Impact of the Trials@Home final recommendations for the DCT field

The research leading to these results has received support from the EU/EFPIA Innovative Medicines Initiative [2] Joint Undertaking (H2020-JTI-IMI2) Trials@Home grant n° 831458.

Perspectives from experts in the field

Today's panelists

Monique Al
Dutch Central Committee on Research Involving Human Subjects. (CCMO)
Clinical Trials Coordination Group (CTCG)

regulatory perspective

Maria Dutarte
European Patients' Academy on Therapeutic Innovation *(EUPATI)*

patient advocacy

Martine Dehlinger-Kremer
European CRO Federation (EUCROF)

clinical research organizations

Martin Johnson
Future Meds Ltd/

site network

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Block 3 – Trials@Home Impact

Trials@Home recommendations from experts outside the consortium

- **Cross-border Trials & Regulatory Harmonization** • Monique Al will address multi-country DCT implementation challenges
- **Patient Training & Engagement** • Maria Dutarte will discuss T@H course materials and patient organization insights
- **Pediatric DCTs & Future Opportunities** • Martine Dehlinger-Kremer will explore regulatory evolution and pediatric applications
- **RADIAL Trial Experience & Site Perspective** • Martin Johnson will share practical learnings from the RADIAL proof-of-concept study

- **Our Goal Today:** Identify key opportunities for broader DCT adoption through diverse stakeholder perspectives

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Clinical Trials with decentralised elements - View of a regulator/ethics repr.

Monique Al
Special advisor CCMO
The Netherlands

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Recommendations on decentralised elements in CTs
from European Medicines Regulatory Network (EMRN)

RECOMMENDATION PAPER ON DECENTRALISED ELEMENTS IN CLINICAL TRIALS:
Published Dec 14th 2022 on Eudralex Vol. 10

DCT Recommendation paper

Direction of EMRN harmonisation

National provisions overview

Member state specific provisions, where national legislation does not currently allow for alignment

First step towards EMRN harmonised DCT approached
Best practice for EU CT authorisation and inspection
Updated as knowledge and experience evolve

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What have we learned?

- Trials@Home analysed and presented the opportunities and challenges in conducting clinical trials with decentralised elements in a structured way
  - Focus on elements which may have a significant impact on scientific validity, data integrity, benefit-risk ratio or the protection of trial participants' rights like: site selection, IMP shipment, technological infrastructure and digitalization, recruitment and informed consent procedure, trial activities performed at trial participant's home
  - Reports, guidances, training to support clinical trial designers, technology developers, and other stakeholders to successfully implement decentralized elements into clinical trials.
- Many clinical trials still rather traditional – slower uptake of decentralised elements than foreseen after the COVID pandemic
- Challenges on adopting innovative design elements: gap between regulatory guidances/ recommendations and operationalization of decentralised elements

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What have we learned?

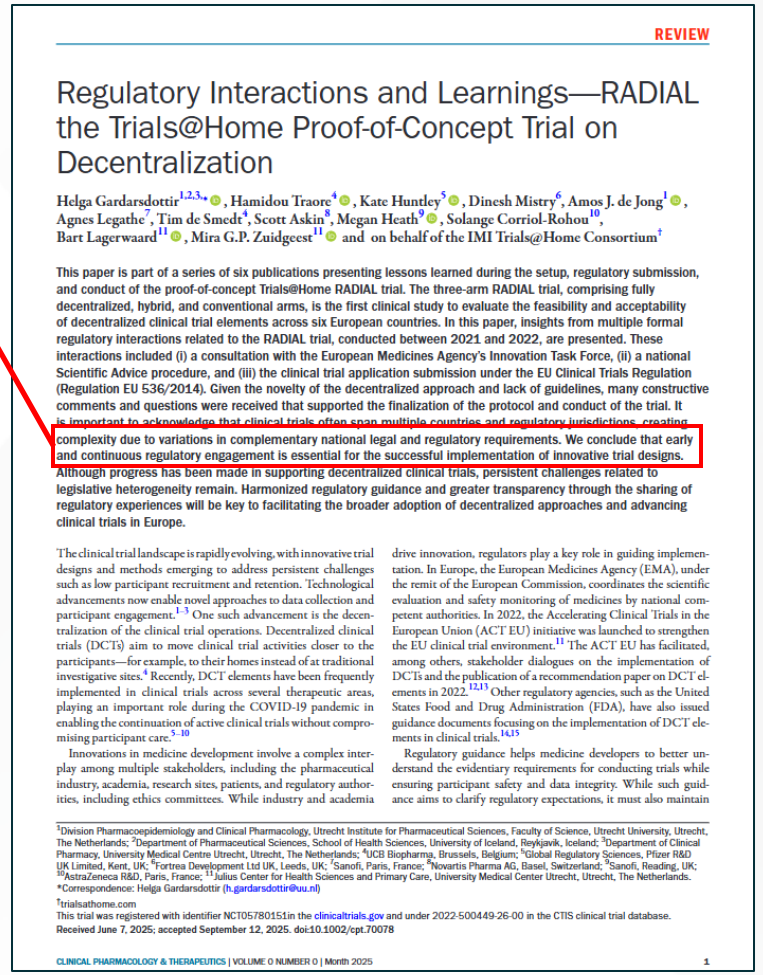
- Early and continuous regulatory engagement is essential for the successful implementation of innovative trial designs.

