Rare Disease PFDD Compendium Project

Overview & Orientation to the Guide

January 27, 2022

The Rare Disease PFDD Guidance Compendium

Workshop Series

TODAY'S AGENDA

TOPIC	SPEAKER
Overview of the Rare Disease PFDD Guidance Compendium Project	Annie Kennedy – EveryLife Foundation
Perspectives on the Need for and Benefit of the Initiative & Guide	Ryan Fischer – PPMD Cara O'Neill – Cure Sanfilippo Foundation Kristin Van Goor – Vertex
Orientation to the Guide	Kim McCleary – The Kith Collective
Discussion of Resources	Annie Kennedy Eric Gascho – National Health Council
Wrap-Up	Annie Kennedy

The Rare Disease PFDD Guidance Compendium

Workshop Series

INTRODUCTION

- Multi-stakeholder, collaborative effort designed to inform the understanding of how patient-focused medical product development and related FDA guidances can be applied to development of therapeutics for rare diseases

- Series of four virtual workshops of PFDD leaders to consider the application of FDA guidances across four lifecycle stages of medical product development to identify challenges and opportunities specific to rare diseases

- Develop an accessible Guide for use by all stakeholders involved in the rare disease therapeutic development ecosystem to embed the perspectives of the people living with a rare disease in the development of therapeutics.

The Rare Disease PFDD Guidance Compendium

Workshop Series

Project Leadership

Annie Kennedy, EveryLife Foundation
Victoria Dohnal, BIO (Aug. 10, 2021 – Nov 2021)
Kate Donigan, BIO (beginning Dec 2021)
Eric Gascho, National Health Council
Danielle Friend, BIO (through Aug. 7, 2021)
Maria Apostolaros, PhRMA
Christine Harhaj, PhRMA
Kim McCleary, Kith Collective
Samantha Mayberry, Kith Collective

Project Steering Committee

Jennifer Bright, The Innovation & Value Initiative
Nimi Chhina, BioMarin
Ryan Fischer, PPMD
Emily Freeman, Lundbeck
Danielle Friend, J&J
Lauren Hetrick, AbbVie
Paul Howard, Amicus Therapeutics
Jill Jarecki, CureSMA
Alexis Miller, Merck
Steve Morin, Merck
Amy Nicole Nayar, Novartis
Cara O'Neill, Cure Sanfilippo Foundation
Samantha Roberts, Genentech
Sean Tunis, Rubix Health
Pujita Vaidya, Amgen
Kristin Van Goor, Vertex

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Workshop Series

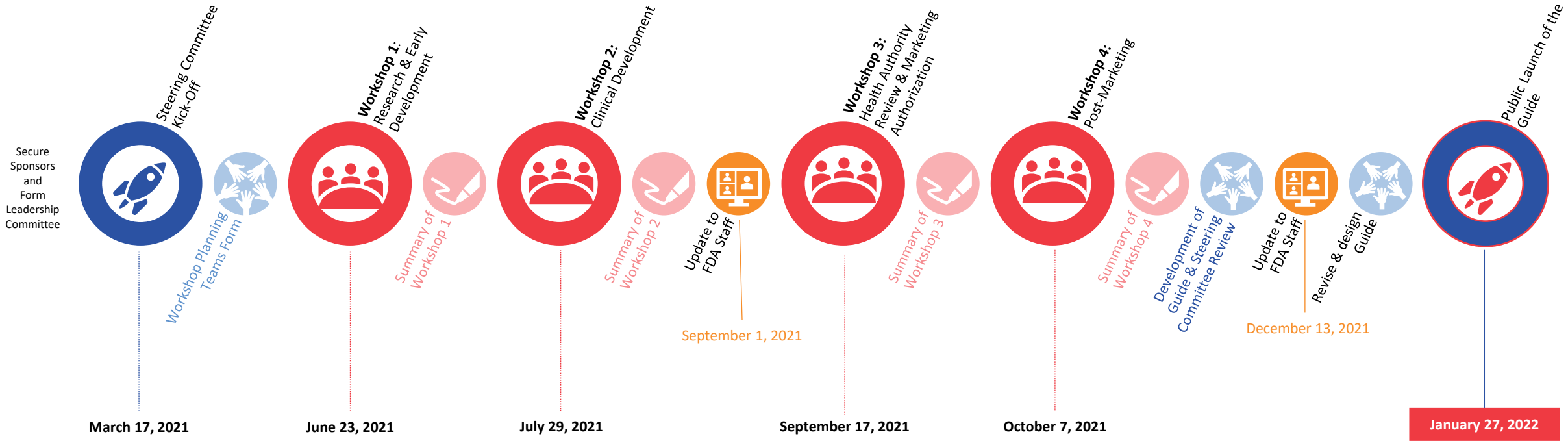
1. June 23, 2021: **Research and Early Development**
2. July 29, 2021: **Clinical Development**
3. Sept. 17, 2021: **Health Authority Review & Marketing Authorization**
4. Oct. 7, 2021: **Post-Marketing**

