



THREAD is the industry leading decentralized clinical trial technology platform with supporting services that help sponsors and CROs to decentralize clinical research.

Rare Disease clinical studies are ideal candidates for decentralized approaches

In particular, rare disease studies are often more complex than other studies because patients and families commonly need to travel long distances to study sites, including overnight travel or even prolonged relocation.

Given 75% rare diseases have childhood onset, rare disease clinical trials involve not only the patient but entire families. DCTs can help mitigate the obstacles to clinical trial participation, opening up access to more families that can benefit from potentially life-changing therapies.

DCTs Expand Accessibility, Making Trial Participation Realistic for More Patients and Families

Decentralized clinical trial approaches use advanced technologies that allow for clinical research data to be collected remotely. This means that patients with Rare Disease no longer need to make frequent clinic visits in order for study teams to administer study medications or assess their progress and record data. DCT elements that are useful for Rare Disease trials include:

- Virtual visits with study clinicians via Telehealth technology
- Electronic outcome assessments (eCOA) that allow patients and families to submit their data from home
- The use of mobile medical devices (spirometry, blood pressure, pulse oximeters, ECG and more)
- Wearable medical devices that transmit data passively and continuously
- Home nursing visits augmented by DCT technologies

**Rare Diseases affect
< 1 in 1,500 to 2,000
people in the population**

**Collectively they affect
5% of the world's population**

**Up to 80% of Rare Diseases
are genetic in origin**

**50% of all Rare Disease
Patients are Children**

Additionally, Rare Diseases often have different drug development pathways compared to other diseases. In some instances, Rare Disease therapies (particularly those treating ultra-rare conditions) may achieve accelerated market approval based on limited clinical evidence depending on unmet need. In many cases these approvals will consider real-world evidence as a part of the approval. Examples of study types common in Rare Disease drug development that are particularly strong candidates for decentralization include:

- Natural History studies
- Disease or Product Registries
- Expanded Access Programs
- Long-term follow up studies
- Post-approval observational studies





Key Benefits of DCTS for Study Participants

- Reduce the travel burden for families and caregivers
- Allow patients with mobility challenges to benefit from clinical trial participation
- Keep patients safe at home – avoid exposure to healthcare-acquired illnesses, included COVID-19
- Reduce the travel burden for families and caregivers
- Expand access to Rare Disease clinical studies far beyond geographies associated with traditional clinical trial sites
- Remote data collection allows for outreach to a more inclusive patient population

THREAD Research Conducted or Conducting

Almost 60 Rare Decentralized Studies

94% hybrid design

5% fully virtual design

Across 40 countries

with 31% of these in ultra-rare disease

30% pediatric studies

12% in elderly

Across all drug development phases from Phase I to Phase IV

Majority in Phase III stage (50%)

Across multiple therapeutic areas including

Neurology

Hematology and oncology

Endocrine and metabolism

Nephrology

Pulmonary and Respiratory

Cardiology

Musculoskeletal

Dermatology

1 Comprehensive Platform

Leading the industry with a singular, configurable, global, proprietary platform with innovative solutions and technology-enabled services to support every stakeholder's clinical research experience

5x More Inclusive

Dedicated to make clinical research more (1) diverse, (2) convenient, (3) accessible, (4) remote, and (5) transparent for participants, sites and study teams

30% More Efficient

Removing inefficiencies out of clinical research by over 30% through faster setup, automation and reducing costs throughout studies in areas such as removing source data verification (SDV)

"Decentralized Clinical Trial (DCT) approaches are exceptionally well-suited for use in rare disease studies. All stages of rare disease research can benefit from implementing the appropriate DCT approaches that meet the needs of all stakeholders, including patients, caregivers, sites and sponsors. The ability of DCTs to enable participation wherever the patient may be is tremendously beneficial for populations that may be physically challenged to make frequent and demanding clinical visits."

Joyce Moore, Head of Rare Disease Solutions, THREAD



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