NORD®
National Organization for Rare Disorders

RareLaunch: Research Ready Standards & Best Practices

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The Patient-Centered Outcomes Research Institute (PCORI)

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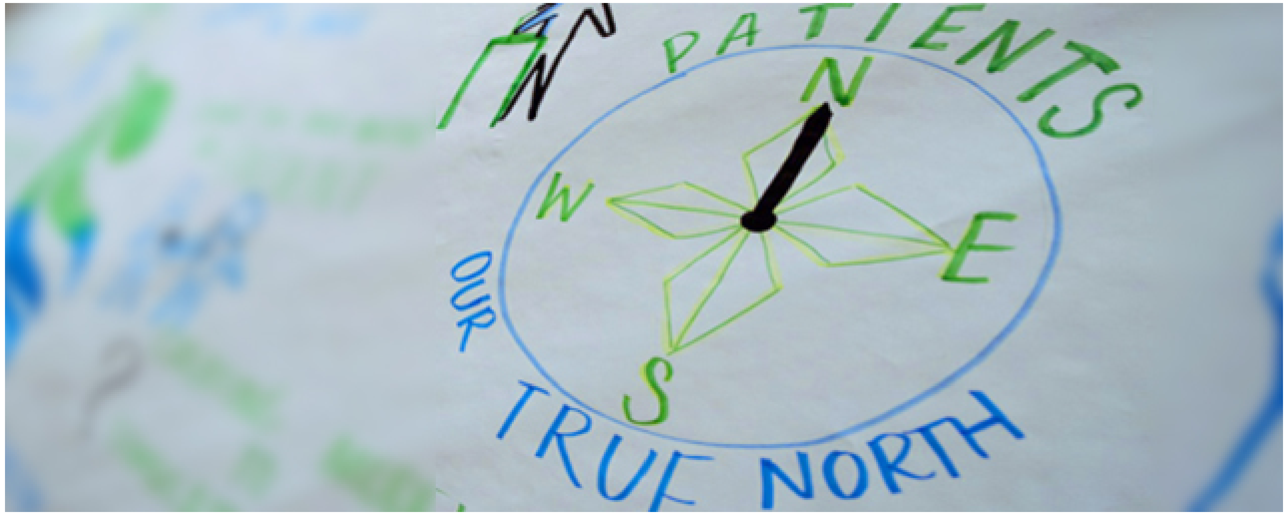
1.

About PCORI

About Us



- An independent research institute authorized by Congress in 2010 and governed by a 21-member Board of Governors representing the entire healthcare community
- Funds comparative clinical effectiveness research (CER) that engages patients and other stakeholders throughout the research process
- Seeks answers to real-world questions about what works best for patients based on their circumstances and concerns; reauthorized in 2019 for 10 years

We Fund Comparative Clinical Effectiveness Research (CER)

- Generates and synthesizes evidence comparing benefits and harms of at least two different methods to prevent, diagnose, treat, and monitor a clinical condition or improve care delivery
- Measures benefits in real-world populations
- Describes results in subgroups of people
- Helps consumers, clinicians, purchasers, and policy makers make informed decisions that will improve care for individuals and populations
- Informs a specific clinical or policy decision

Note: We do not fund cost-effectiveness research

Adapted from *Initial National Priorities for Comparative Effectiveness Research*, Institute of Medicine of the National Academies

Patient-Centered Outcomes Research

PCOR is a relatively new form of CER that….

- Considers patients' needs and preferences, and the outcomes most important to them
- Investigates what works, for whom, under what circumstances
- Helps patients and other healthcare stakeholders make better-informed decisions about health and healthcare options

What We Mean By…

“Patient-centeredness”

- The project aims to answer questions or examine outcomes that matter to patients within the context of patient preferences
- Research questions and outcomes should reflect what is important to patients and caregivers

“Patient and stakeholder engagement”

- Patients are partners in research, not just “subjects”
- Active and meaningful engagement between scientists, patients, and other stakeholders
- Community, patient, and caregiver involvement already in existence or a well-thought-out plan

2.

