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SCHOOL OF PHARMACY

Engaging Patients at Multiple Levels

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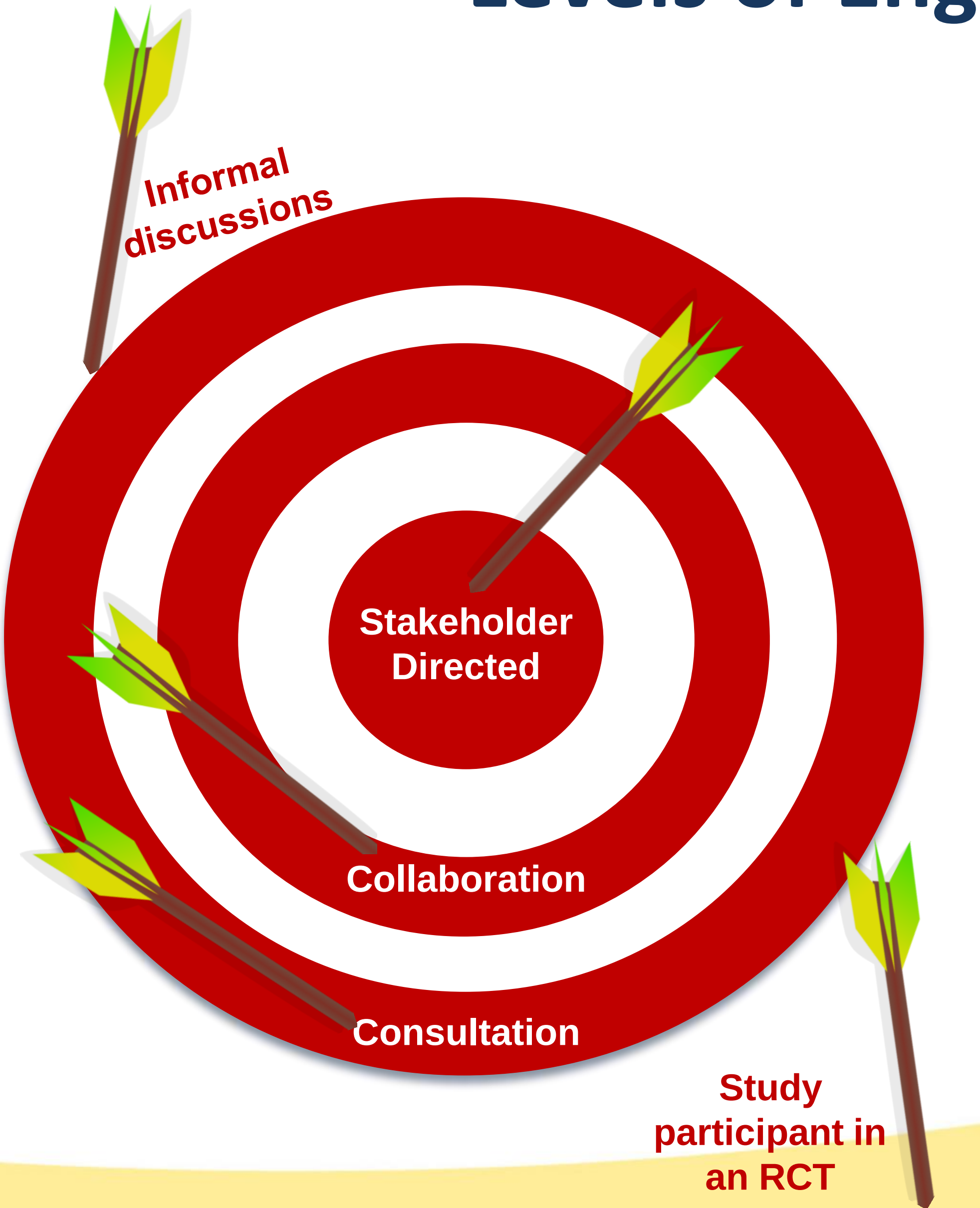
Registry Founder and Coordinator, PSC Partners Seeking a Cure

Levels of Engagement



- ○ **Patient Directed**
  - Patient/Patient group led
- ○ **Partnership**
  - Investigator/Co-investigator
- ○ **Collaboration**
  - Advisory committee member
- ○ **Consultation**
  - Consultant
  - Interviews
  - Focus groups
  - Surveys
- ○ **Informal**
  - Unstructured discussions
- ○ **Study participant/subject**

