Science 37

INSIGHT BRIEF

Powering Rare Disease Research with Agile, Decentralized and Patient-Centric Approaches.

The increased adoption of more agile, decentralized and more patient-centric approaches is a very welcome trend for patients and providers in just about every therapeutic area, including the spectrum of rare disorders for which clinical trial participation presents significant challenges to patients, their families, and their caregivers. By eliminating barriers, leveraging technology or bringing a clinical study into a rare disease patient's home, researchers can remove many of the unique, logistical challenges that limit the amount of eligible patients and qualified investigators — while also ensuring biopharma companies and Clinical Research Organizations (CROs) with a more patient-centric study flow to generate data efficiently and securely.

Here are key take-aways from a recent Science 37-hosted rare disease focused webinar:

1. Agile and decentralized approaches are here to stay

Even prior to the pandemic, the industry was moving toward a more patient-centric model from the traditional model where patients are screened and enrolled at a limited number of sites to help reduce (if not eliminate) travel time and other logistical barriers for participating in a clinical trial. And as we look ahead at continuing research in the post-pandemic era, an agile or hybrid model leveraging patient-friendly technology and remote study visits will surely emerge.

An agile model is particularly appropriate for rare disease studies, and a recent survey of rare disease patients and families by Xperiome confirmed that most would prefer to participate in a clinical trial through a mix of clinic- and home-based activities. The same study also showed that although the vast majority of rare disease patients are interested in taking part in a clinical trial, they cite not knowing where to look for information as a significant barrier to participation.

2. It's not an all-or-nothing approach

Because of the unique manifestations and characteristics of rare disorders, clinical trials within these patient populations will almost always require a unique agile or hybrid model; with some functions conducted remotely through decentralized methods and others conducted through traditional site or clinic visits. A study utilizing remote coordinators, for example, could potentially include additional decentralized elements such as eConsent and mobile nurse management to optimize clinical workflow, while also including scheduled site visits for medication dispensing and specialized assessment more conducive to in-person examination. Again, this agile or hybrid model and traditional, site-based components is here to stay — and will increasingly gain traction not only in rare disease research, but in other therapeutic areas as well.

3. Early input from patient advocacy groups is most effective

To fully implement a technology-led, patient-centered clinical development model for rare diseases, biopharma sponsors must include patient advocacy groups in the very early stages to advise on trial design and patient-important endpoints. Because natural history information for rare disorders is often so limited, input from disease-specific patient advocacy groups can help illuminate appropriate endpoints, and whether such data — including blood draws and lab tests — can or ought to be collected remotely.

4. There's work left to do

As with other new technologies introduced to help optimize clinical trial operations, improving the overall uptake of agile and decentralized tools and methods will take time, education, and evidence. It will take time to get investigators and site staff comfortable with new systems; it will take an effort to educate patients and their families on how they can participate in clinical research from their own homes; and it will take evidence to assure sponsors and regulators that generated data is of comparable quality to — and can be integrated seamlessly with — data from traditional sites or clinics.

5. Biopharma sponsors and CROs need quality assurance… and help with operationalizing agile clinical trials

According to an Industry Standard Research (ISR) survey of biopharmaceutical executives commissioned by Science 37, more than 80% expect their company to conduct a clinical trial using at least some decentralized elements this year. The study also found broad consensus for a hybrid model, as less than one-in-six of these trials are expected to be fully virtual.

Of concern, however, is that nearly 60% of biopharma executives say that their organization does not have the internal capabilities to operationalize any components of a decentralized clinical trial; thus establishing a familiar conundrum of whether to build internal infrastructure to coordinate and integrate into clinical trial operations, or to outsource these activities to any number of service providers.

Since its inception, Science 37 has led the development of decentralized clinical trial technologies and methods, assembling patient and investigator networks, and managing the voluminous flow of clinical data collected virtually. Led by coordinated expertise, Science 37's Operating System can orchestrate workflows, generate evidence and harmonize data — across multiple therapeutic areas, including rare diseases — while offering clinical research professionals with data of unmatched quality.

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