FasterCures Webinar Series presents

Engaging with FDA:
A Guide for Foundation Funders of Research

April 18, 2012

Moderated by
Margaret Anderson
Executive Director, FasterCures
Engaging with FDA: A Guide for Foundation Funders of Research

Panelists

Mary B. Dwight
Vice President, Government Affairs
Cystic Fibrosis Foundation

Cynthia Rice
Vice President, Government Relations
JDRF

Moderator

Margaret Anderson
Executive Director
FasterCures
Back to Basics:
HIV/AIDS Advocacy As a Model for Catalyzing Change

Elements of the Model:
• Attention
• **Knowledge and solutions**
• Community
• Accountability
• Leadership

“It wasn’t that they were simply advocates. It was that they really were contributors and that they really brought a very sophisticated understanding.”
– Margaret Hamburg, MD
A little background on the FDA

- 12,000 employees in more than 200 offices and 13 labs in 50 states
- Regulates more than $1 trillion of consumer goods, 25% of all consumer spending
  - Drugs, vaccines, biological products, and medical devices
  - But also food, cosmetics, pet food, cell phones (radiation)
- Responsible for monitoring a third of all imports
- 25 years ago, FDA and CDC were the same size; today the CDC budget is nearly three times as large
- FDA’s appropriation is almost entirely staff costs, requiring a nearly 6% increase each year to sustain program levels
- Today’s investment: 2 cents per day per American
Why should patient foundations think about engaging with the FDA?

- This is **not** necessarily about advocating that FDA approve a particular product
- This is **not just** about bringing your passion as patients to the table
- This is **is** about bringing to the process your expertise in the disease, your access to patients and physicians/researchers, and your ability to fund research into key questions
What have we been able to achieve?

- CF severity and demographics
- Defining endpoints
- Artificial Pancreas Project
Many manifestations of CF

**Sinopulmonary**
Thick, sticky mucus clogs the lungs and sinuses leading to serious, life-threatening infections; problems clearing airways

**Pancreatic & Hepatic**
Mucus clogs the pancreas, stopping natural enzymes from breaking down food and nutrients; poor weight gain; malnutrition

**Endocrine**
Insulin resistance, diabetes

**Reproductive**
Reduced fertility

**Salt Balance**
High levels of salt in sweat

**Others**
Clubbing of fingers/toes, metabolic alkalosis
Provided Genetic Mutation Data

All Therapeutic Classes:
- Patient Reported Outcomes
- Defining Pulmonary Exacerbations
“The unique and mutually beneficial partnership that led to the approval of Kalydeco serves as a great model for what companies and patient groups can achieve if they collaborate on drug development.”

-Dr. Margaret A. Hamburg, Commissioner, U.S. Food and Drug Administration
Artificial Pancreas a Top Priority for JDRF to Better Treat Type 1 Diabetes
Some Highlights of JDRF-FDA Actions

- Early collaboration around academic studies – progress and also challenges
- By 2010, needed to define pathway for outpatient studies and product testing
- Decided to convene expert clinical panel, propose guidance to FDA
- Engaged agency at all levels, worked with allies in clinical community, Congress
- Public advocacy campaign underscored need for action
Exciting Results

- FDA released draft artificial pancreas guidance in December 2011 – just 9 months after JDRF proposal
- Specific suggestions made by JDRF and clinical allies seem to have been incorporated
- FDA seems open to comments to draft
- And – in March – FDA approved first outpatient academic study
Getting started

When and why did we think about working more closely with FDA?

What were our goals?

What were our expectations?

How did we prepare?
Challenges at FDA

What were some of the obstacles and challenges we encountered?

How did we address them?
What you need to know about yourself

✓ Know what your goal is

✓ Know what you bring to the table
  • As a nonprofit foundation, distinct from an industry sponsor
  • As a patient representative, distinct from academic scientists
  • As much as you can, bring the community to FDA under your umbrella

✓ Know what your internal rules of engagement are
  • What is your relationship with industry trial sponsors?
  • Are you comfortable advocating for a specific product? How do you gauge when it’s appropriate?
What you need to know about FDA

✓ Know who you need to deal with at FDA and how they work

✓ Know where the gray areas are
  • FDA is a complex decisionmaking organization
  • There are not clearly prescribed ways for FDA to interact with patient groups
  • There are not clearly prescribed ways for FDA to interact with external groups about things that are not products
  • Different centers within FDA behave in different ways

✓ Know when to hold’em – and when to fold’em
  • What FDA can do and can’t do
  • When is it helpful to meet – and when is it not helpful
Q&A

Panelists

Mary B. Dwight
Vice President, Government Affairs
Cystic Fibrosis Foundation

Cynthia Rice
Vice President, Government Relations
JDRF

Moderator

Margaret Anderson
Executive Director
FasterCures
View an archive of this Webinar
www.fastercures.org/train
• PARTICIPATE in outcomes-oriented dialogue on solving the challenges that slow medical progress
• DISCOVER new and scalable models for improving research efficacy and efficiency
• PARTNER with others who share your goals, and could advance your programs.
• CONSULT with strategy and regulatory experts onsite, for FREE, on tailored advice on your initiatives
• PRESENT your transformative multi-sector partnership/project to potential collaborators and supporters
@fastercures

follow, like, read, add, link, watch

connect

FasterCures