Forsythe, et al. JGIM, 2015 &[2]
Perfetto, ISPOR, 2015 Annual Meeting

Partnership on the Project:
UMB and NORD – Pre-engagement

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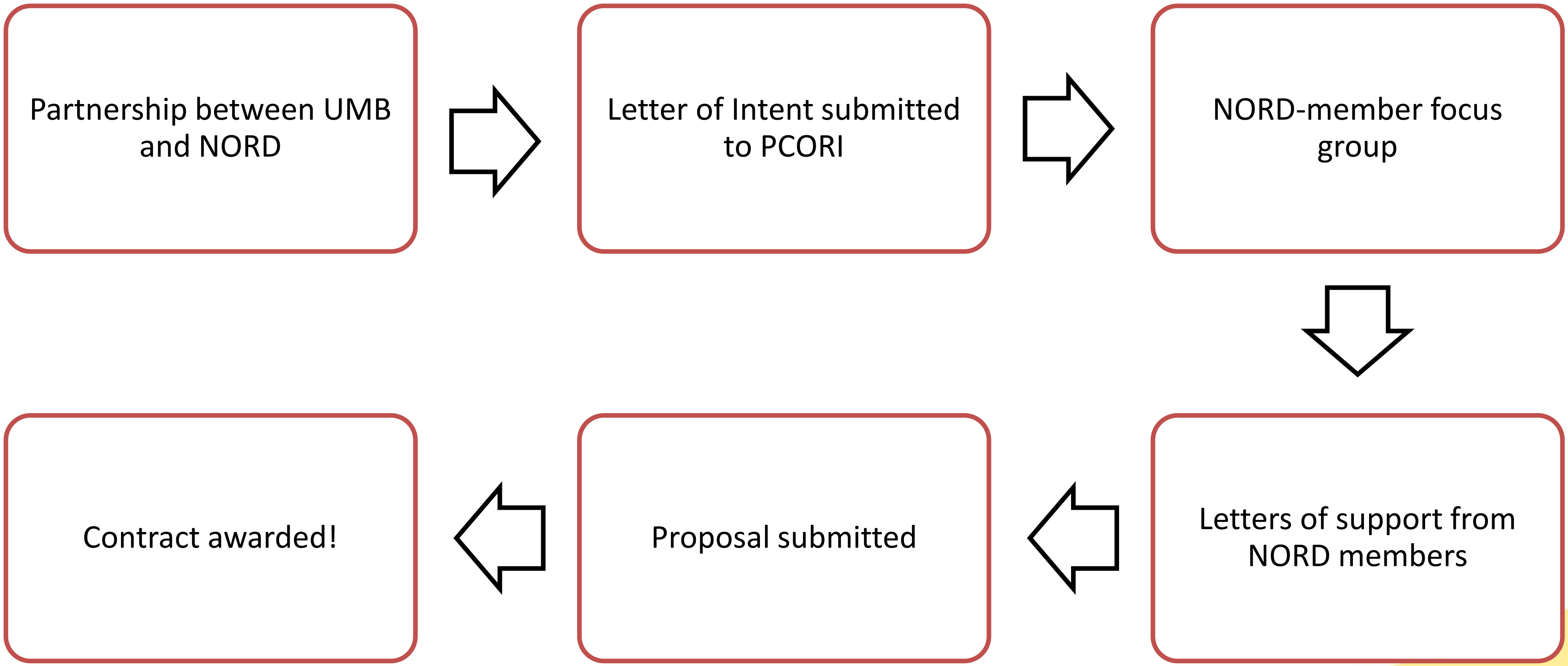
NORD
National Organization for Rare Disorders

Preliminary discussions

Partnership formed

First letter of intent submitted (unsuccessful)

Pre-Award Project Phases



Partnership between UMB and NORD

Letter of Intent submitted to PCORI

NORD-member focus group

Letters of support from NORD members

Proposal submitted

Contract awarded!

Partnership on the project: UMB and NORD – Engagement

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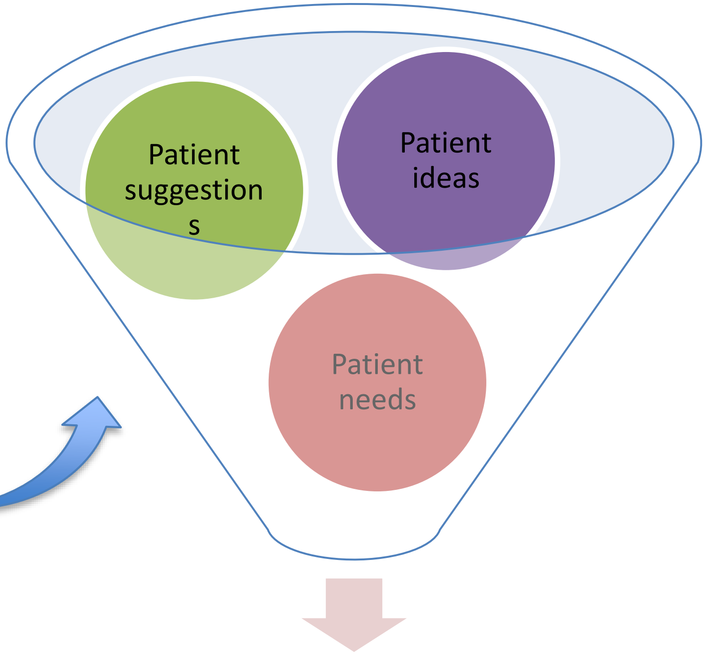
NORD
National Organization for Rare Disorders