PCORI Rare Disease Research: Past and Present

PCORI Advisory Panels

- PCORI maintains advisory panels that include representation of clinicians, patients, scientific and health services research, and industry

- PCORI Advisory panels
  - Clinical Effectiveness and Decision Science
  - Healthcare Delivery and Disparities Research
  - Patient Engagement
  - Clinical Trials*
  - *Rare Disease**

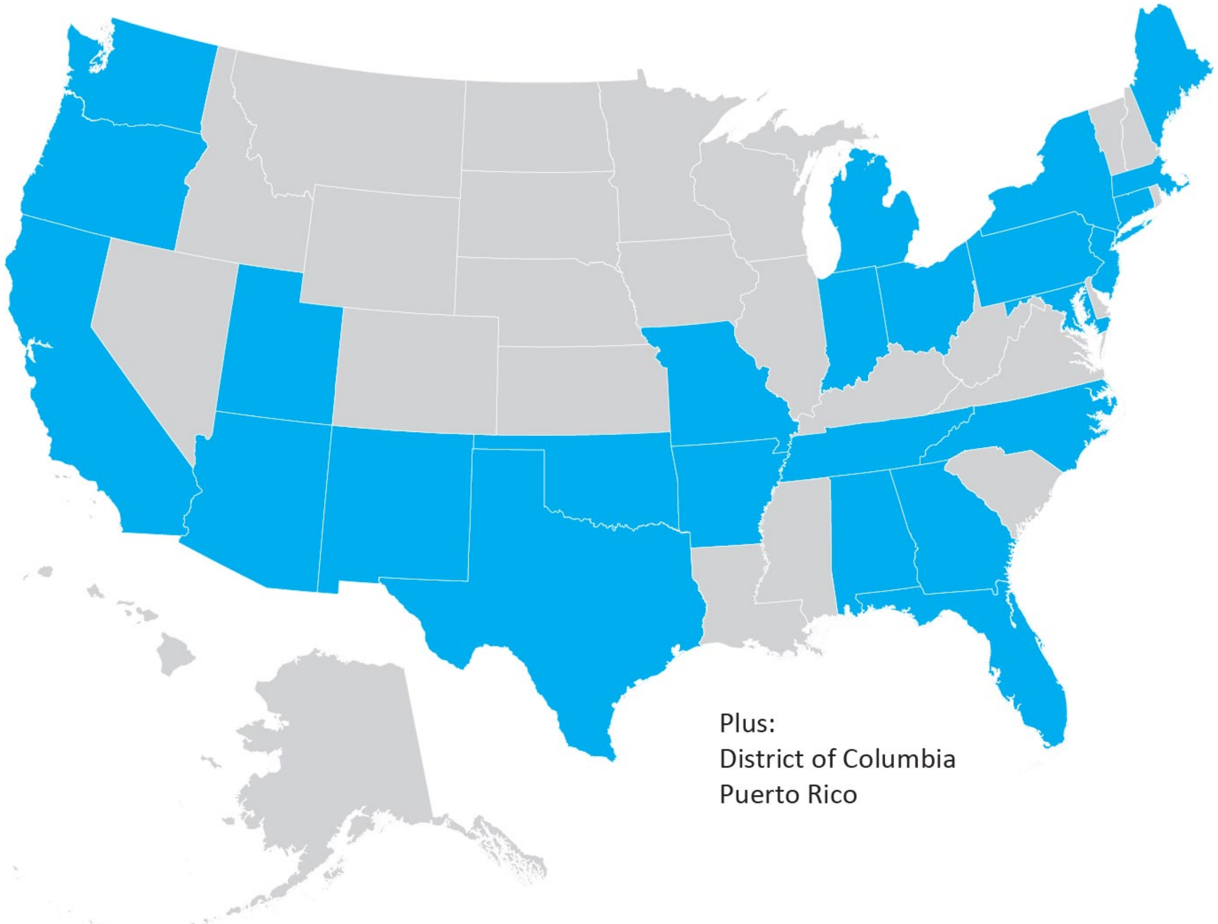
  - Applications for the 2021 cycle for all advisory panels are currently open

