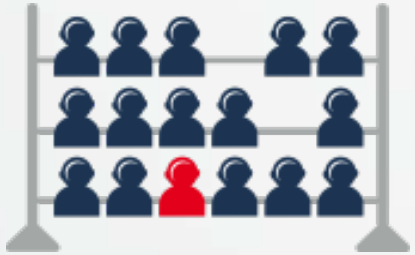

EVERYLIFE FOUNDATION FOR RARE DISEASES

@EveryLifeOrg

2018 Scientific Workshop #10

Conceptualizing an FDA Rare Disease Center of Excellence

Presented by

PhRMA

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EVERYLIFE FOUNDATION FOR RARE DISEASES

Thank you to today's sponsors!

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Mission and Core Principles

EVERYLIFE FOUNDATION FOR RARE DISEASES

Accelerating biotech innovation through science-driven public policy

What We Believe:

- No disease is too rare to deserve treatment
- Rare disease therapies should be safe and effective
- We could do more with the science we already have

What We Do:

- Advocate for evidence-based changes in public policy, development strategies & regulatory review

How We Get it Done:

- Grassroots action
- Scientific and policy expertise

Our Board of Directors

- **Chair, Mark Dant, Executive Director, Ryan Foundation**
- **Vice-Chair, Frank Sasinowski, Director, Hyman, Phelps & McNamara, P.C.**
- Secretary Julia Jenkins, Executive Director, EveryLife Foundation
- Treasurer, Vicki Seyfert-Margolis, PhD, Founder and CEO MyOwnMed
- Founder, Emil D. Kakkis, MD, PhD, President/CEO, Ultragenyx
- Ritu Baral, Managing Director/Senior Biotechnology Analyst, Cowen & Company

Our Team

Julia Jenkins, Executive Director

John Lally, Director of Operations

Lauren Grinnals, Rare Hub Manager

Christina Hartman, Senior Director, Policy & Advocacy

Vacant, Director of Policy

Lindsey Cundiff, Associate Director of Patient Engagement

Shannon von Felden, RDLA Program Manager

Sarah Gelbard, Newborn Screening Fellow

Carol Kennedy, Chief Development Officer

Ted Brasfield, Director Development

Vacant, Senior Director, Marketing & Communications

Grant Kerber, Deputy Director Communications

Erin Garcia, Special Events Manager

Lisa Schill, Event Development Consultant

Foundation Address

After nine years in California the Foundation has moved its headquarters to Washington, DC!

- **Temporary Address:**
  - WeWork White House: 1440 G St NW, Washington, DC
  - Mailing: P.0. Box 77210, Washington DC 20013
- Phone Number: 202-697-RARE (7273)
- In October the Foundation will open a Rare Hub at 1012 14th Street NW • Suite 500 • Washington, DC

RAREHUB

WASHINGTON D.C.

No Disease Is Too Rare to Deserve Treatment

Public Policy Objectives

The Foundation seeks practical policy solutions:

- Close the innovation gap for the 95% of rare diseases that have no FDA-approved treatment
  - Support initiatives and new technologies that foster novel and innovative treatment
- Ensure patients receive earliest access to diagnostic and treatment opportunities
- **Improve the development/regulatory process and advance regulatory science for rare disease therapies**
- Enhance the patient voice in policymaking, drug development and regulatory decision-making

Policy and Advocacy Initiatives

EVERYLIFE FOUNDATION FOR RARE DISEASES

RDLA Rare Disease Legislative Advocates

Rare Disease Legislative Advocates clearinghouse to train patients and parents on how to be effective in changing policy

NEWBORN SCREENING

Expanding Newborn Screening
state legislation to require a state to screen for a disease once it's on the federal RUSP

COMMUNITY CONGRESS

Community Congress
Collaboration between patient organizations and industry representatives to seek policy solutions

OPEN ACT Rx HR 971 S 1421
Orphan Product Extensions Now – Accelerating Cures & Treatments

Incentivizing Rare Repurposing
federal legislation to double the number of rare disease therapies approved by FDA