Focus group with 10 NORD members



Refined Project Objectives

Collaborative proposal submitted

Funded by pcori

Project Overview

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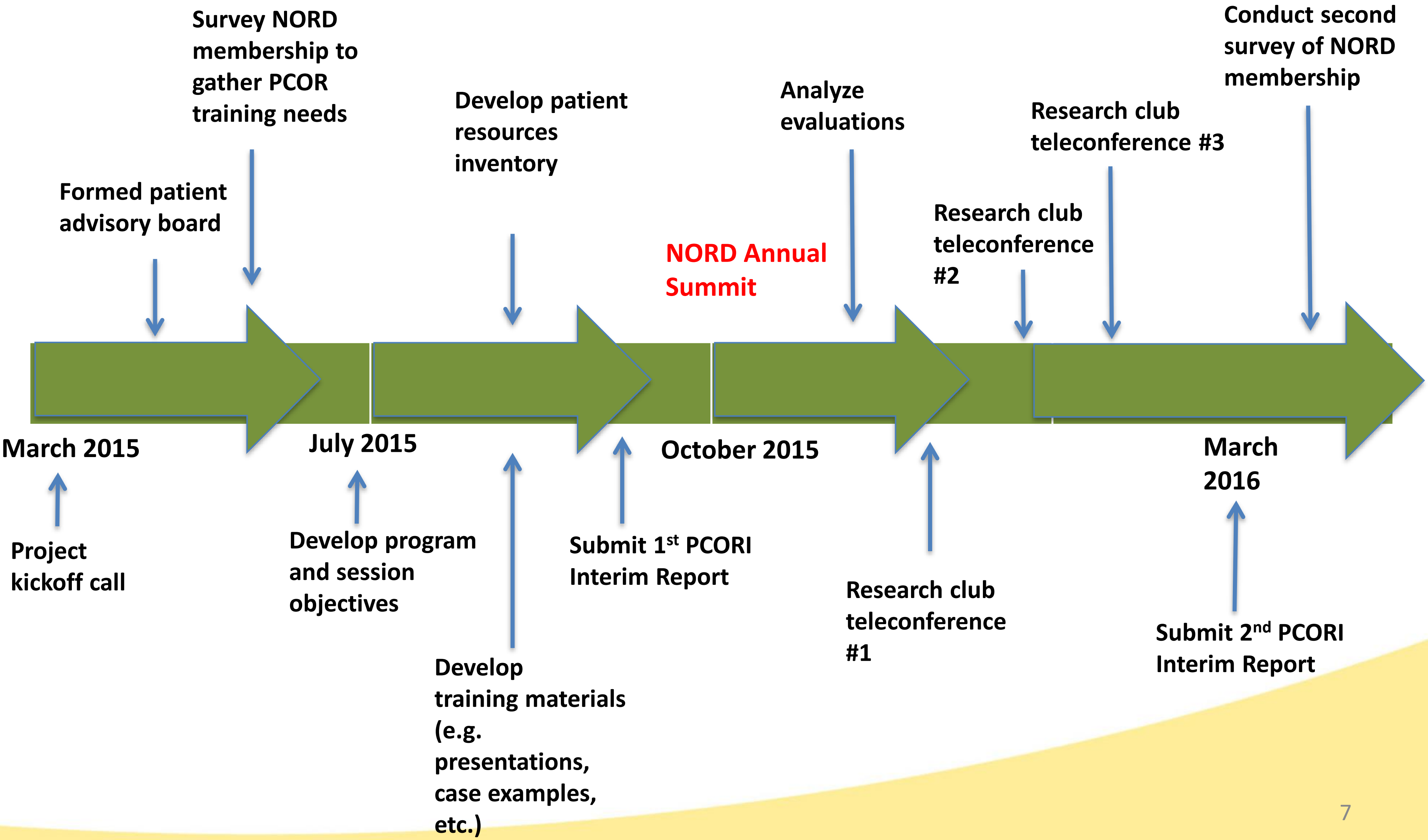
NORD
National Organization for Rare Disorders

