opportunity.

-Dr. Mark Gladwin, National Heart, Lung and Blood Institute
Witness at a congressional hearing on pulmonary hypertension

A rare or “orphan” disease is one that is known to impact fewer than 200,000 people in the United States. There are more than 6,000 rare disorders that, taken together, affect approximately 25 million Americans. Investing in rare disease research means acknowledging that individuals with rare diseases are just as important, just as valuable, as those with more common, well known conditions.

Investment in rare disease research is the right thing to do. It also makes sense. Often, as is the case with pulmonary hypertension, rare disease research presents a “perfect storm” of talent and opportunity that, if nurtured, will have a broad impact on medical knowledge. Recent collaboration in the fields of pulmonary hypertension and sickle cell disease has allowed an increased understanding of both diseases. Health policy leaders must look beyond high profile disorders to the power of ongoing investment in rare disease research.

The pulmonary hypertension community looks for to working closely with the new administration. More information is available at www.PHAssociation.org. Rino Aldrighetti, PHA President, can be reached at 301-565-3004 x741.