Patient and Caregiver Outcomes in Rare Disease Service

In partnership with the North Carolina Translational & Clinical Sciences (NC TraCS) Institute, RTI International has established the Patient and Caregiver Outcomes in Rare Disease Service (PCORD) to help researchers accelerate cost-effective data collection on patient and caregiver outcomes in rare diseases. Building on RTI's successful development and application of a survey registry for other diseases, this RTI-based NC TraCS service will help researchers to create useful and flexible survey registries to learn about the real-life consequences of disease.

Overview

Translational research can be hampered by incomplete data on the consequences of a disease for patients and caregivers, and rare diseases are especially vulnerable to this problem. Such information is essential when designing clinical trials to determine benefits from treatment. Clinical researchers seeking this information need access to adequate samples of patients and caregivers, an expensive and time-consuming process. There is a need for alternative ways to find and work with larger populations of patients with rare disorders in order to gather outcomes data unattainable with smaller samples.

Survey methodology provides a cost-effective way of gathering data to supplement or inform data collected directly by medical professionals. Through its previous work, RTI has shown that large-scale survey registries can generate new and accurate descriptions of the disease experience for patients and caregivers. Drawing on this work, PCORD helps researchers to develop registries and surveys for rare diseases so they may gain insight into previously unknown patient and caregiver outcomes.

Services

PCORD can help investigators

- Determine the need, feasibility, and goals of a survey registry
- Identify key stakeholders (advocacy groups, clinicians, researchers)
- Develop and implement a strategic plan to create a registry
- Design graphic materials and images to "brand" the registry
- Recruit registry enrollment
- Create high-quality surveys
- Identify the most appropriate methods and technologies to distribute surveys
- Determine effective modes for survey completion
- Use ethical and effective strategies to maximize response rate
- Develop data analysis strategies to maximize clinical and research utility.

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Tier 1: Consultation and Initial Planning (2–8 hours)

Tier 1 services are designed to determine the need for PCORD services, rationale for a registry, and likelihood of success. The service coordinator will meet with interested investigators to discuss topics such as the disease of interest, any existing registries, local expertise and commitment, existing connections with stakeholder groups, and emerging findings in disease treatment or prevention. The goal of Tier 1 services is to determine whether there is sufficient interest and potential to move forward with planning a registry.

Tier 2: Strategic Planning and Development (10–40 hours)

The goal of Tier 2 services is to provide more targeted support to projects for which there is a clear justification and where success is potentially achievable. The service coordinator will work with interested investigators to develop a strategic plan, including a detailed set of goals, objectives, and strategies. Consultation will be available as needed from key services at the University of North Carolina, Chapel Hill (UNC), as well as subject matter experts in RTI's Survey Research Division and Center for the Advancement of Health IT. PCORD will support and facilitate meetings among investigators and stakeholders to finalize commitments to registry development. Tier 2 services could also include support from RTI's Multimedia Communications Services group to develop a logo, color scheme, or other images to support the vision of the registry. At the conclusion of Tier 2, investigators should have a clear commitment to establish a registry.

Tier 3: Registry Development and Pilot Testing (TBD)

The goal of Tier 3 services is to provide support to create a registry, enroll participants, and conduct a pilot test to provide evidence in support of using the registry as the basis for extramural research applications. A maximum of one to two Tier 3 projects will be supported in any given year. Advancement to Tier 3 is usually contingent on approval from the Translational Sciences Advisory Board or receipt of an NC TraCS pilot study award. PCORD can help investigators with all phases of registry construction and implementation, including support for survey design, cognitive testing to assure quality content, selecting survey methodology and software, creating stationery or brochures to support recruitment, communicating with potential registry members, establishing a database, and programming and launching a survey.

Resources

PCORD draws on a broad range of resources relevant to registry creation and survey development.

- RTI's extensive survey experience is enhanced by UNC's survey capabilities, and the PCORD team guides investigators to the best solutions for each project.
- Survey methodologists can ensure that PCORD surveys reflect best practices for questionnaire design and result in the collection of high-quality data.
- RTI has a history of developing innovative technological solutions to modern survey challenges, including data management systems, mobile computing questionnaire design, and the use of mobile survey technology in low-resource settings.
- PCORD provides support for multiple modes of survey data collection, including person-to-person interviewing, traditional paper surveys, Web-based interviewing, telephone surveys, and mobile applications.
- Resources in design, illustration, graphics, and branding support PCORD by creating visually appealing registry and survey materials.

Leadership Team

Don Bailey, PhD

Don Bailey is a Distinguished Fellow at RTI and Chair of the RTI Fellow Program. His success building a survey registry for patients with fragile X syndrome and their caregivers was the impetus for establishing PCORD.

Lisa Gehtland, MD

Lisa Gehtland is the PCORD Service Coordinator and RTI's Clinical and Translational Science Award Project Coordinator.

More Information

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