HOSTED BY: EveryLife Foundation for Rare Diseases; Bio Biotechnology Innovation Organization; National Health Council; PhRMA Research · Progress · Hope

BY THE NUMBERS

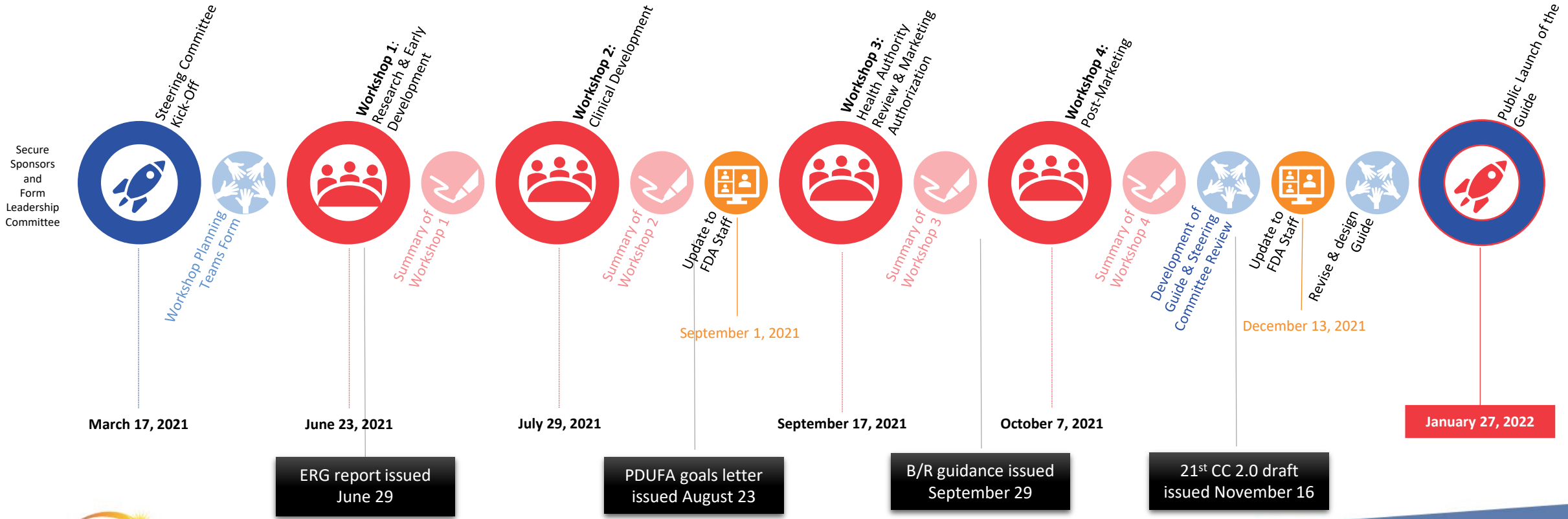
- **23** Leadership and Steering Committee Members
- **88** TOTAL Subject Matter Experts
  - 8 Academia
  - 40 Industry
  - 28 Patient Advocacy Organizations
  - 5 Payer Organizations
  - 7 Other
- **20** FDA Guidances
- **12** Hours of Workshops
- **8** Cross-Cutting Topics and Sets of Action Steps
- **4** Workshop Summaries
- **112** Resources Linked in This Guide

PLANNING & EXECUTION



PLANNING & EXECUTION

Responding to a dynamic environment



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Perspectives on the Need for and Benefit of the Initiative & Guide

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The Rare Disease PFDD Guidance Compendium

Workshop Series

Orientation to the Guide to Patient Involvement in Rare Disease Therapy Development

HOSTED BY: EveryLife Foundation for Rare Diseases; Bio Biotechnology Innovation Organization; National Health Council; PhRMA Research · Progress · Hope

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CONTEXT & ORIENTATION

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8 FOCUS AREAS

EVERYLIFE FOUNDATION FOR RARE DISEASES

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Layout for each of the 8 focus areas

BUILDING RELATIONSHIPS THAT LAST

The expanding practice of patient-focused therapy development in rare disease has provided new and increased opportunities for patient advocacy organizations and sponsors to collaborate.