No Disease Is Too Rare to Deserve Treatment

Workshop Series Topics

❑ Workshop #1	Statistical Analyses of Rare Disease Studies
❑ Workshop #2	Clinical Evaluation of Rare Disease Treatments
❑ Workshop #3	Surrogate Endpoints and Accelerated Approval
❑ Workshop #4	Developing Policy Recommendations for Accelerated Approval
❑ Workshop #5	Accelerated Approval in Rare Disease: Review of a White Paper Proposal
❑ Workshop #6	Rationalizing Safety Testing to Enable Clinical Studies and Approval in the US for Rare Disease Treatments
❑ Workshop #7	Incorporating the Patient Perspective in Rare Disease Drug Development
❑ Workshop #8	Evaluating Early Access Models for Patients: Flashpoints, Frameworks and Case Studies for Advancement
❑ Workshop #9	Emerging Technologies for Rare Diseases: Clinical and Regulatory Case Studies and Approval Pathways

Slides from workshops are posted at www.rareworkshop.org

Improving the Specialization of Drug Review

EVERYLIFE FOUNDATION FOR RARE DISEASES

In 2009 the Foundation launched the CureTheProcess Campaign to create a specialized review division for complex rare metabolic diseases

- **Create a new ODE unit with three smaller, more specialized review divisions**
  - Hire more specialized reviewers
  - More division heads trained in the relevant field of medicine and more involved in reviews
- **Improve workload conditions and academic opportunities**
  - **Fewer applications per division**, and more time for deeper review and thought on complexities
  - **Joint academic appointments at NIH** Allow time and resources for division heads and team leaders to conduct research, perform clinical work and attend or participate in scientific conferences in their topic area

