National Organization for Rare Disorders (NORD)
Rare Diseases & Orphan Products Breakthrough Summit
Arlington, VA
Track VI: Patient-Centered Outcomes Research
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Partnerships and Funding for PCOR Projects
Presenters

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Acknowledgements

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Nathalie Kayadjanian, PhD, *Foundation for Prader-Willi Research*

Janalee Heinemann, MSW, *Prader-Willi Syndrome Association (USA)*
Learning Objectives

By the end of this session, participants will be able to:

• Describe and leverage the assets of a rare disease patient group to attract partners for PCOR and funding

• Describe strategies for partnering with various stakeholders to conduct successful PCOR in applying for funding

• Differentiate PCOR funding opportunities from traditional funding streams and opportunities for rare disease groups (e.g., need for foundational data)

• Identify PCOR funding opportunities available for patient groups (alone or in partnership) from various organizations e.g., PCOR and NIH
Session Overview

1. Win-Win Partnerships in PCOR
   - Eleanor Perfetto, University of Maryland School of Pharmacy
   - Stephanie Okey, formerly Genzyme

2. Case Example
   - Theresa Strong, Foundation for Prader-Willi Research

3. Questions and Discussion
Patient Groups Bring Unique Value to a PCOR Partnership

- Especially rare disease patient groups!
- ‘Quid pro quo’
- Perspectives that stem from experience
  - Patient, caregiver, family member
- Experts in engaging the patient population
- Experts on the condition/disease
- Can promote dissemination and education
# Partnership Checklist

## MY CHECKLIST FOR A SUCCESSFUL PARTNERSHIP

<table>
<thead>
<tr>
<th></th>
<th>YES</th>
<th>NO</th>
<th>Notes</th>
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<tbody>
<tr>
<td>1. Is the research question patient centered?</td>
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<td>2. Can patient engagement improve the research?</td>
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<td>3. Is there a patient-engagement strategy I can contribute to?</td>
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<td>4. As an engaged patient or organization, can I clearly describe what my role would be in the research?</td>
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<td>5. Would it be active engagement?</td>
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<td>6. Would patients have any power to contribute toward decisions about the research?</td>
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Collaborating with Stakeholders

Benefits

- Gain a greater understanding of needs across the health system
- Bi-directional learning: shared skills and expertise
- Improve study design
- Meaningful results for patients
- Gain access to information and data
- Secure funding and resources

Challenges

- Differing expectations or goals
- Maintaining independence and understanding boundaries
- Logistics: stakeholders may live in different geographic locations/time zones

Adapted from: International Alliance of Patients’ Organizations. Assessed at: https://www.iapo.org.uk/sites/default/files/current/iapo_toolkit.html
Formal versus Informal Partnerships

• Informal partnerships include:
  • Information sharing
  • Advice from key opinion leaders
  • Manuscripts or grant application review

• Formal partnerships include:
  • Shared risk and responsibility
  • Vested interest (often monetary)
  • Balanced accountability
"You make a good point; we both hate the cat. I’m just not sure what it is you’d bring to a partnership."
Framework for Effective Partnerships in PCOR

Reciprocity of Value

Clarity of Purpose

Strategic Engagement of Stakeholders

STRENGTH OF RELATIONSHIP

Source: Schools in Partnership
Characteristics of Good Partnerships

- Mutual respect
- Good communication
- Transparency
- Mutual appreciation for one another’s value
- Fiduciary duty over self promotion
Win-Win Partnerships in PCOR: Where to Start?

• Have a clear rationale for why you want to partner
  – Your organization has no research experience
  – Avoid duplication of effort
  – Multiple sectors of the community need to be engaged for success

• Why does this partnership make sense for both parties?
Win-Win Partnerships in PCOR: Choosing Potential Partners

When identifying potential partners, consider:

• What do they bring to the table?
• Is this the right time?
• Have they partnered before? Successfully?
• What do you envision each partners role to be?
• What potential barriers exist to recruiting partners?
• What strategies can help overcome those barriers?
Win-Win Partnerships in PCOR: Finding Partners

