Tim Rich
Vice President, Digital and Decentralized Solutions, PPD Clinical Research Services, Thermo Fisher Scientific

Decentralized Clinical Trials Bring Research to the Patient—in More Ways Than One

DCTs proving to solve numerous pain points for patients in clinical research

One perennial challenge to running a successful clinical trial is low patient participation and retention, which invariably impede timely data collection. Among the many factors contributing to low participation are lack of awareness of clinical research as a treatment option, the fear of unknown outcomes, inability to meet inclusion/exclusion criteria, or the burdens of required travel. In-person site visits may rule out would-be participants who have prohibitive work and family demands, transportation challenges, or fragile health.

The recent proliferation of decentralized clinical trials (DCTs) expands the potential of drug development, making clinical research a more viable possibility for more participants. Increasingly sophisticated remote digital tools and the demands of a global pandemic have powered the existing shift toward DCTs. In the decentralized model, the physical structures of the traditional trial are reimagined to bring clinical research to the patient, meeting them where they are and making participation as easy as possible.

From virtual and mobile sites to wearables, telehealth and eConsent, virtual tools are reducing complexity and patient burden to greatly broaden access and outreach to anyone, anywhere who wants to participate. This can be especially true for patients with rare diseases and those in underserved or underrepresented populations.

We generally think of the decentralized trial model as bringing the clinical trial into the patient's home, when in fact that's only one among many possible configurations that will be discussed in this article.

Accelerating rare disease research

The paradigm shift to DCT supports rare disease research particularly well. Rare disease indications are by nature challenging to study, with unpredictable symptoms or triggers with short turnaround times. Patient populations are typically small and geographically dispersed, making the traditional large-scale, site-based trial burdensome for both patients and caregivers—everything from expenses and complicated travel logistics to potentially hours-long travel that might exacerbate symptoms.

An unusual hurdle for rare disease research is that many patients come to identify themselves in terms of their conditions. Lacking treatment, those with unmet needs have had to learn to manage things on their own. In some cases they've structured their entire lives around managing their symptoms and have joined communities of others with the disease simply to cope. Being asked to participate in research that challenges their routine can both fill them with hope and stir fears of yet another disappointment. The prospect of a research channel that might offer life-changing relief is unimaginably significant.

In a global ultra-rare disease trial we supported, one patient's situation resulted in DCT opening the door to treatment. Telemedicine, eConsent and other virtual tools already were built into the study. But the patient didn't want nurses or other health care staff in the home, so we took all the elements of the standard brick-and-mortar research site and recreated them in a mobile site solution inside an RV, which parked outside the patient's home. The RV—outfitted with treatment rooms, toilets and waiting areas—brought the trial directly to the patient's doorstep.

Choosing from a menu of flexible tools

As the previous example illustrates, decentralized trials are demonstrating that there's more than

one way to collect the clinically relevant data required to support new drug submissions. An innovative, dynamic protocol and process can offer critical elements in a virtual format—evolving to keep the patient experience at center, all while allowing the study team and health care providers to stay connected without on-site visits.

Success rests on breaking down the physical fundamentals of a trial into a decentralized or virtual structure, taking advantage of a comprehensive menu of remote clinical resources and innovative shared technologies.