*Legislatively mandated

Snapshot of PCORI-Funded Rare Disease Projects

- Our legislative mandate requires particular attention and funding for research focused on rare diseases

- Amount awarded to Rare Diseases:
  - $89 million to fund 29 studies



Plus:
District of Columbia
Puerto Rico

As of August 2018

Rare Disease Portfolio: Specific Conditions

- Acute myeloid leukemia
- Cerebral palsy
- Chiari type I malformation (CM) & syringomyelia (SM)
- Disorders of sex development
- Duarte galactosemia
- Eosinophilic esophagitis
- Hydrocephalus
- Idiopathic subglottic stenosis
- Lupus nephritis
- Kawasaki disease
- Non-CF bronchiectasis
- Pediatric Crohn’s disease
- Pediatric transverse myelitis
- Polyarticular Juvenile Idiopathic Arthritis
- Sickle cell disease
- Spinal cord injury and spina bifida
- Systemic scleroderma
- Urea cycle disorders
- Myasthenia gravis

3.

Research Opportunities

Eugene Washington PCORI Engagement Award Program

- Support projects to build a community of patients and other stakeholders equipped to participate as partners in PCOR/CER, as well as serve as channels to disseminate PCORI-funded study results
- Funding to support engagement in, and with, research, not to conduct research

Engagement Award Funding Opportunities

$250,000
Up to 2 years

Engagement Award: Capacity Building

Objective: Prepare patients and stakeholders to participate as partners in PCOR/CER and/or develop partnerships and infrastructure to disseminate and implement PCORI-funded research findings

Most Recent Letter of Intent due date: October 1, 2020

$250,000
Up to 2 years

Engagement Award: Dissemination Initiative

Objective: Support communities and organizations to actively disseminate PCORI-funded research findings

Most Recent Letter of Intent due date: October 1, 2020

$100,000
Up to 1 year

Engagement Award: Stakeholder Convening Support

Objective: Convene stakeholders to explore critical issues related to PCOR/CER and/or communicate PCORI-funded research findings to targeted end-users

Most Recent Application due date: October 1, 2020

4.

Funding Example: Conference Award

Example: Conference Award

Wilms Tumor in WAGR Syndrome

- The International WAGR Syndrome Association (IWSA) received a conference support award to:
  - Engage parents and researchers in planning clinical studies
  - Develop a patient-centered model for this research
  - Develop consensus on research questions
  - Create educational awareness materials for use by all stakeholders