- Professional meetings
  - Clinical specialty professional societies
  - Health services research
- PCORI workshops/website
  - PCORI “What we’ve funded” lists the PI and their affiliations
- Universities
  - Publications, grant announcements, centers of excellence
  - e.g., University of Maryland has CER-PCOR Workgroup
- Literature and publications
- They call you!
Creating a Win-Win Scenario: Potential Roles

• Partners can provide:
  • Research methodology skill
  • Grant-writing skill
  • Literature review
  • Manuscript writing expertise

• You should expect
  • Co-Authorship
  • Opportunities to disseminate/present findings
  • Fair compensation
    • PCORI Financial Compensation of Patients, Caregivers, and Patient/Caregiver Organizations

PCOR versus Traditional Research

**PCOR**
- Patient engagement
- Outcomes are those identified by patients as important
- Diverse patient populations/subjects
- Comparative
- Patients inform study design
- Patients involved in dissemination

**Traditional Research**
- Investigator initiated
- Patients as subjects
- May not be comparative
- Not necessarily diverse populations/subjects
- Patients may help in design of consent documents, but little else
- No dissemination plan
### Examples of Research Funding Sources

<table>
<thead>
<tr>
<th>PCORI</th>
<th>AHRQ</th>
<th>NIH</th>
<th>Robert Wood Johnson Foundation</th>
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<tbody>
<tr>
<td><strong>Mission:</strong> Help people make informed healthcare decisions. <strong>Example:</strong> Patient-Powered Research Networks Research Demonstration Projects&lt;sup&gt;1&lt;/sup&gt;</td>
<td><strong>Mission:</strong> Produce evidence to make health care safer, higher quality, more accessible, equitable and affordable. <strong>Example:</strong> Patient-Centered Outcomes Research Clinical Decision Support Learning Network&lt;sup&gt;2&lt;/sup&gt;</td>
<td><strong>Mission:</strong> Seek fundamental knowledge about the nature and behavior of living systems and application of that knowledge. <strong>Example:</strong> BRAIN Initiative: Short Courses in Computational Neuroscience&lt;sup&gt;3&lt;/sup&gt;</td>
<td><strong>Mission:</strong> Improve the health and health care of all Americans. <strong>Example:</strong> Evidence for Action: Investigator-Initiated Research to Build a Culture of Health&lt;sup&gt;4&lt;/sup&gt;</td>
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## Engagement Plan Example

**Exhibit 10. Roles and Responsibilities at Every Stage in Research**

<table>
<thead>
<tr>
<th>RESEARCH STAGE</th>
<th>ACCOUNTABLE</th>
<th>CONSULTED</th>
<th>GIVE FINAL APPROVAL</th>
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<tbody>
<tr>
<td>Topic Selection, Formulating Study Question</td>
<td>Investigator Team</td>
<td>Patient Advisory Panel</td>
<td>Patient Advisory Panel</td>
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<td>Design of Recruitment and Consent Procedures</td>
<td>Investigator Team</td>
<td>Patient Advisory Panel</td>
<td>Research Support Team</td>
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<td>Identifying Outcomes for Measurement</td>
<td>Patient Advisory Panel</td>
<td>Investigator Team</td>
<td>Patient Advisory Panel</td>
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<td>Learning Collaborative Agenda Setting</td>
<td>Patient Advisory Panel</td>
<td>Research Support Team</td>
<td>Patient Advisory Panel</td>
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<td>Data Collection</td>
<td>Investigator Team</td>
<td>Patient Advisory Panel</td>
<td>Investigator Team</td>
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<td>Interpretation of Findings</td>
<td>Investigator Team</td>
<td>Patient Advisory Panel</td>
<td>Investigator Team</td>
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<tr>
<td>Dissemination Plan</td>
<td>Research Support Team</td>
<td>Investigator Team</td>
<td>Research Support Team</td>
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<tr>
<td>Final Dissemination</td>
<td><strong>ALL PARTICIPATE IN DISSEMINATION</strong></td>
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Putting Your Best Foot Forward

• Don’t rush! Ensure adequate time to develop the vision and purpose for your partnership

• Be prepared with your ‘Ask’ and your ‘Give’
  – Attainable needs communicated in a clear, concise manner
    • Compelling rationale for the proposed partnership
    – Ensure partners can visualize the benefits – what is their ROI?