An example for organizational changes to help specialization: Current structure

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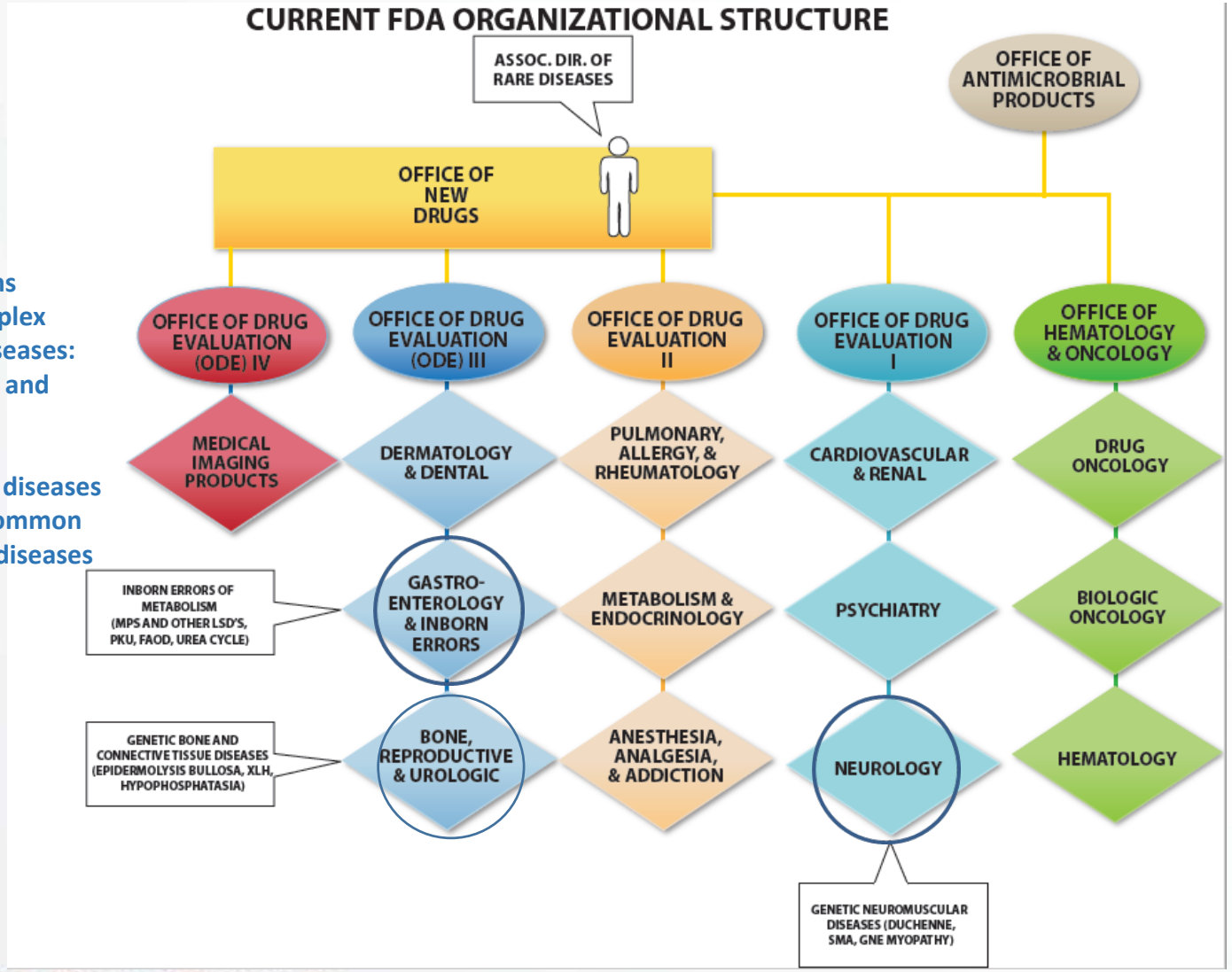
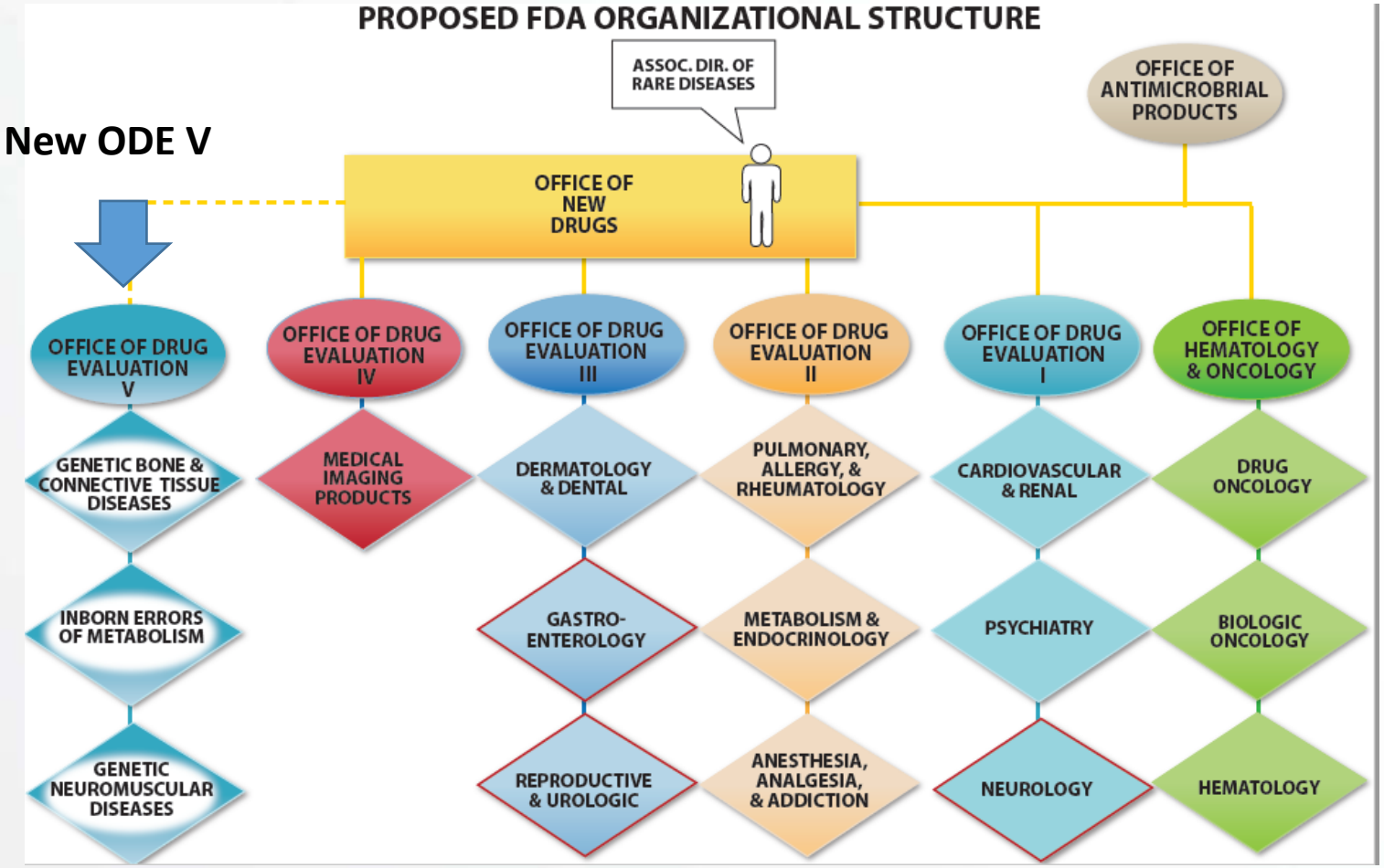
Three divisions evaluate complex mixture of diseases: DGIEP, DRUB, and Neurology.