Objective: Equip NORD-member organizations for engagement in PCOR

1. Training on PCOR for NORD membership at the NORD Annual Summit (October 2015), in Washington, DC:
   - PCOR 101: Summit half-day track for any attendee
   - PCOR 201: Post-Summit, full-day, Special Session for 49 selected applicants
2. Post-Summit Research Club (for selected applicants)
   - Three monthly teleconferences
3. Evaluation of Summit programs and Research Club

Project Timeline



March 2015

Project kickoff call

Formed patient advisory board

Survey NORD membership to gather PCOR training needs

July 2015

Develop program and session objectives

Develop patient resources inventory

Develop training materials (e.g. presentations, case examples, etc.)

Submit 1st PCORI Interim Report

NORD Annual Summit

October 2015

Analyze evaluations

Research club teleconference #1

Research club teleconference #2

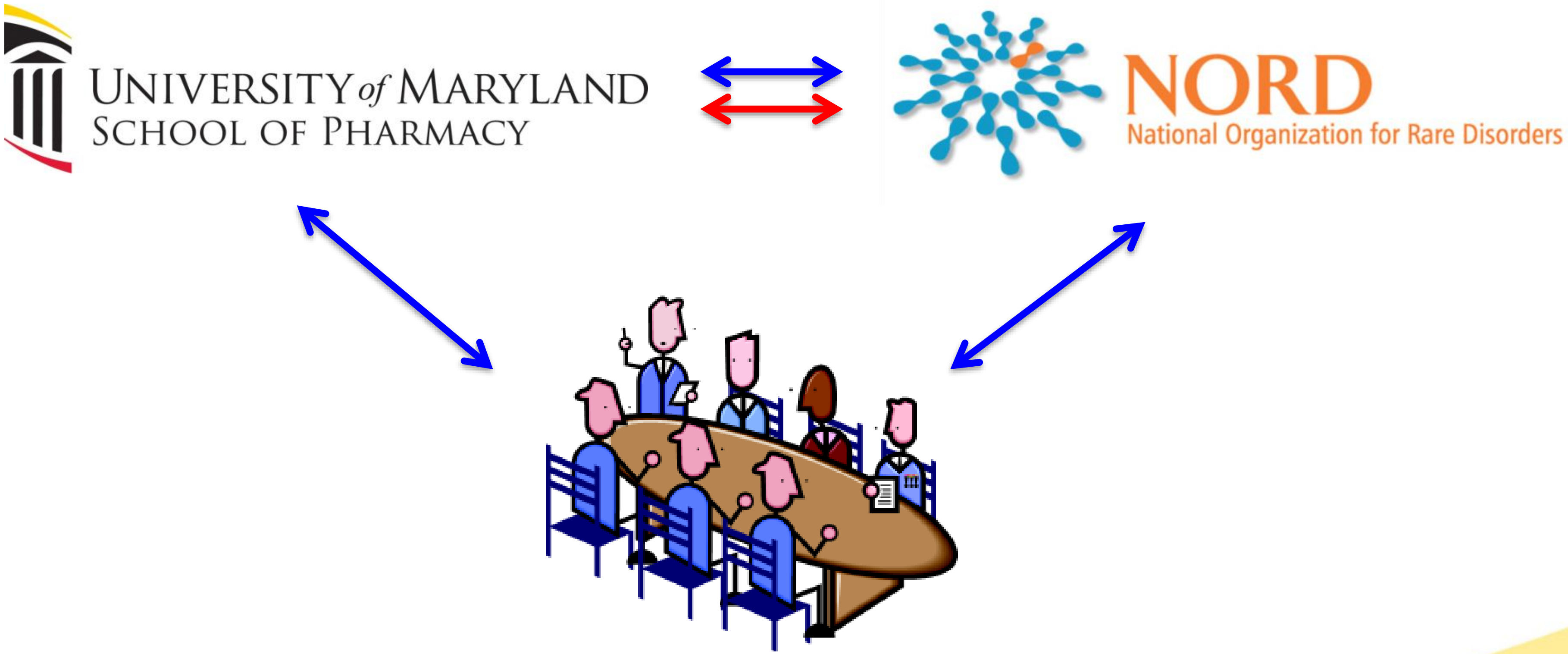
Research club teleconference #3

Conduct second survey of NORD membership

March 2016

Submit 2nd PCORI Interim Report

Patients Engaged at Multiple Levels



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NORD
National Organization for Rare Disorders