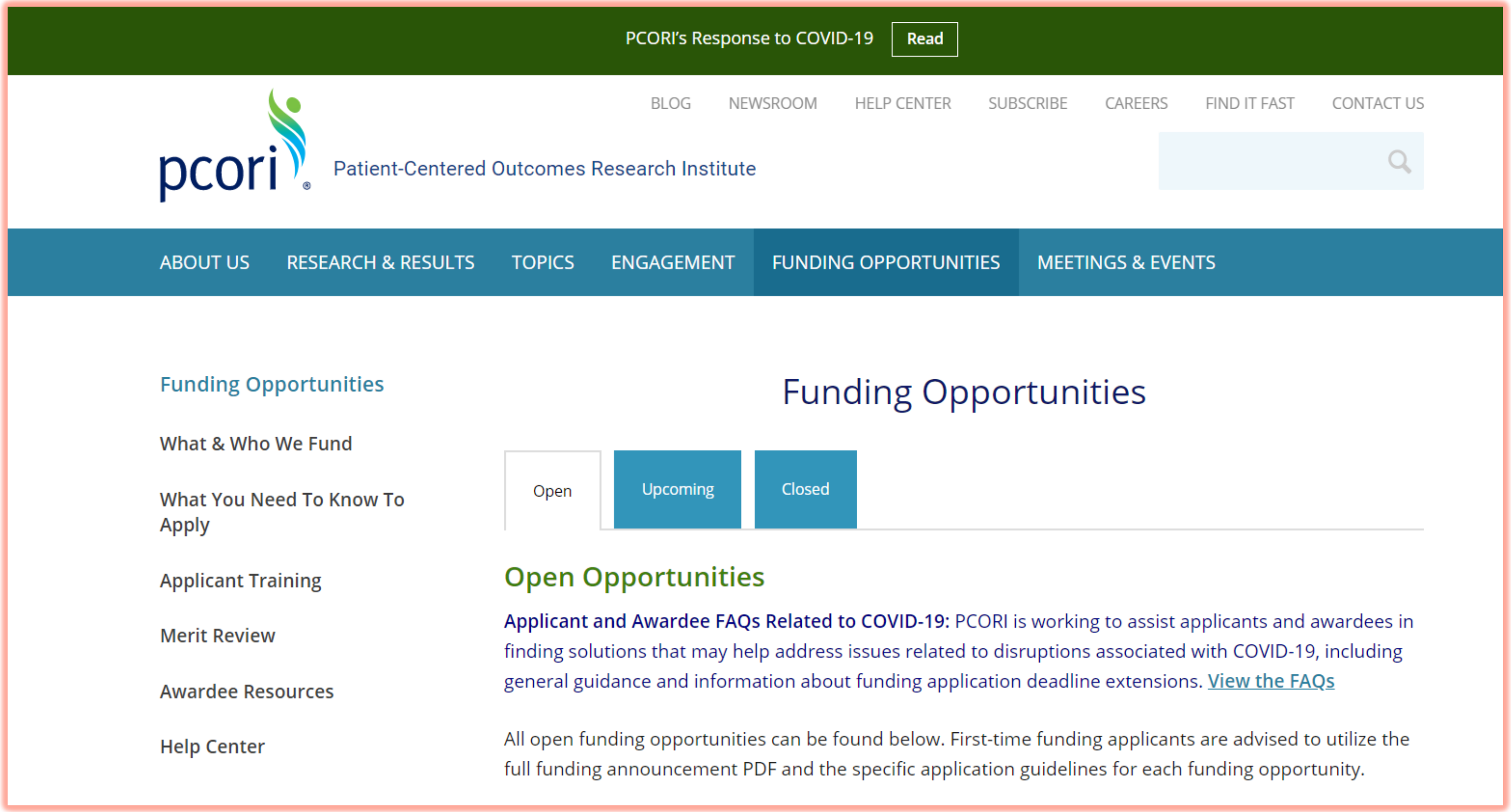
Click this link to visit the project page and learn more about this conference award

5.

Resources

PCORI Funding Opportunities

- Our research funding is awarded through PCORI Funding Announcements.
- Open opportunities are posted at pcori.org/apply.



Funding Opportunities

Open Opportunities

Applicant and Awardee FAQs Related to COVID-19: PCORI is working to assist applicants and awardees in finding solutions that may help address issues related to disruptions associated with COVID-19, including general guidance and information about funding application deadline extensions. View the FAQs

All open funding opportunities can be found below. First-time funding applicants are advised to utilize the full funding announcement PDF and the specific application guidelines for each funding opportunity.

WELCOME | Research Fundamentals

https://www.pcori.org/engagement/research-fundamentals pcori

WELCOME | Building Effective Multi-Stakeholder Research Teams

Coming
Late 2020

start

PCORI Rare Disease Resources

PCORI-Funded Rare Disease Projects and Related Resources

View listings of PCORI-funded rare disease clinical effectiveness research projects, as well as projects on coordination and engagement with the rare disease research community, and related resources.

