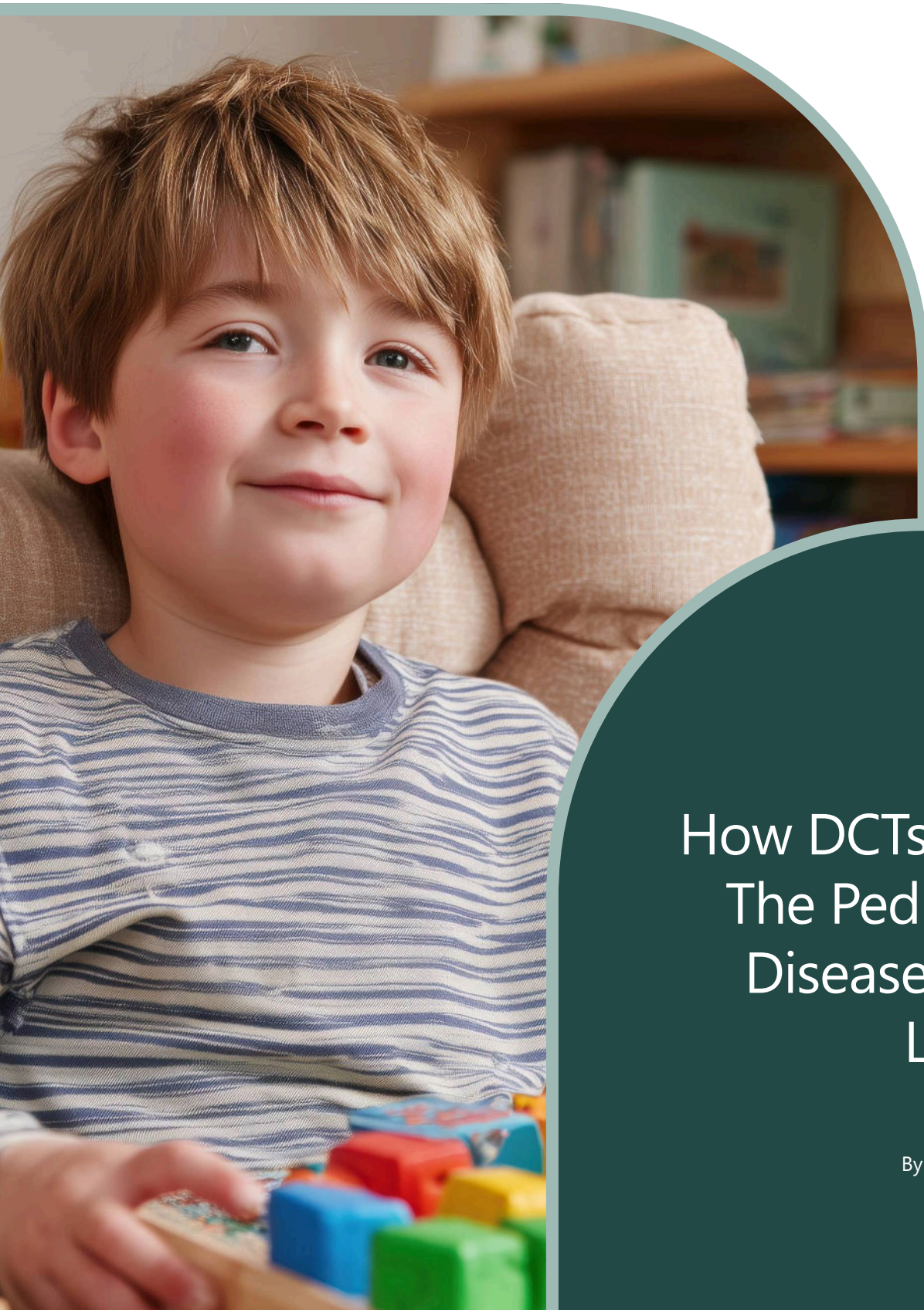

How DCTs Can Shift The Pediatric Rare Disease Research Landscape

By Medical Research Network

Rare diseases affect an estimated 300 million people globally with approximately 70% of those conditions beginning in childhood, yet fewer than 10% of rare diseases have an approved therapy.[1] Further, if we look beyond just rare diseases, just over 25% of new drugs approved between 2011 - 2023 were approved for pediatric use.[2] The message is clear – even though medical innovation continues to accelerate, pediatric research has struggled to keep pace.[3]

Children living with rare diseases often face a difficult, uncertain path: long diagnostic journeys punctuated with uncertainty, limited treatment options, and complex care routines that touch every aspect of family life. Traditional, site-based clinical trials exacerbate these already tiresome challenges faced by pediatric patients, their families, and caregivers and often require additional or extended travel and time away from school.[4] This makes trial participation beyond reach for those who may benefit the most.