Patient Advisory Board

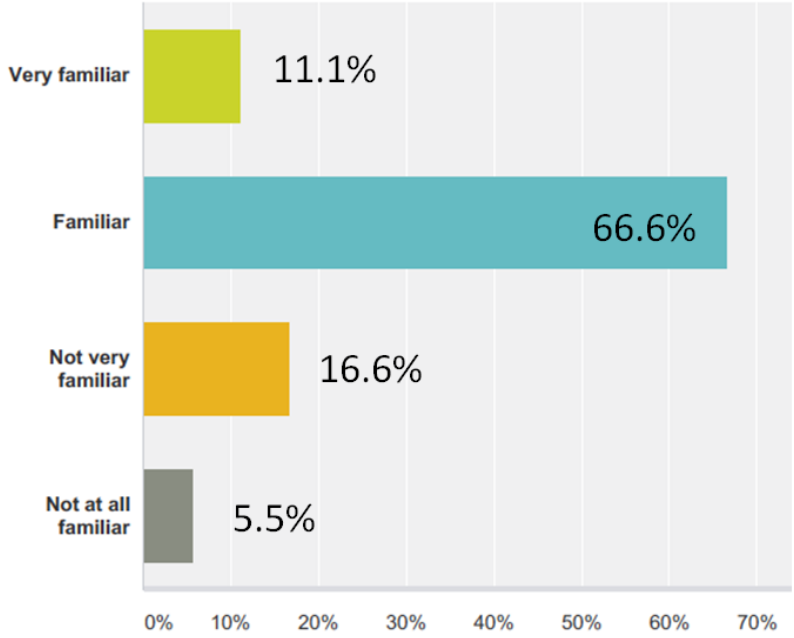
Patient Advisory Board

- Guide the team on learning objectives, topic selection, and the agenda
- Iterative development of agenda and materials
- Review and comment on draft materials for the programs to ensure suitability for the proposed purpose and audience needs
- Actively participate in committee meetings
- Provide periodic feedback in between scheduled committee meetings
- Disseminate information about the training program to respective organization members and partners

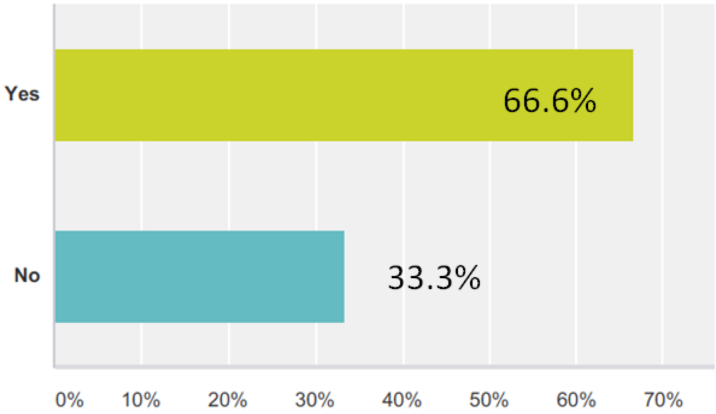
Needs Assessment Survey

Survey NORD membership to assess PCOR training needs

How familiar are you with patient-centered outcomes research?



Has your organization interacted with PCORI?



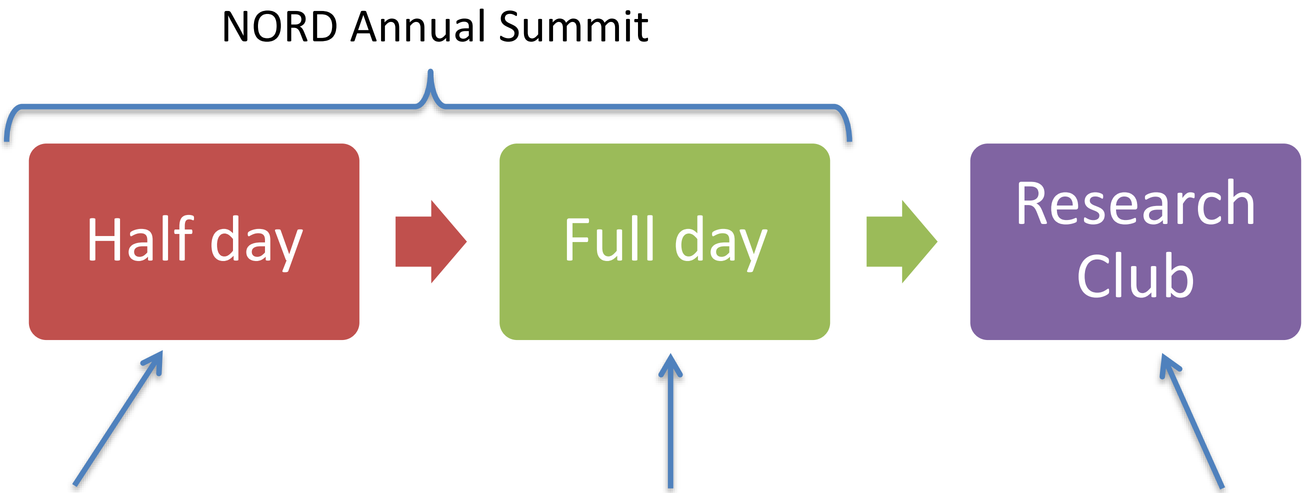
In order for your organization to become more engaged in PCOR, in general, and with PCORI, what are the top 3 skills your organization needs to be successful?