Yet, these relationships have unique characteristics and may be unfamiliar – if not uncomfortable – at least at first. One party or the other may have limited experience with the "world" that the other inhabits. After all, patient advocacy organizations and biopharmaceutical companies are very different entities, although both may be dedicated to improving and extending patients' lives. That shared goal is an important one and can be a touchstone to guide interactions.

Acknowledging challenges each may perceive or encounter in establishing and maintaining a productive relationship can help smooth the path. There might be concerns about conflicts of interest or not having adequate processes in place to interact smoothly. Patient advocacy organizations' hesitation may stem from a lack of understanding of the therapy development process and an appropriate way to inform it. They may also lack capacity or be wary based on prior experiences with industry where mutual benefit was not achieved.

Timing of outreach can be an issue for sponsors. They may be hesitant to engage patients early in product development to avoid raising premature hopes. They may be reluctant to approach an advocacy organization for the first time in later development stages of a program, fearing criticism for not having engaged sooner. There might also be concerns about perceptions of appearing promotional and the potential disclosure of sensitive information all along the way.

Actions for Patient Advocacy Leaders to Consider

GET THE LAY OF THE LAND

- Learn about the process of therapy development to become a knowledgeable partner. [#19] Establish relationships with experienced advocacy leaders and learn from them.
- Recognize that not every development program will succeed. Those that do may not gain approval (or access) at the same time in every country. Educate your community about the process and help manage expectations.
- Understand that you may not have access to as much information or as soon as you would like. FDA cannot disclose information about its confidential interactions with companies; however, companies can determine what and when to share, subject to regulations by the Securities Exchange Commission and other entities and according to internal policies.

PREPARE YOUR ORGANIZATION

- Inventory the assets your organization and community have to offer. [#28] Think about ways to help de-risk the intensive process of developing a new therapy for a rare disease.
- Work with your Board and other advisors to proactively develop guidelines that clarify the types of activities your organization will and will not engage in with sponsors. [#21-22] Define a process to manage requests.
- Map out PFDD-related roles appropriate for organization staff and how best to involve community members, including patients, caregivers and other family members. Building a roster of research-ready community participants may require up-front investment in planning, communication and training, yet it may also enhance interest in engaging patients.

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- If multiple companies are actively developing new therapies, consider hosting a pre-competitive forum to identify common challenges and shared solutions. [#23]
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There are many reasons to work together to form and foster productive, lasting relationships. They are the foundation for achieving the promise of patient-focused therapy development.

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- Educate legal and compliance staff about new expectations arising from patient-focused therapy development and work with them to establish internal processes to support meaningful interactions with patients and advocates.
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- Become familiar with fair market rates for compensating patients, caregivers and advocates who engage in research-related activities and strive to meet or exceed them. [#24]
- Identify internal silos that may inhibit cross-functional learning across the product life-cycle and plan to overcome them.

BEGIN EARLY AND STAY OPEN

- Identify when (at what stage) patient involvement is critical for your development program and engage patients early enough that patient involvement can inform thinking, planning and decisions and not be just "check a box" activity.
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Introduction to the topic as it relates to patient involvement