Researchers and Sponsors need to look beyond "the patient" – they need to see that patients are sons, daughters, siblings, and classmates whose daily lives are frequently interrupted with hospital visits, missed school days, and constant travel for treatment. **An urgent paradigm shift is needed: designing trials around the child, not the site.**[5]

The Unique Landscape Of Pediatric Rare Disease Research

Time and timing can be critical for rare disease patients. Time can be of the essence when it comes to diagnosis and treatment – if standard of care treatment is even available. Access to treatment early can play a critical role in slowing the condition down and limiting/delaying symptoms.

For the majority, participation in clinical research represents the only opportunity treatment. Yet as symptoms develop, worsen, or change with age, eligibility for participation can shift rapidly – which often means the window for participation in a trial can close quickly.

For those that do meet strict protocol inclusion criteria, participation can be a challenge, for patients, parents and siblings alike.
Parents frequently describe the process as a second full-time job, involving repeated visits, strict scheduling, and high emotional stress.[4,6] This reality disproportionately affects families in rural areas, single-parent households, and low-income populations.[6] This is compounded further for children that have severe disabilities or medical fragility, existing barriers become compounded. Travel in particular, over short or long distances, may be nearly impossible for children reliant on ventilators, feeding tubes, or mobility aids.

Despite these challenges, recent surveys reveal growing optimism. According to the 2025 CISCRP Perceptions & Insights Study, patients and families are more open than ever to participating in trials—but expect them to adapt to their lives, not the other way around.[7]

Barriers To Access & Participation

Children are not simply "small adults." Their bodies metabolize therapies differently, their daily routines center on family, school, and social development, and their caregivers bear the brunt of the logistical, operational, and emotional weight of care.[8]

These factors multiply when faced with rare disease conditions: specialist visits may occur far from home, caregivers may lose workdays, and siblings may be affected. The logistics of participation become a barrier before the first consent form is signed.[4,6,9]

It's important to not only acknowledge that rare disease pediatric patients and their families are facing these barriers, but to also examine them, and then consider how to address them. Key considerations should include:

- **Distance & Travel Burden:** These can pose critical obstacles as families may have to travel hundreds of miles to reach a site. This in turn means time off work, school disruption, sibling-care challenges, and transportation costs. In rare-disease contexts this is magnified because specialist sites are limited.[10]

- **Trial Design Misalignment:** This is another hurdle, with protocols built on adult-centric assumptions that often ignore school schedules, require frequent site visits, or impose rigid monitoring windows. The child's world of class, lunchtime, siblings, and family routines is rarely accommodated.[5]
- **Consent, Trust, & Information Gaps:** These can weigh heavily. Parents may be eager to enroll their child, but face consent documents filled with complex terminology and limited opportunities for discussion. Mistrust of pharma or fear about side effects remain commonly cited barriers.[11,12]
- **Lack Of Representation & Evidence:** These gaps also further reinforce the above mentioned fear or mistrust and misunderstandings. With fewer pediatric or rare-disease trials completed and published, clinicians and families lack the evidence base that supports confident patient enrollment.[12]

Each of these factors impacts recruitment and retention – and in pediatric rare-disease trials, every missed visit or withdrawn child jeopardizes not just the trial, but the potential for new therapies.[13]

Centering Trials Around The Child

A 2024 overview of systematic reviews found that just one in ten eligible children are recruited for pediatric clinical trials.[13] To improve trial access and equity, protocol design must shift from "bring the patient to the site" to "bring the trial to the patient." Decentralized and hybrid trial models offer practical solutions, and by bringing elements of the trial closer to the patient, sponsors can dramatically reduce barriers to participation.[14]