- Definitions/ thorough understanding of PCOR
- Info on how patient advocacy groups can contribute
- Skills for collecting data on PRO’s
- Communication skills
- Grant writing skills/information on what is available
- Research/scientific mentorship and partnerships
- Social media training
- Specific examples of small organizations being involved in PCOR
- Budgeting

What are the least important things patient advocacy organizations need to learn about PCOR? Is there anything that is well known and not worth spending time on?

- General response: level of knowledge is varied, so there is very little that could be skipped

Outcome of Needs Assessment: Program Delivery



NORD Annual Summit

Half day

Full day

Research Club

<u>“PCOR 101”</u>
Session during the 2015 NORD Annual Meeting (open to all attendees and required for full day participants)

<u>“PCOR 201”</u>
Day after NORD Annual meeting. All NORD members were invited to apply to attend.

<u>Continuing Engagement</u>
Three webinars following the Annual Meeting: PICOTS, PRO’s, and current funding opportunities. Active participation was encouraged.

Program Evaluation – Half Day

- How will you use the information you learned in this track in your work life?
  - *In every way. For registry, for creating partnerships, in grant application. We always place the patient at the center, but you provided us with ways to place the patient at every level and have the tools to do it well*
  - *Consider applying to PCORI for support for appropriate projects*
- What was the most useful topic covered?
  - *Developing industry partners and an overview of the process for approval*
  - Patient-centered research and patient-engagement early & often throughout
- What was the least useful topic covered? Please explain
  - The policy advocacy piece-seemed specific to (case studies)
  - Partnerships—too elementary

Program Evaluation – Full Day

- Participants valued time to network with other participants
- Topics Identified as most useful:
  - *Open discussion on patient registries*
  - *Leveraging assets*
  - *“De-mystifying” PCORI funding-- Q&A with PCORI staff*
  - *Negotiating partnerships with industry partners*
- Ways participants will use information from training in their work life:
  - *Develop initial survey to learn patients’ research priorities*
  - *Incorporate patients voice to all projects (e.g., research, website, publications, etc.)*
  - *Resubmit PCORI applications*
  - *Educate board members and partners*
  - *Evaluate organization’s current programs*

Program Evaluation – Full day

Suggestions for improvement:

- *PCORI presentation at beginning of training*
- *One-on-one project support, writing proposals correctly*
- *Step-by-step guidance to successful application*
- *Add small group discussions*
- *Session to walk through a successful PCOR application (from conceptualizing ideas, proposal development, implementation, etc.)*
- *Discussion on PCORI's Engagement Rubric*
- *Teaching types of comparative-effectiveness research questions (PICOTS)*
- *Time for introductions and networking*
- *Conference/training focused on patient registries*

Program Evaluation – Research Club

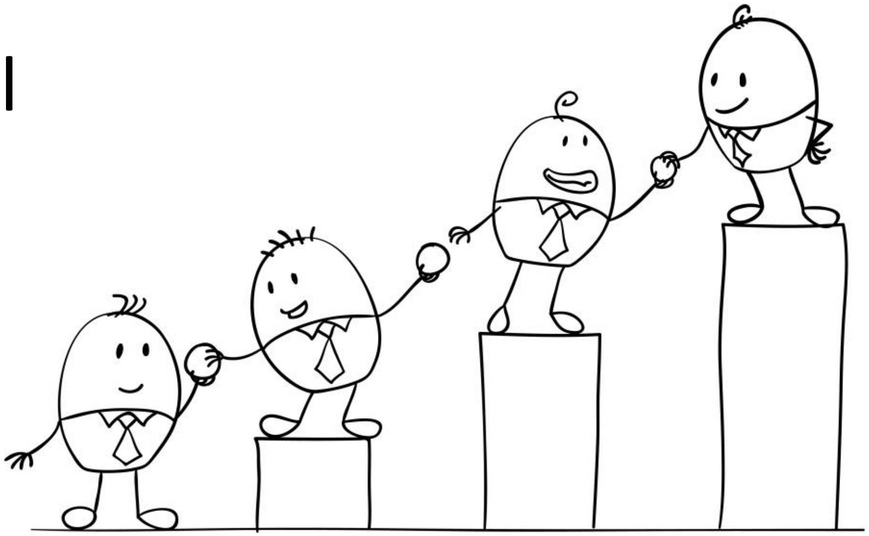
- #1: PICOTS Research Question Framework (n=16)
  - 81% found the pre-call materials helpful in understanding the topic
  - 75% found this discussion valuable
  - 63% feel confident they could develop a research question using this framework *
- #2: PCORI Eugene Washington Engagement Award Application (n=16)
  - 69% agree that the pre-call materials were useful in understanding the topic
  - 69% found the topic valuable
  - 50% of participants indicated they feel confident that they can participate in completing a PCORI application
- #3: Patient Reported Outcomes (n=16)
  - 63% agreed that the pre-call materials were helpful in understanding the topic
  - 63% found this topic valuable
  - 44% confident that they can participate in a project that includes PROs

