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Providing Meaningful Outcomes for Rare Disease Patients and Clinical Developers

Successful rare disease studies demand different approaches on a number of levels. Early access studies for patients who are living with life altering of rare diseases call for differences regarding study design, regulatory considerations, patient support in study conduct, and an eye to early phase translational medicine techniques to provide successful patient-centric study outcomes.

Fast to patient studies can be broadly based, however at the heart of these studies lie precision techniques pulled from research science and applied to the clinical development realm, inherently valuable for rare disease studies and patients who seek to enroll.

Serving the Patient Who Needs Early Access to Treatments and Achieving Meaningful Outcomes

JUN 20

Medpace VP, Medical Affairs, Richard Scheyer, M.D., will present insights into this challenging aspect of early phase studies, at the 2017 Annual DIA Conference, Tuesday, June 20, at 10:30 am, in Chicago.

Dr. Scheyer will discuss the intersection of patient centricity with sound clinical data.

Session Focus: As most rare diseases have no approved therapies, these patient populations are frequently desperate for any kind of treatment. As a result, they request earlier and more extensive access to investigational drugs before many of the safety and dosing questions have been addressed. This could result in erroneously high adverse event profiles or subjective benefits that skew the development program. How do we better define how to broaden access while simultaneously maintaining rigid control of the data?

Visit Medpace at DIA at Booth #1118

Patient-Focused Advocacy

Patient centricity in trial design is challenging for rare disease patients. All aspects of a study, patient recruitment, accommodations, and other aspects of patient care must be carefully considered. Access to patient advocacy groups are key to conducting most rare disease studies.

Before regulatory authorities recognized the importance of drug development for rare disease — 1980, US — only 10 compounds were approved. Globally, organizations such as the National Organization of Rare Disease (NORD), US; CORD – Canada; and EURORDIS, Europe, have been active to help establish an international focus on rare disease, which in turn has resulted in regulatory action to expedite research. These groups have driven international cooperation between regulatory bodies, investigators, researchers, and patients to develop common guidance for study approach, registries building, and other key tools to connect researchers and patients. Patients with rare diseases can find support systems, patient communities and news about their disease. Researchers can understand the challenges these patients face and adapt programs to drive enrollment and retention.

Fast to Patient Study Design

In the case of drug development, innovation exists from the translation of non-human research findings into innovative therapies for patients with chronic diseases. Traditional clinical development, in many chronic disease states can take decades. In these cases the failure rates and sheer expense can be prohibitive. With the advent of genomics and early phase study design, benefit for patients with the disease can be achieved early on with bench to bedside techniques. Scientists must seek answers utilizing techniques that bridge the research lab with clinical medicine.

Focusing on Rare Disease Patient Centricity for Better Outcomes

Bridging the divide between clinical outcomes and patients who require early access raises many issues. Singularly, "Are patient centricity and meaningful clinical outcomes mutually exclusive?"

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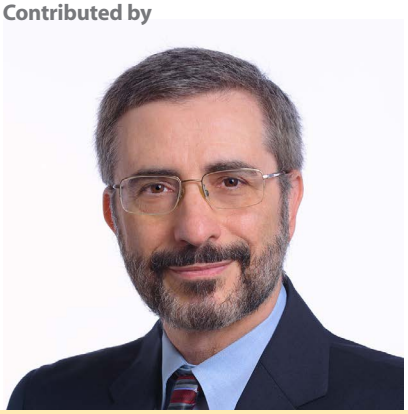

RICHARD SCHEYER
VP, Medical Affairs
Medpace

Rare disease studies call for different approaches in patient recruitment and retention. Medpace uses a broad set of tactics, ensuring a patient-centric approach. These tactics are inclusive of study feasibility innovation as the site selection process progresses. This may include tapping advocacy groups, using vendor partnerships, or analytic databases to determine a best course of action. Rather than sending automated feasibility questionnaires to sites, Medpace would do literature searches, identify key opinion leaders who have information on specific indications, and reach out to specific patient advocacy groups to really understand what is involved with patients who have to live with these life altering disease states on an everyday basis. Best practices should include scheduling one-on-one calls with key investigators. And most often sites for these patients are local sites and as a consequence, demand different study start up initiatives. Rare disease studies demand personal attention to sites. These studies are truly customized and cannot be regarded as a "one-size-fits-all" approach. PV

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