Complex rare diseases mixed with common large market diseases



The above example based on an earlier review division organization which has changed since created

One new ODE V to operate with specialized staff and focused review scope



FDA to create more “focused” review divisions

EVERYLIFE FOUNDATION FOR RARE DISEASES

PREVISIONPOLICY

April 27, 2018

FDA Office of New Drugs Reorg Will Create More Review Divisions; Create Openings For New Generation Managers; Unsettle Staff

The Food & Drug Administration is expected to increase the number of offices and review divisions in the crucial new drug review operations of the agency as part of the planned reorganization of the Office of New Drugs.

The “modernization” of the new drug review operations is still very much in the planning stages, but the eventual reorg chart will most likely add one additional Office of Drug Evaluation to the six existing ODEs, and expand the current 19 review divisions to a number closer to two dozen. While the timing is still uncertain, the new structure could be announced internally as early as this summer.

Community Congress Update & Overview of the Day

Christina Hartman

Senior Director, Policy & Advocacy

EveryLife Foundation for Rare Diseases

Collaboration is Key!

COMMUNITY CONGRESS

We bring patient organizations, industry leaders, and other rare disease stakeholder organizations together to provide valuable insight on prioritizing our future policy initiatives.

Patient Organizations & Industry Partners have an equal seat at the table working together on shared goals!

Working Group Membership

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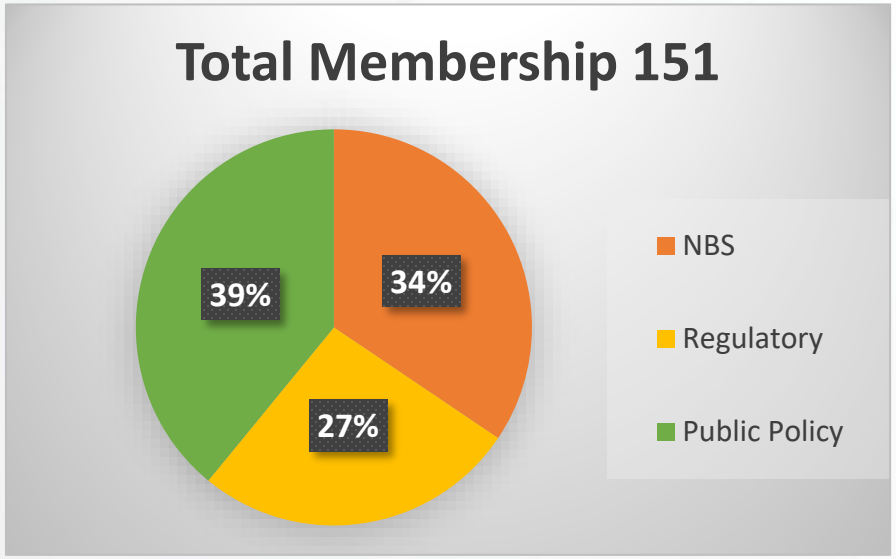

