

## Powerful Patient #2

Host: Joyce Graff, WebTalkRadio.net

### Orphan Drugs

Orphan Drugs are defined as drugs with a limited market, serving people with rare disorders.

Rare disorders are defined as those that affect fewer than 200,000 Americans (with nearly 300 million citizens, that amounts to less than one-tenth of one percent of the population).

Drug companies base their investment decisions on an expectation of return – if they invest a certain amount of money, can they make it back within a reasonable amount of time and then go on to make a profit?

The Orphan Drug Act was passed to give drug companies some additional protections and incentives to make it more affordable to develop and manufacture drugs that serve people with rare diseases. Without these financial incentives, drugs for many conditions would not exist.

#### 1) Our Guests

**Abbey Meyers**, President of the National Organization for Rare Disorders (NORD) was the driving force behind the passage of the U.S. Orphan Drug Act in 1982. She tells the story of how she came to be involved in this effort, and how a “housewife from Connecticut” waged a campaign to get help for her son, that became a global effort to help all people with all rare diseases.

**Mary Nathan**, a patient and advocate in the Gaucher disease community, is continuing to build upon the work Abbey began in Washington, working to educate members of Congress and their aides on the importance on building upon this legislation. As drug development research becomes increasingly specific not only to a single disease but to multiple sub-divisions of diseases, we are moving into a time when drugs will be tailored to an individual’s genetic make-up, making each one an “orphan” disease, custom designed for an individual. The economics of making this affordable are daunting.

**Dr. Pramod Mistry** of Yale University, one of the world's leading experts in Gaucher Disease. It was delightful to speak with this caring and knowledgeable physician.



Abbey Meyers, President  
National Organization for Rare  
Disorders (NORD)

## 2) References

### *On Rare Diseases*

- National Organization for Rare Disorders (NORD) <http://www.rarediseases.org>

### *On the Orphan Drug Act*

- The wording of the Act itself, as amended <http://www.fda.gov/orphan/oda.htm>
- The U.S. Office of Orphan Drug Development <http://www.fda.gov/orphan/index.htm>

### *On Gaucher Disease*

- Mary Nathan and drug development <http://www.hdfoundation.org/news/20030225-NYT-arms.htm>
- Dr. Mistry's page <http://info.med.yale.edu/pediat/faculty/mistry.html>
- Gaucher Disease – U.S. National Institute of Neurological Disorders and Stroke <http://www.ninds.nih.gov/disorders/gauchers/gauchers.htm>
- National Gaucher Foundation <http://www.gaucherdisease.org/>