Applicant Resources

- Guidance for RD Orgs for Research Awards
- FAQs for Rare Disease Applicants

Webinars & Other Events

- Webinar: PCORI Funding for Rare Diseases (2015)
- Town Hall: Management of Care Transitions for Emerging Adults with Sickle Cell Disease
- Rare Diseases Roundtable (2013)

Blogs, Feature Stories, Videos & Other Resources

Blogs

- Big Data versus a Rare Disease

- Using the PCORI Rare Disease Resources link you can find:
  - All of PCORI's funded rare disease projects
  - Applicant resources (rare disease-specific)
  - Past webinars
  - Rare disease PCORI-produced media, videos, and blogs
- Consider subscribing to PCORI email alerts about upcoming funding announcements and other PCORI news

Contact Information

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NORD®
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NORD Research Webinar Series: Insights & Advice

Patricia Seymour, CCRC, MA, CIP, IRB Administrator
and Human Subject Protection Director

AGENDA

- What is an IRB? Why do we need it?
- When do you need to obtain approval for your research?
- What will you need to submit to the IRB?
- Who should be involved in designing and carrying out research?
- What about external research requests?
- Day to day questions and resources
- Some self-promotion and good news

When Do I Need IRB Review of My Study?

- When there is interaction with participants
- When you are collecting data (or specimens) from/about participants

What is an IRB and Why Do We Need It?

- Independent or Institutional Review Boards exist to apply US regulations and guidance to the conduct of all kinds of research.
- The IRB is composed of scientists, physicians and community or non-affiliated local people.
- Nearly 90% of the research conducted in the US requires IRB review.

What Do I Need to Submit to the IRB?

- Protocol
- Consent, assent, information sheet
- Recruitment materials
- Assessments or surveys
- CV/resume of the Principal Investigator (Person responsible for the Research study)
- IRB's application for submission

WHO SHOULD BE INVOLVED IN DESIGNING AND CARRYING OUT RESEARCH?

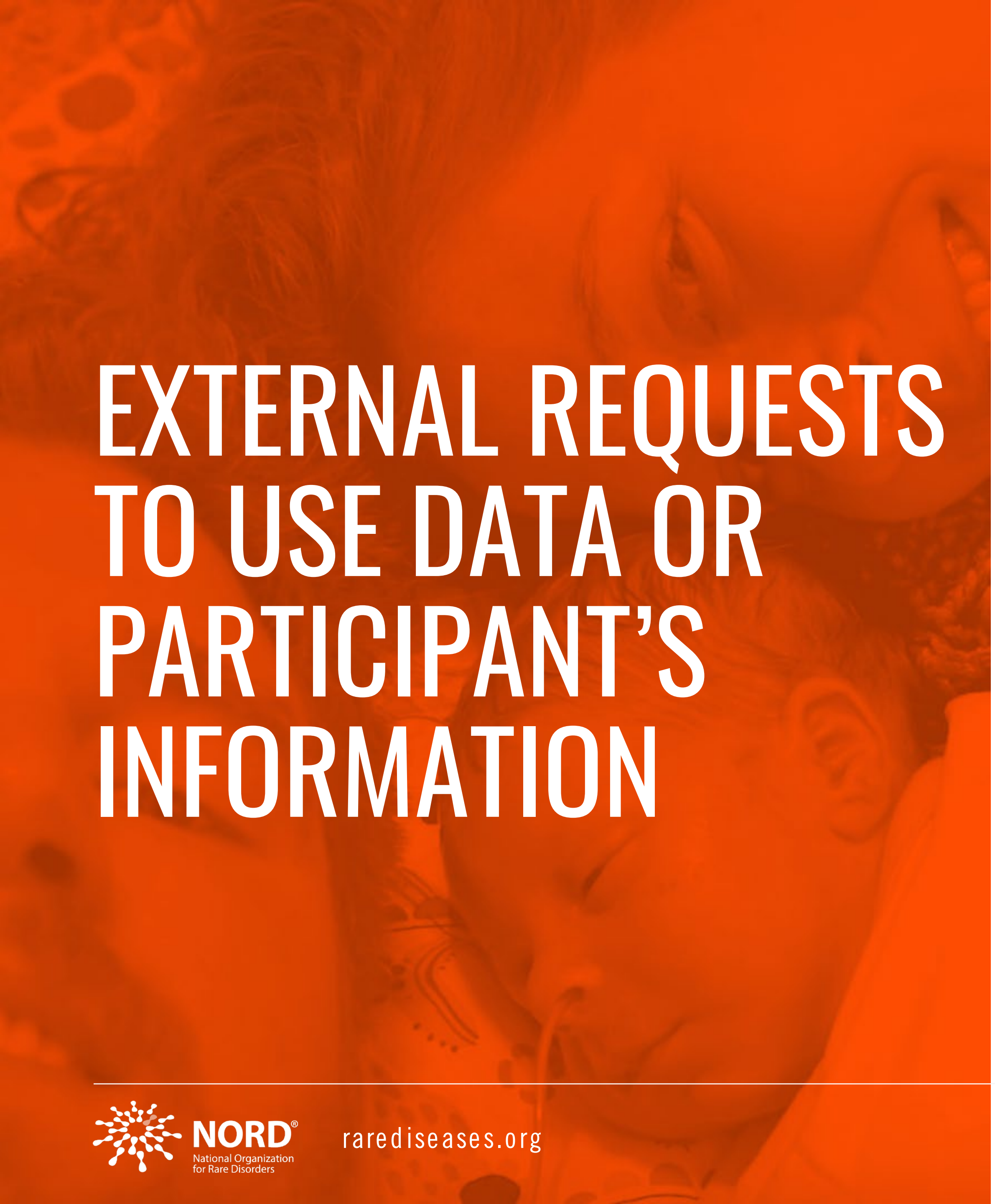
- Research takes time, careful documentation and lots of reliable help!
- If there is no funding, committed volunteers may be able to assist in start-up and sustain the research and the organization.
- Training should be sought, online courses available for free, NORD staff members are knowledgeable and extremely helpful.
- Medical advisory boards can be useful in reviewing protocol design, assisting in deciding on whether sponsor studies are right for your organization and helping with interpreting data.
- Please have a back-up plan for when a key member of your team has an emergency or has to step away from the research. Redundancy in research is important to advance the progress of research.

