

Stimulating Awareness and Research on Rare Diseases and Orphan Products Through the Media

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The Importance of Public Awareness

- **New patients get diagnosed.**
- **Public becomes familiar with the disease.**
- **Fundraising.**
- **Doctors learn about the disorder.**
- **Researchers may become interested.**
- **Diagnosed patients are helped.**

Barriers to Rare Disease Patient Awareness

- **Difficult to compete with more familiar diseases for the attention of the media.**
- **Complicated medical names that the public cannot pronounce or spell (e.g., epidermolysis bullosa, agammaglobulinemia, dysautonomia, ectodermal dysplasia, ichthyosis, etc.**

Barriers to Rare Disease

Patient Awareness

(Continued)

- **Getting volunteers to contact reporters.**
- **Finding families willing to tell their stories.**

Some Rare Diseases are Well-Known

U.S. Population

Disorder

Reason:

25,000-30,000

Cystic Fibrosis

(Local fundraising)

250,000-350,000

Multiple Sclerosis

(Local fundraising walkathons)

Some Rare Diseases are Well-Known (Continued)

U.S. Population/

Disorder

Reason:

10,000-15,000

Hemophilia

(Russian Tsar's family; transmission of HIV through blood transfusions)

10,000

Duchenne Muscular
Dystrophy

(Fundraising telethons)

Some Rare Diseases are Well-Known (Continued)

U.S. Population/

Disorder

Reason:

100,000

**Severe Combined
Immune Deficiency
(SCID)**

(Movie: Bubble Boy Disease)

25,000

**Amyotrophic Lateral
Sclerosis (ALS)**

(Lou Gehrig's Disease (sport hero))

Some Rare Diseases are Well-Known (Continued)

U.S. Population/

Disorder

Reason:

100,000

Tourette Syndrome

(TV Shows and Jokes)

100,000

Neurofibromatosis

(Elephant Man Disease)

Lessons Learned

- **Local fundraising events will educate people about rare diseases.**
- **Identifying a disease with famous people is a good way to promote your disorder (e.g, movie stars, sports figures, famous historic people, etc.).**

Lessons Learned

(Continued)

- **Getting your disease in a book or movie guarantees immortality for your message.**
- **Even negative or inaccurate publicity stays in the public mind and gives you an opportunity to get more publicity.**

Lessons Learned

(Continued)

- **Repetition is important:**

If the muscular dystrophy telethon stops this year, in 5 or 10 years, no one will know what muscular dystrophy is.

Attracting Researchers

- **Use your Medical Advisory Committee to reach researchers and clinicians.**
- **Exhibit at various medical conventions.**
- **Physicians listen to physicians.**

Attracting Researchers

(Continued)

- **Ask your Medical Advisory Committee (MAC) members to make presentations at medical conventions, submit articles and letters to the editor of major medical journals, seek out researchers from varied disciplines for membership on your MAC, do Grand Rounds at major academic hospitals, etc.**

Attracting Drug Companies

- **Understand the pharmaceutical industry before you approach companies.**
- **Research the type of company you should approach: If you represent a neurological disease, make sure the company has a program for development of neurological drugs.**

Attracting Drug Companies

(Continued)

- **Drug companies do not do research on diseases. They research compounds and then decide which disease it will work on.**
- **If an academic researcher discovers a treatment, don't try to find a company to adopt it unless the researcher asks. He/she may be negotiating with a company secretly.**

You Must Have a Story to Tell

- **If you know that a major journal article will be published about your disease soon, contact “health reporters” in advance.**
- **Make your organization the expert consumer group that reporters can go to when they want quotes.**

You Must Have a Story to Tell

(Continued)

- **Locate health reporters at major newspapers and magazines (hint: Amy Marcus and Geeta Anand at *Wall Street Journal*) and keep the list up-to-date for your press releases.**

You Must Have a Story to Tell

(Continued)

- **Locate individuals and families who are willing to tell their stories to the press.**
- **Tell your chapters/local support groups to encourage their families to tell their stories to local press.**

The Ultimate Public Relations Story

- **Adam Seligman's drug was confiscated by U.S. Customs because it was not approved in the USA.**
- **His mother called her Congressman.**
- **The Congressman held hearings.**
- **Reporter from the *Los Angeles Times* attended the hearing and wrote a story.**

The Ultimate Public Relations Story

(Continued)

- **Jack Klugman's brother read the story and convinced Jack to do an episode of Quincy about the orphan drug problem.**
- **Public pressure resulting from the program compelled passage of the *Orphan Drug Act*.**

Lessons Learned

- **The public cares about disadvantaged people.**
- **It is the responsibility of rare disease support groups to focus public attention on your disease.**
- **You must have a story to tell.**

Lessons Learned

(Continued)

- **Major news reporters want stories that touch on broad healthcare issues such as the uninsured, why our healthcare system needs reform, access to health insurance, prescription drugs, etc.**

Lessons Learned

(Continued)

- **In Europe, follow the problems of drug reimbursement in each country.**
- **Find a sports hero or movie star!**
Good luck!