Extended Abstract Journal of Clinical Medicine and Therapeutics 2020
Vol.5 No.3

Orphan Drugs 2020: The rare diseases clinical research network (RDCRN) program (a model for collaborative research)- Rashmi Gopal Srivastava - National Institutes of Health

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Any disease which has a U.S. prevalence of less than 200000 is defined as a rare disease. There are several challenges and opportunities for doing drug development and research for rare diseases. To facilitate clinical development and research of treatments for neglected and rare diseases, the National Center for Advancing Translational Sciences (NCATS) has established numerous programs. This presentation will specialise in the Rare Diseases Clinical Research Network (RDCRN) program for multisite clinical research involving patient advocacy groups (PAGs) as research partners and can provide a brief overview of some other scientific programs that support development of therapeutics for rare diseases. To facilitate clinical trials and natural history studies for rare diseases, the Office of Rare Diseases Research (ORDR) within NCATS has established the RDCRN program. The Rare Diseases Clinical Research Network (RDCRN) is a successful, innovative and collaborative international clinical studies network of 22 distinct multisite clinical research consortia and a central Data Management and Coordinating Center (DMCC). The research conducted in this network has involved 144 Patient Advocacy Groups and explores the natural history, epidemiology, diagnosis and treatment of 200 rare diseases at 267 clinical research sites in USA and 17 various other countries. Each consortium is required to conduct two multi-site clinical studies 3 related rare diseases, develop a educational program for brand spanking new investigators involve patient advocacy groups as research partners and provide info about rare diseases to health care providers, researchers, patients and general public. The Data Management and Coordinating Center (DMCC) supports the consortia by supplying infrastructure; user-friendly resources for the Webbased and public referral and recruitment tools; logistical and administrative assistance and data coordination, sharing and management. The goal of RDCRN is to contribute to the treatment and research of rare diseases by working together to spot biomarkers for disease risk, disease activity and severity and clinical outcome, while encouraging the event of latest approaches to diagnosis, prevention and treatment. Some other translational programs of NCATS include New Therapeutic Use program (NTU), Therapeutics for Rare and Neglected Diseases (TRND), the Bridging Interventional Gaps (BrIDGs), NIH Chemical Genomics Center (NCGC) and Tissue Chip or Microphysiological Systems (MPS) program.

The pace of research in rare diseases has been accelerated by the Rare Diseases and Clinical Research Network. The time from study concept of initiation has shortened, attesting to the streaming of the process, the availability of key infrastructure, and the dedication of the partnership among investigators, the NIH, and PAG members. Protocol development time compares quite favourably to the results reported by the National Cancer Institute on cooperative group protocol development, which has been under way for how longer time.

Establishing the patient CR has become a serious initiative of the RDCRN. Connecting with patients online is a important and novel way to reach patients beyond the referral catchment of major academic centers. Increased access to participants in ongoing research promotes equality of representativeness and access in medical research, provides opportunities for patients to interact with disease experts, and increases cohort sample sizes. This is particularly important for rare diseases, where access to experts are often limited and study sample sizes tend to be small. The opportunity to promote awareness and education about research in rare diseases expands the potential research community, and is important for both study subject retention and recruitment. The RDCRN includes several proven samples of its successful utilization of the CR to gather data directly from patients to conduct novel research studies that address questions of importance to patients. Features of the CR that make it such a useful gizmo are

1) Only the DMCC IRB approval is needed, since the DMCC's university is the host of the website, obviating the need to obtain multiple IRB approvals, as is necessary in multi-institutional studies;

2) The DMCC has HIPAA approval to collect patient identifiers and contact information permitting two-way communication;

3) An IRB/HIPAA-approved feature is the ability to share information with RDCRN investigators (and more recently with PAGs) at the discretion of the registrant, facilitating the linkage between patient and investigator to boost protocol enrollment.

The data from all of the studies conducted by the RDCRN consortia are collected during a database maintained by the DMCC, making it a valuable resource for future research. International data standards and recommended common data elements are wont to provide a basis for comparison with other studies. Numerous rare disease clinical research network studies have related clinical observations to patient quality-of-life outcomes, and reflect a growing interest in incorporating patient-reported outcomes in both clinical natural and trials history studies. By every measure, the Rare Disease and Clinical Research Network continue to be a successful model for rare-diseases research.

This work is partly presented at Annual Congress on Rare Diseases & Orphan Drugs October 26-27, 2016 Chicago, USA

The RDCRN response to the challenges of conducting health outcomes research in rare diseases has been to make multi-institutional consortia, including sites in 14 countries, that specialize in specific diseases, and employing technologies that are scalable, extensible, and generalizable to provide a common set of tools to facilitate research through the DMCC. Those tools permit a good range of study designs, safety monitoring, biospecimen tracking, research pharmacy drug inventories, image collection, mobile and interactive voice technologies, and extensive study monitoring and analysis. The embodiment of common data elements and standards in the RDCRN enables the exchange of information across studies and across diseases, enhancing the value of the accumulating data to the larger scientific community.

The CR permits ascertainment and comparison of practice patterns and outcomes achieved within the consortium centers of excellence and the broader community at large. While the CR has proven to be an efficient and useful tool, it is not without its limitations. Because it is ascertained directly from patients and their families, the information that can reliably be collected may be limited or at least subject to interpretation. While it are often argued that those suffering from rare diseases become expert in their disease and its outcomes, research that depends directly upon patient reporting must be carefully designed.
With this caution in mind, the will to integrate patient-reported outcomes into the conduct of clinical trials makes it imperative to develop instruments applicable to those disease settings and to find efficient ways to administer them. The challenge for the longer term is to still adapt these technologies to reinforce research in rare diseases

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