CONSENT

- Consent in a research environment is different than the medical field.
- Voluntariness, which of course extends to medical procedures, is interpreted a little differently in research.
- Research participants can take as much time as they wish, ask as many questions as they have and ask to speak to the Principal Investigator to answer questions as well.
- Research participants can withdraw their decision to consent at any time.
- Consent has to be obtained before any research activities take place.
- Consents must be saved in a secure location.
- Participants have a right to a copy.

Data Sharing

- The consent will detail the extent and circumstances of when data will be shared within your organization, with an organization such as NORD, and which organizations will have a right to see the data as a result of the research oversight.
- Data can only be shared according to the language of the consent.
- The Medical Advisory Board as referenced in external research considerations should have final say when data may be shared.



EXTERNAL REQUESTS TO USE DATA OR PARTICIPANT’S INFORMATION

- Our participants rely on their advocates and organizations to keep their data safe and confidential.
- External investigators and companies may present themselves as wanting to contribute to research on the condition, but we have a responsibility to become involved in only the most scientifically sound and well-designed research.
- This is why we have IRBs, organizations such as NORD and our Medical Advisory Boards. All work together to choose and support you and your constituents.
- Please use your Medical Advisory Boards to review requests from outside entities and then use the IRB to further protect the rights and welfare of participants.

New, Nonprofit IRB

- Some of you may remember Hummingbird IRB which prided itself on flexible pricing and helpful service.
- Hummingbird was purchased by a large, central IRB system and had to assume a new pricing structure.
- My commitment to students, nonprofit organizations and small businesses has brought me to starting a new IRB that will feature a sliding scale approach to pricing and a more traditional fee schedule for industry sponsored research.
- The good news? We will be ready in January to accept research!

North Star Review Services

- Dr. Stephen Rosenfeld and I are joining forces to bring you this new IRB.
- We will be joined by some of the people from Hummingbird IRB so you know that you will receive great reviews and service.
- Stephen Rosenfeld, MD, MBA is the immediate past Chairman of The Secretary’s (Health and Human Services) Advisory Committee on Human Research Protections (SACHRP).
- Former Chairman of Quorum Review Board
- Former President and CEO Western Institutional Review Board

If you have additional questions

Please Contact the NORD research team:

research@rarediseases.org