Program Evaluation – Research Club

- What did you like most about research club? (n=14)
  - *The opportunity to learn first-hand about PCORI and connect with others were learning along with me. It is very helpful to hear questions/discussion from the other club members.*
  - *The presentations were focused on the information relevant to applying for a PCOR grant. The topics were precisely those that are now important in collaborations with pharmas, and researchers as well as in applying for any grant. How to incorporate the patients into every level of the research process is applicable to the projects rare disease organizations get involved in.*
- What did you like least about Research Club? (n=15)
  - *Volume of participants and time not always enough to allow everyone to participate verbally*
  - *I wasn't able to attend as many calls as I would have liked. Were they recorded?*
  - *Not being able to access one of the calls.*
  - *That it was over. I feel like it could continue and teach us invaluable things.*

Summary of engagement methods from this project

- Partnership
- Iterative interactions among partners (formal and informal)
- Focus group
- Advisory Board
- Member survey
- Interactive training sessions
- Program evaluations



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Perfetto, ISPOR, 2015 Annual Meeting

How do we gauge our success?

- Good evaluations
- Learner success with PCORI LOIs and proposal funding
- Trusted by NORD members who contact us for advice
- Desire by the Patient Advisory Board to submit additional LOIs to PCORI

Thank you

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rachel@gomel.org

Our Team

- NORD staff:
  - Jacqueline Kraska, MA, Research Programs Manager
  - Lisa Phelps, MPH, Director of Marketing and Community Relations
  - Mary Dunkle - Vice President for Educational Initiatives
  - Jennifer Huron –Associate Director of Communications & Marketing
  - Kristine Kristen Angell - Membership Manager
  - Carrie Lucas - Associate Director of Events
- UMB staff:
  - Eleanor M. Perfetto, PhD, MS, Principal Investigator
  - C. Daniel Mullins, PhD, Co-Investigator
  - Chinenye Anyanwu, PharmD, MPH, Project Manager
  - Elisabeth Oehrlein, Research Assistant
  - Maya Hanna, MPH, Research Assistant

Our Team

- Advisory Board Co-Chairs:
  - C. Daniel Mullins, PhD, Professor & Chair, Pharmaceutical Health Services Research, University of Maryland, Baltimore (UMB) School of Pharmacy
  - Jacqueline Kraska, MA, Research Programs Manager, National Organization for Rare Disorders (NORD)
- Advisory Board Members:
  - Ron Bartek, *Friedrich's Ataxia Research Alliance (FARA)*
  - Marc Boutin, JD, *National Health Council*
  - Christine Brown, *National PKU Alliance*
  - Laurie Burke, BS Pharm, MPH, *The Lora Group*
  - Rachel Gomel, *PSC Partners*
  - Reta Honey-Hiers, RNC, *Tarlov Cyst Disease Foundation*
  - Jacqueline Kraska, MA, *NORD*
  - C. Daniel Mullins, PhD, *UMB School of Pharmacy*
  - Christopher Scalchunes, *Immune Deficiency Foundation (IDF)*