These can include:

- **In-home / Community-based Visits:** Experienced healthcare professionals (HCPs) perform procedures – traditionally done at the clinical trial site – at home, school, local or satellite clinics. This immediately reduces travel, school disruption, and caregiver burden. For example, MRN's Home Trial Support solution has been applied in rare-disease contexts, supporting flexibility and reach and increasing enrollment and retention.[9,10]
- **Telemedicine & Remote Monitoring:** eConsent, ePROs, wearable sensors, and teleconsults allow children and caregivers to engage from home, on their schedule, with fewer in-person site visits.[10,14]
- **Flexible Scheduling:** Evening or weekend visits and/or local settings mean children miss fewer school hours, and families manage care more easily. For example, satellite and physical sites like those of VCTC, part of MRN's global Site Network, can be implemented in such a way that the trial is effectively right on the doorstep. This can allow for a clear physical and emotional separation between the child's "everyday" and "patient" lives, which can help alleviate stressors and allow them, and the family, a mental "break" from their condition and all it entails.[6,15]

These solutions reduce operational and emotional burdens and create space for children to live more of their lives while contributing to research.

Moreover, when protocols adapt to family context, and when caregivers and children are treated as partners in research, the result is deeper engagement, better data, and stronger outcomes.[7,10] Decentralized trial solutions are not just about bringing the trial to the patient's home and reducing logistical burdens, but also strengthening the family-researcher connection – fitting the trial around the family's needs.

Moving Toward The Future Of Pediatric Rare-Disease Trials

As the industry evolves, the message has become clear: the future of pediatric rare-disease research is flexible, equitable, and patient-centered.[16] Regulatory landscapes are also adapting with the European Medicines Agency guideline (ICH E11 Rev.1) emphasizing that children must not be left behind in therapeutic development.[17]

Sponsors and CROs must convert that intention into design. Decentralized and hybrid trial models are not optional add-ons, rather they are essential enablers for pediatric rare-disease trials. By integrating in-home visits, remote data capture, and scheduling flexibility, trials become accessible to children across geographies and conditions.[6,8,10]

It's time to see trial participation not as a burden but as a collaboration with children and families. The site is no longer the sole clinic – it may be the home, school, or local health center. The visit is no longer a full day away – it could be a short evening check-in.

Conclusion

For children with rare diseases, the opportunity to participate in research often means hope – for therapies, for evidence, for inclusion. But hope alone is not enough: accessibility, design, and experience matter. Decentralized and hybrid trial models bring research into children's lives rather than taking children out of their lives.[9,13]

By embracing in-home visits, community site locations, mobile logistics, and remote monitoring, the industry can deliver trials that are not only feasible but meaningful and crucially, that work for the child and their family.[9,10]

When we design trials around normality, flexibility, respect, and partnership, we unlock participation. We unlock retention. We unlock evidence. And ultimately, we unlock hope for children and families living with rare disease conditions.

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About MRN

MRN accelerates patient recruitment and improves patient engagement and retention through site-centric and patient-centric solutions.

As an innovative market-leader, MRN provides customized solutions to optimize each individual protocol and create more flexible, efficient and accessible clinical trials that deliver accelerated timelines.

Through integrated in-home visit delivery and a vast global network of trained, research ready sites, all empowered by MRN's digital solutions, MRN engages with and empowers diverse communities around the world to participate in and advance medical research.

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Our Offices

UK
Medical Research Network Ltd
Talon House
Presley Way
Milton Keynes Buckinghamshire
MK8 0ES
United Kingdom

USA
Medical Research Network Inc. 540
Lake Cook Road
Suite 400
Deerfield, IL 60077
USA

France
Medical Research Network France
Part Dieu Plaza
93 rue de la Villette
69003 Lyon

Germany
Medical Research Network Germany GmbH
Zettachring 12A
70567
STUTTGART
Deutschland

Spain
Calle Francos Rodriguez
N:51 Chalet 25
28039
Madrid
España

Japan
32F Shinjuku Nomura Building
1-26-2 Nishi Shinjuku
Shinjuku-ku Tokyo
163-0532
JAPAN

enquiries@themrn.co.uk
www.themrn.io