• Develop relationships that are genuine and sustainable
  – Stay transparent
  – Foster frequent communication
  – Acknowledge your partners on all activities that come out of that partnership

Expect the same in return!
Case Example: Foundation for Prader-Willi Research
Foundation for Prader-Willi Research (FPWR): “Stalking” Biotech Companies

• FPWR established in 2003 by parents of children living with Prader-Willi Syndrome (PWS)
• Mission: Eliminate the challenges of PWS through advancement of research
• Funds research projects - collaborative, scientifically meritorious, and relevant to individuals with PWS and their families.
• Research topics include:
  – Genetics and pathophysiology of PWS
  – Improving academic and learning skills for children with PWS
  – Understanding and treating clinical complications of PWS
  – Development of novel therapeutics for PWS
Foundation for Prader-Willi Research: “Stalking” Biotech Companies

- Company X publicly discussed testing a drug in PWS
- FWPR provided Company X with the brief report, “Evaluation of Pharmacologic Interventions in the Prader-Willi Syndrome Population: Special Considerations”
- Partnered with Company X to bring patient perspective to drug development and inform clinical trial design
- Educated PWS community to prepare them for clinical-trial involvement
- Helped with recruitment efforts – hosted a webinar, used social media reach to spread awareness about the clinical-trial opportunity
Foundation for Prader-Willi Research: “Stalking” Biotech Companies

- FPWR suspected Company Y’s drug might be an effective PWS treatment
- Company Y had no active PWS program
- FPWR provided the report (previously mentioned) and rationale for why their drug might be well suited for PWS patients
- Partnered with Company Y to develop a successful Orphan Product Grant Program application, currently funded by FDA

http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm463539.htm
Foundation for Prader-Willi Research: “Stalking” Biotech Companies

• Lessons learned
  – Stay current on drugs in development, understand your targets and find biotech/pharma companies that may be looking at those targets
  – Learn about the industry partner’s objectives and determine where your goals are aligned
  – Show your value early:
    • Understand your disorder and your population
    • Know the research tools and resources that are available
    • Be able to articulate unmet medical needs and disease burden
    • Facilitate interaction with patients and KOLs
    • Understand confidentiality issues
  – Be willing to provide some seed funding if it’s needed / if you can
  – Engage all stakeholders
Active Learning Exercise: Partnership Checklist

• Practice using the “My Checklist for a Successful Partnership” tool based on the case example you just heard.

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**Experience living with the disease**
- Study recruitment
- Education
- Policy/Advocacy
- Funding support of the research investigators
- Other (specify) Inform clinical trial design; advise researchers; dissemination

**What do I want my organization to get out of this partnership? (My ASK)**
- Funding
- Manuscript/other writing expertise
- Strong research methodology support
- Patient engagement opportunity
- Education

**Is this a good partner for my organization?**
- Formal partnership
- The timing is right
- Candidate possesses characteristics of a good partner
- Candidate has a history of partnership success

Notes: Developing/testing a drug in PWS; partner does not have

Notes: Data—Report: “Evaluation of Pharmacologic
Key Takeaways

• Partnership is the key word
• Bi-directional learning
  – Researchers and advocacy groups need to understand one another’s language, challenges and needs.
• Collaboration strengthens:
  – Research question
  – Study design
  – Proposals for funding
  – Relevance of findings
  – Ability to communicate findings in a meaningful way
Win-Win Partnerships in PCOR: Qualities to Look For

- Interest in the disease area/purpose of partnership
- Empathy
- Transparency
- Communication
- Equality
- Determined
Questions?
Session Resources

- International Alliance of Patient Organizations. Working with Partners and Stakeholders Toolkit. 
  https://www.iapo.org.uk/sites/default/files/current/iapo_toolkit.html

- PCORI Funding Opportunities. PCORI (Patients-Centered Outcomes Research Institute) website. http://www.pcori.org/funding-opportunities

- RareConnect: Connecting Rare Disease Patients Globally. https://www.rareconnect.org/en