EVERYLIFE FOUNDATION FOR RARE DISEASES

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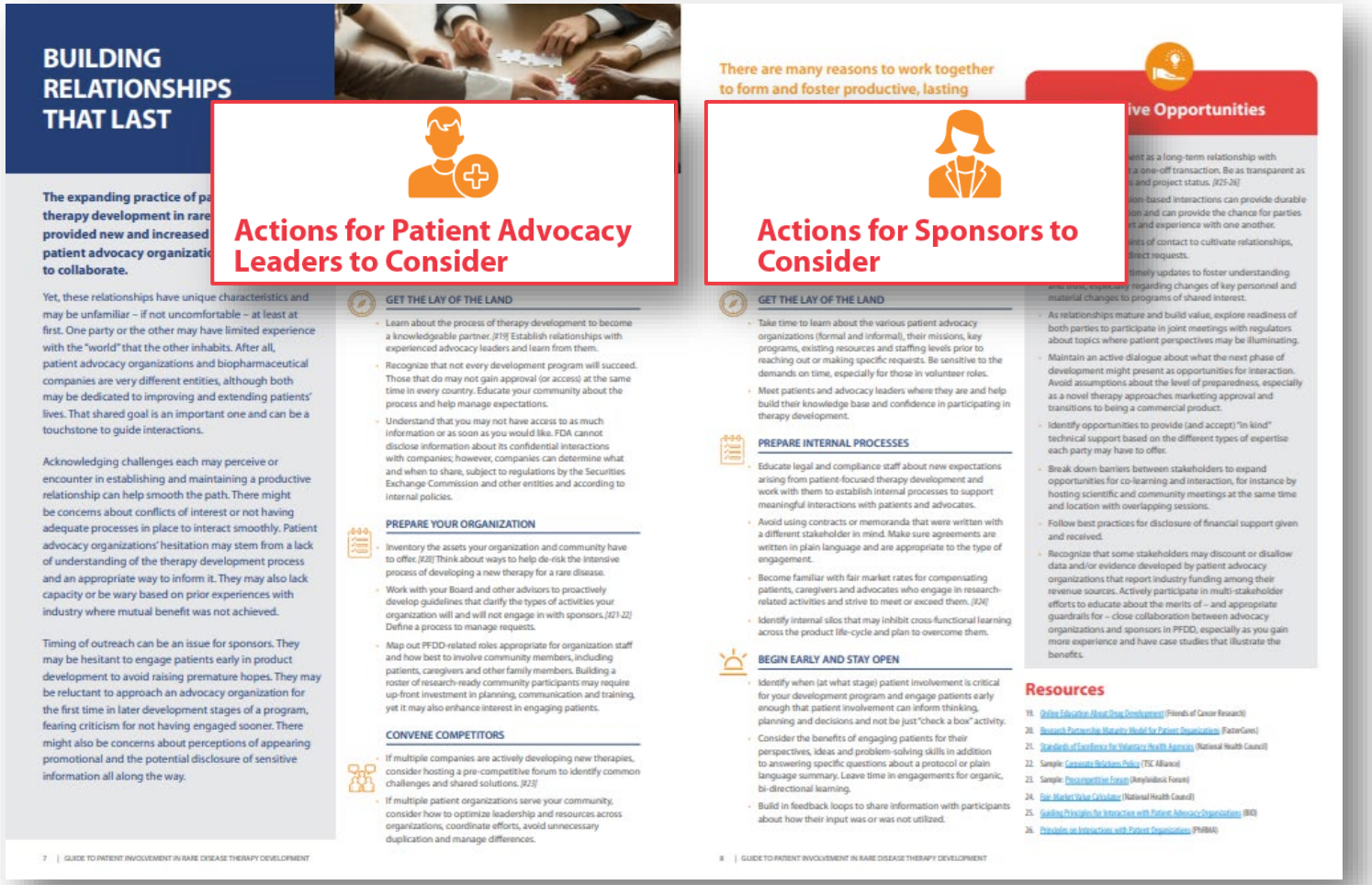
Guide To Patient Involvement In Rare Disease Therapy Development

A PUBLICATION OF THE RARE DISEASE PFDD COMPENDIUM WORKSHOP SERIES

HOSTED BY: EveryLife Foundation for Rare Diseases · Bio Biotechnology Innovation Organization · National Health Council · PhRMA