Project Organization

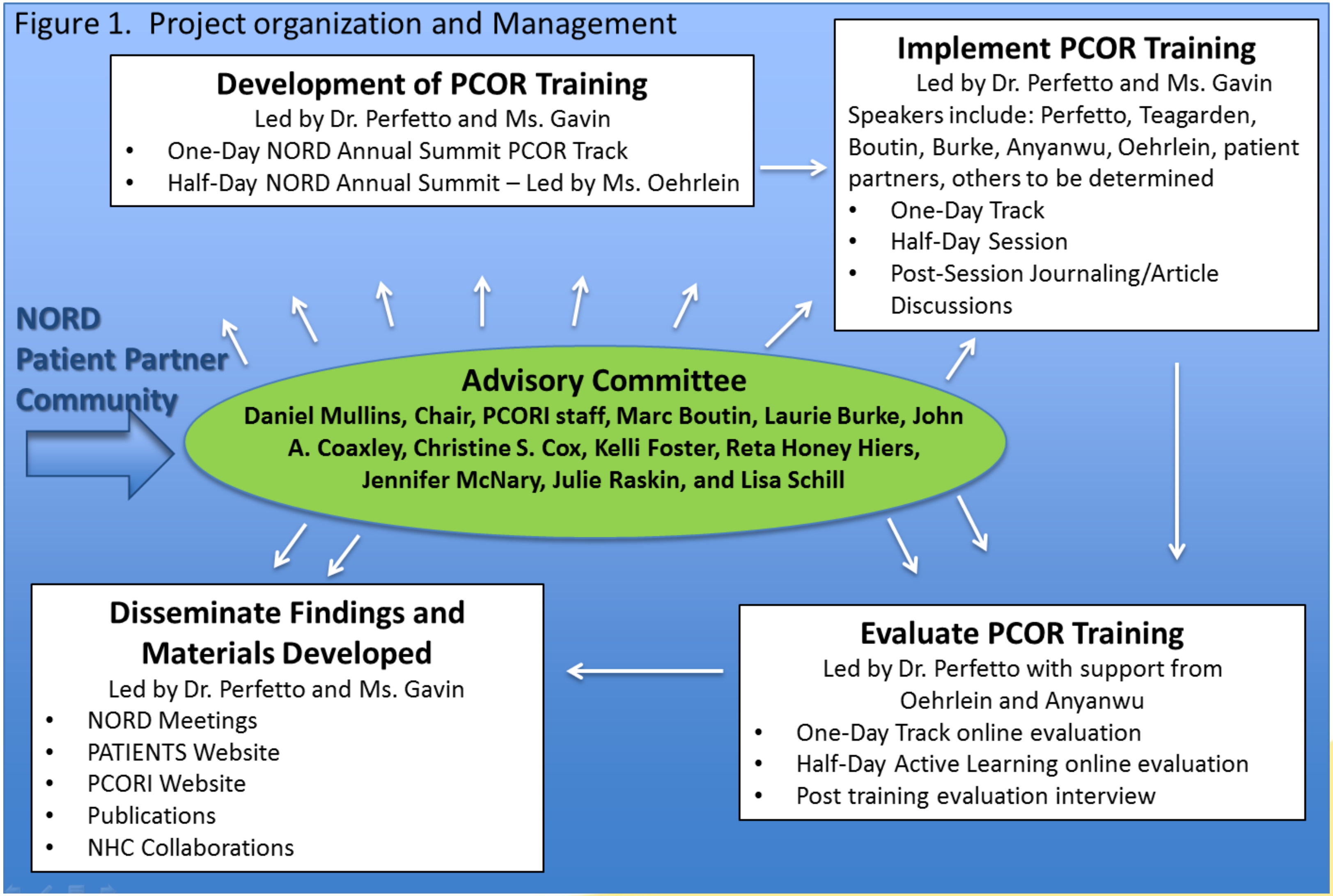

Figure 1. Project organization and Management

Development of PCOR Training

Led by Dr. Perfetto and Ms. Gavin

- One-Day NORD Annual Summit PCOR Track
- Half-Day NORD Annual Summit – Led by Ms. Oehrlein

Implement PCOR Training

Led by Dr. Perfetto and Ms. Gavin

Speakers include: Perfetto, Teagarden, Boutin, Burke, Anyanwu, Oehrlein, patient partners, others to be determined

- One-Day Track
- Half-Day Session
- Post-Session Journaling/Article Discussions

NORD Patient Partner Community

Advisory Committee

Daniel Mullins, Chair, PCORI staff, Marc Boutin, Laurie Burke, John A. Coaxley, Christine S. Cox, Kelli Foster, Reta Honey Hiers, Jennifer McNary, Julie Raskin, and Lisa Schill

Disseminate Findings and Materials Developed

Led by Dr. Perfetto and Ms. Gavin

- NORD Meetings
- PATIENTS Website
- PCORI Website
- Publications
- NHC Collaborations

Evaluate PCOR Training

Led by Dr. Perfetto with support from Oehrlein and Anyanwu

- One-Day Track online evaluation
- Half-Day Active Learning online evaluation
- Post training evaluation interview