- **Community facilities:** Making use of local pharmacies, clinics and other health hubs as convenient alternative locations for testing, bloodwork and the like.
- **Telemedicine:** Easy-to-use digital tools enable patients to stay connected with their doctors. Sites are able to continue their standard of care, reduce protocol deviations and collect quality data.
- **Patient communication:** Available technologies make it easier to maintain contact with patients by sending upcoming patient-reported outcome reminders, further ensuring timely data collection. Information also can be shared with the patient about the status of the study drug or treatment—as well as how many patients have benefited—which may encourage continued participation.
- **Direct-to-patient shipping:** If and when patients are unable or unwilling to visit a site, clinical materials such as supplies and investigational medicines can be shipped to—or collected from—the patient's home.
- **Documents/eConsent:** Using tools allowing patients to securely review and sign required study documents from the comfort of their home helps keep timelines on track, while ensuring patients are constantly informed and in compliance.
- **Home trial services (HTS):** A network of nurses and other medical professionals that come to the patient's home to prepare and administer study drugs, conduct clinical assessments, ensure eDiary compliance and many more procedures normally restricted to the clinic.
- **Direct data collection:** Electronic clinical outcomes assessments (eCOAs) and electronic patient-reported outcomes (ePROs) use digital platforms to enable direct data collection from study stakeholders. This collection method yields less paper, more timely data and greater consistency.
- **Remote monitoring:** Enabling clinical research associates (CRAs) to use remote tools for generating data analytics, conducting remote source data verification (rSDV) and performing other monitoring activities.
- **Wearables:** Sophisticated devices and wearable technologies generate much higher levels of clinical data than traditional trial models. Tools such as smartphone and tablet apps, sports watches, meters and monitors, garments and textile patches can be more convenient and less intrusive for patients.

In a recent oncology study, we developed a dynamic protocol to enable the investigator and patient to choose from a menu of DCT solutions. The protocol enabled the PI and patient to determine the best method for that particular patient, within the parameters of the approved protocol. This included deciding whether the patient went in for a site visit every few months or whether we managed those visits in a decentralized manner, such as in the patient's home following the signed DCT consent. This approach was instrumental in securing participation—no small feat for a study that spanned more than seven years.

Improving the patient experience

DCTs allow drug developers to do everything possible to optimize the patient experience with minimal delays and inconvenience—whether patients visit a local care hub or a physician or a nurse makes in-home visits. We have found that adding a patient concierge—a study team member who is a dedicated resource for patients and their families—is another way that study teams can elevate the research experience, keep patients engaged and ensure that all data and materials are handled appropriately and collected on time.

The concierge serves as a guide for patients throughout the trial, organizing everything from travel to reimbursement of expenses and coordination of home health nurse visits. They also can provide reminders about appointments and patient-reported outcomes (PROs) requirements

and can provide technology support when necessary. Having a single point of contact can be a game changer, especially in rare disease studies where patients may invest years of their time before receiving a correct diagnosis. A lack of steadfast engagement while a trial is underway leads to a higher risk of patient dropout.

Ensuring more trial diversity

Minority groups are even less likely than the average patient to participate in clinical trials, contributing to underrepresentation. In just one example, African Americans comprised less than 5% of the participants in trials for 24 oncology drugs approved by the FDA between 2015 and 2018.1 These included trials that targeted cancers disproportionately affecting African Americans. Patients in oncology studies sponsored by the National Institutes of Health (NIH) were required to travel an average of 40 miles to study sites, creating a burden that may be more acutely felt by minority and low-income patients and their caregivers.1 Removing such a barrier to participation could improve diversity in oncology and other DCTs.

Additionally, fully decentralized trials naturally lend themselves to new recruitment strategies, leveraging channels such as online ads, social media and enhanced engagement with patient advocacy groups. With powerful digital tools, developers can tailor messaging and track the effectiveness and reach of specific outreach. They can also easily assess the demographics of potential participants and change course to achieve broader diversity. For example, a study could turn off channels dominated by white and middle-aged male respondents and shift to other channels that target under-represented communities. Such a change in course can be done in minutes versus the months it might take to reorient to different populations in a traditional trial.

In summary

As sponsors evolve to better address the myriad challenges of drug development, the decentralized trial is rising to the top as the most flexible and effective model. More than being just an alternative option, it presents an opportunity to disassemble traditional clinical trial assumptions and entirely reinvent how patients participate. Done correctly, DCTs stand to offer easier recruitment, more robust data collection and ultimately faster speed to market, all by keeping the patient at the core.

Reference

1. Terry, Mark. "Pushing for More Ethnic and Racial Diversity in Clinical Trials." *BioSpace*. 12 Jun. 2020.