Layout for each of the 8 focus areas



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Actions for Spo… Consider

GET THE LAY OF THE LA…

PREPARE INTERNAL PRO…

BEGIN EARLY AND STAY…

Collective Opportunities

- Challenge the binary notion that "unmet need" relates solely to whether a rare disease has an approved therapy or not.
- Co-create patient journey maps that describe the path to diagnosis; common misdiagnoses, comorbidities and complications experienced; burdens arising from symptoms and current therapies/standard of care; challenges adjusting to life with a rare disease; progression and changes in function that may affect care decisions; and end-of-life issues.
- Collaborate to clarify the heterogeneity of unmet needs within a particular rare disease and how patient preference studies might be used to quantify various priorities and tradeoffs. *[#46]*
- Assess legacy measures through the lens of patient-defined unmet needs to identify whether novel measures may need to be developed to align with patient-centered benefit-risk assessment.
- As programs move forward, consider the unmet needs that may hamper access to a novel treatment such as the long diagnostic odyssey, absence of genetic test in newborn screening panels, lack of ICD code for the condition, underpowered registry/data collection efforts, limited understanding of the economic burden, etc. Wherever possible, work collaboratively to address these needs to reduce barriers to timely access to new products in the event marketing approval is granted. *[#47-49]*
- Upon approval of a new product, undertake a fresh look at unmet needs – including developing a new understanding of the different unmet needs that may present for treated patients versus those who cannot access treatment, those for whom the treatment is not safe and/or is not effective and those for whom the treatment is not appropriate or acceptable.

Opportunities for multiple stakeholders to collaborate

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7 | GUIDE TO PATIENT INVOLVEMENT IN RARE DISEASE THERAPY DEVELOPMENT

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8 | GUIDE TO PATIENT INVOLVEMENT IN RARE DISEASE THERAPY DEVELOPMENT

112 linked resources, referenced to help put in context

EveryLife Foundation for Rare Diseases

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WORKSHOP SUMMARIES

EVERYLIFE FOUNDATION FOR RARE DISEASES

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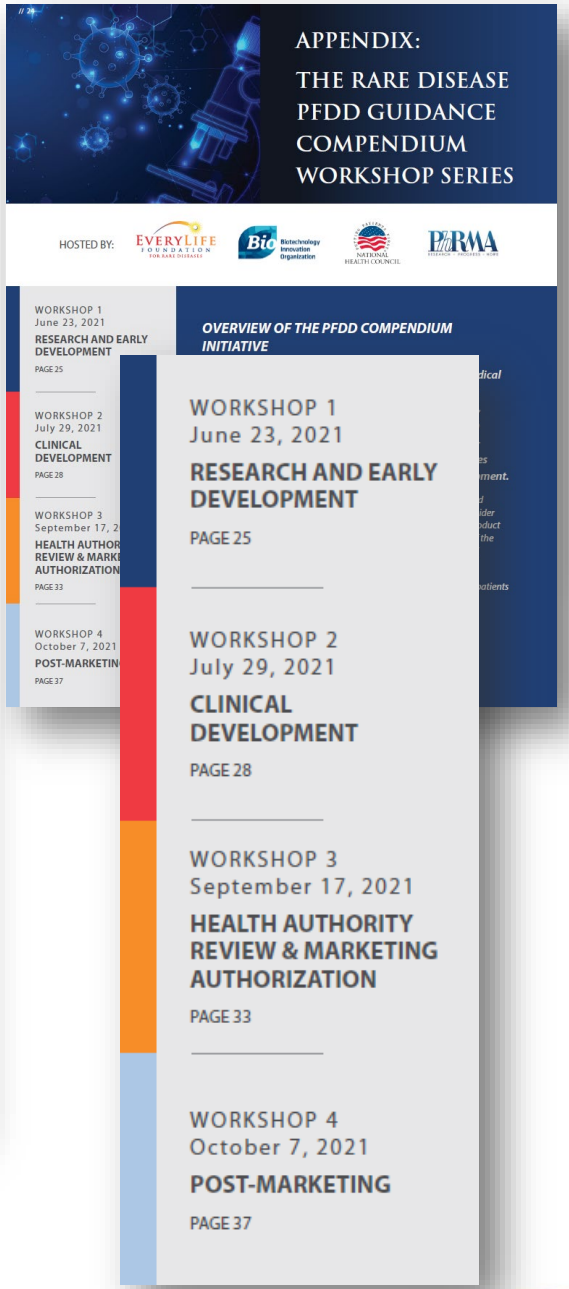

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Thank You!

With sincere appreciation to the following individuals who made extensive contributions to the design and convening of the workshop series and development of this guide.

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Annie Kennedy, EveryLife Foundation
Victoria Dohnal, BIO (Aug 2021 – Nov 2021)
Kate Donigan, BIO (beginning Dec 2021)
Eric Gascho, National Health Council
Danielle Friend, BIO (through Aug. 7, 2021)
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