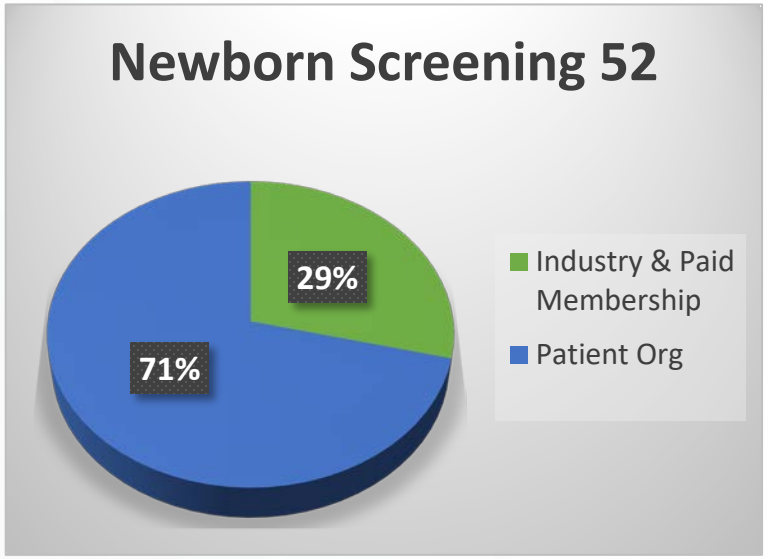

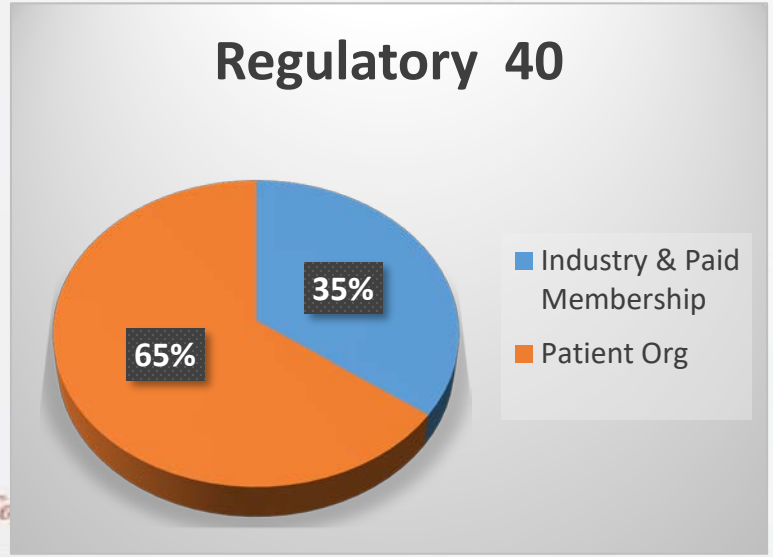

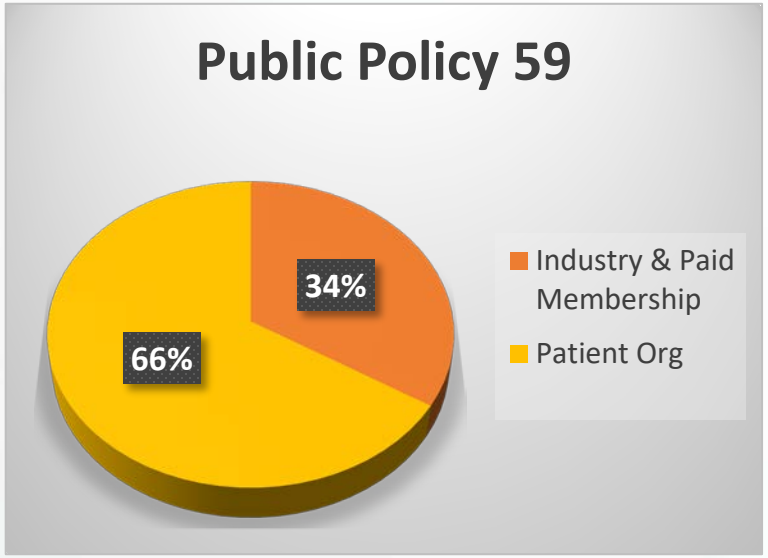

Regulatory Working Group

Isabelle Lousada
President & CEO
Amyloidosis Research Consortium

Lisa Carlton
Senior Director
Regulatory Affairs
RegenxBio

Goals of Working Group

- To partner with advocacy groups to facilitate understanding of regulatory issues
  - Understanding regulatory pathways for rare diseases
  - Expanded access regulations for unapproved drugs
  - Role of patients in drug development
  - Role of advocates at FDA meetings and advisory committees
  - Facilitate input into relevant guidance

Preliminary Survey Results

- Goal was to understand real and perceived challenges of both industry & patient community
- 32 respondents total: 21 patient organizations and 11 companies
- Just under 50 percent of respondents have worked with FDA on a specific product (6 patient organizations and 8 companies)
- Of those 14 products, 10 have been approved

Preliminary Survey Results

- Wide variation in level and type of engagement with the FDA
- 1/3 of survey respondents were unaware of Commissioner Gottlieb's proposed reorganization to modernize the FDA
- Over ½ of respondents were unsure whether proposed FDA reorganization would expedite approval for rare products

Preliminary Survey Results

- Over 90 percent of respondents thought an FDA Rare Center of Excellence would help the FDA
  - Understand the challenges of clinical trial design for rare diseases
  - Improve FDA staff expertise in rare diseases
- Will perform deeper dive of roadblocks identified by survey respondents
- Over 40 percent of respondents though FDA's PFDD efforts were improving drug development for rare diseases
- 32 percent of respondents (majority) thought Rare Disease CEO was no. 1 thing that could improve rare disease drug development

Today's Agenda

EVERYLIFE FOUNDATION FOR RARE DISEASES

8:30 am	Welcome & Overview
9:20 am	Perspectives on Progress at the FDA
10:35 am	Coffee Break
10:50 am	Continued Challenges at the FDA: Patient & Academic Perspectives
12:05 pm	Lunch
1:00 pm	Continued Challenges at the FDA: Industry Examples
2:30 pm	Break
2:45 pm	FDA Center of Excellence for Rare Diseases Potential Model & Panel Discussion
4:00 pm	Final Discussion & Thoughts on Next Steps
4:30 pm	Adjourn

No Disease Is Too Rare to Deserve Treatment

FDA Rare Disease Center of Excellence

EveryLife Foundation for Rare Diseases

- ✓Pass Legislation to Authorize Creation
- ✓Build Community Support
- ❑**Engage KOLs, experts and FDA on development**
- ❑Start with small and simple, avoid creating bureaucracy
- ❑Engage Congress for additional FDA funding

Discussion & Outcomes

EVERYLIFE FOUNDATION FOR RARE DISEASES

- How do we leverage the expertise across FDA review divisions to:
  - Harmonize regulatory approaches
  - Handle complexities of clinical trial designs
  - Establish endpoints for small heterogeneous patient populations
- How can we increase collaboration with international regulatory agencies to allow for clinical trial designs to be accepted across multiple agencies?
- What can be learned from the successes and challenges of the FDA Oncology Center of Excellence?
- ✓ **Goal is to publish a white paper outlining how a Rare Disease COE would be established at FDA**