- Many opportunities for scientific and regulatory advice at EMA and national
- Two ACT EU pilots:
  - CTCG pre-CTA advice
  - SAWP-CTCG

- Use of decentralised elements: fit for purpose with a risk- based approach

- Transparancy and regulatory interactions with MS concerned (NCA and Ethics) may contribute to less regulatory fragmentation

- Update on national provision overview in EU DCT recommendation paper in October 2025 including notice on cross-border trials with decentralised elements



REVIEW

Regulatory Interactions and Learnings—RADIAL the Trials@Home Proof-of-Concept Trial on Decentralization

Helga Gardarsdottir[1,2,3,*], Hamidou Traore[4], Kate Huntley[5], Dinesh Mistry[6], Amos J. de Jong[1], Agnes Legathe[7], Tim de Smedt[3], Scott Askin[8], Megan Heath[9], Solange Corriol-Rohou[10], Bart Lagerwaard[11], Mira G.P. Zuidgeest[11] and on behalf of the IMI Trials@Home Consortium[†]

This paper is part of a series of six publications presenting lessons learned during the setup, regulatory submission, and conduct of the proof-of-concept Trials@Home RADIAL trial. The three-arm RADIAL trial, comprising fully decentralized, hybrid, and conventional arms, is the first clinical study to evaluate the feasibility and acceptability of decentralized clinical trial elements across six European countries. In this paper, insights from multiple formal regulatory interactions related to the RADIAL trial, conducted between 2021 and 2022, are presented. These interactions included (i) a consultation with the European Medicines Agency's Innovation Task Force, (ii) a national Scientific Advice procedure, and (iii) the clinical trial application submission under the EU Clinical Trials Regulation (Regulation EU 536/2014). Given the novelty of the decentralized approach and lack of guidelines, many constructive comments and questions were received that supported the finalization of the protocol and conduct of the trial. It is important to acknowledge that clinical trials often span multiple countries and regulatory jurisdictions, creating complexity due to variations in complementary national legal and regulatory requirements. We conclude that early and continuous regulatory engagement is essential for the successful implementation of innovative trial designs. Although progress has been made in supporting decentralized clinical trials, persistent challenges related to legislative heterogeneity remain. Harmonized regulatory guidance and greater transparency through the sharing of regulatory experiences will be key to facilitating the broader adoption of decentralized approaches and advancing clinical trials in Europe.

The clinical trial landscape is rapidly evolving, with innovative trial designs and methods emerging to address persistent challenges such as low participant recruitment and retention. Technological advancements now enable novel approaches to data collection and participant engagement.[1–3] One such advancement is the decentralization of the clinical trial operations. Decentralized clinical trials (DCTs) aim to move clinical trial activities closer to the participants—for example, to their homes instead of at traditional investigative sites.[4] Recently, DCT elements have been frequently implemented in clinical trials across several therapeutic areas, playing an important role during the COVID-19 pandemic in enabling the continuation of active clinical trials without compromising participant care.[5–10]

Innovations in medicine development involve a complex interplay among multiple stakeholders, including the pharmaceutical industry, academia, research sites, patients, and regulatory authorities, including ethics committees. While industry and academia drive innovation, regulators play a key role in guiding implementation. In Europe, the European Medicines Agency (EMA), under the remit of the European Commission, coordinates the scientific evaluation and safety monitoring of medicines by national competent authorities. In 2022, the Accelerating Clinical Trials in the European Union (ACT EU) initiative was launched to strengthen the EU clinical trial environment.[11] The ACT EU has facilitated, among others, stakeholder dialogues on the implementation of DCTs and the publication of a recommendation paper on DCT elements in 2022.[12,13] Other regulatory agencies, such as the United States Food and Drug Administration (FDA), have also issued guidance documents focusing on the implementation of DCT elements in clinical trials.[14,15]

Regulatory guidance helps medicine developers to better understand the evidentiary requirements for conducting trials while ensuring participant safety and data integrity. While such guidance aims to clarify regulatory expectations, it must also maintain

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This trial was registered with identifier NCT05780151in the clinicaltrials.gov and under 2022-500449-26-00 in the CTIS clinical trial database.
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Patient involvement & patient education perspective

Maria Dutarte
European Patients' Academy on Therapeutic Innovation (EUPATI)

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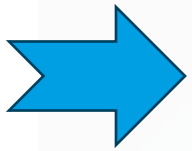
Patient Involvement in medicines R&D

Patients are directly affected by the outcomes of research and development (R&D) of medicines and other health technologies. They also possess **unique experience of living with a condition** and can provide **valuable insights** in different stages of the development of these solutions.

Involving patients in the R&D processes benefits all stakeholders. It ensures relevant research priorities & methods from the outset, improves clinical trial recruitment & retention and results in therapies and health interventions that are better tailored to patients' needs.



Education & training needs among patients and all other stakeholders

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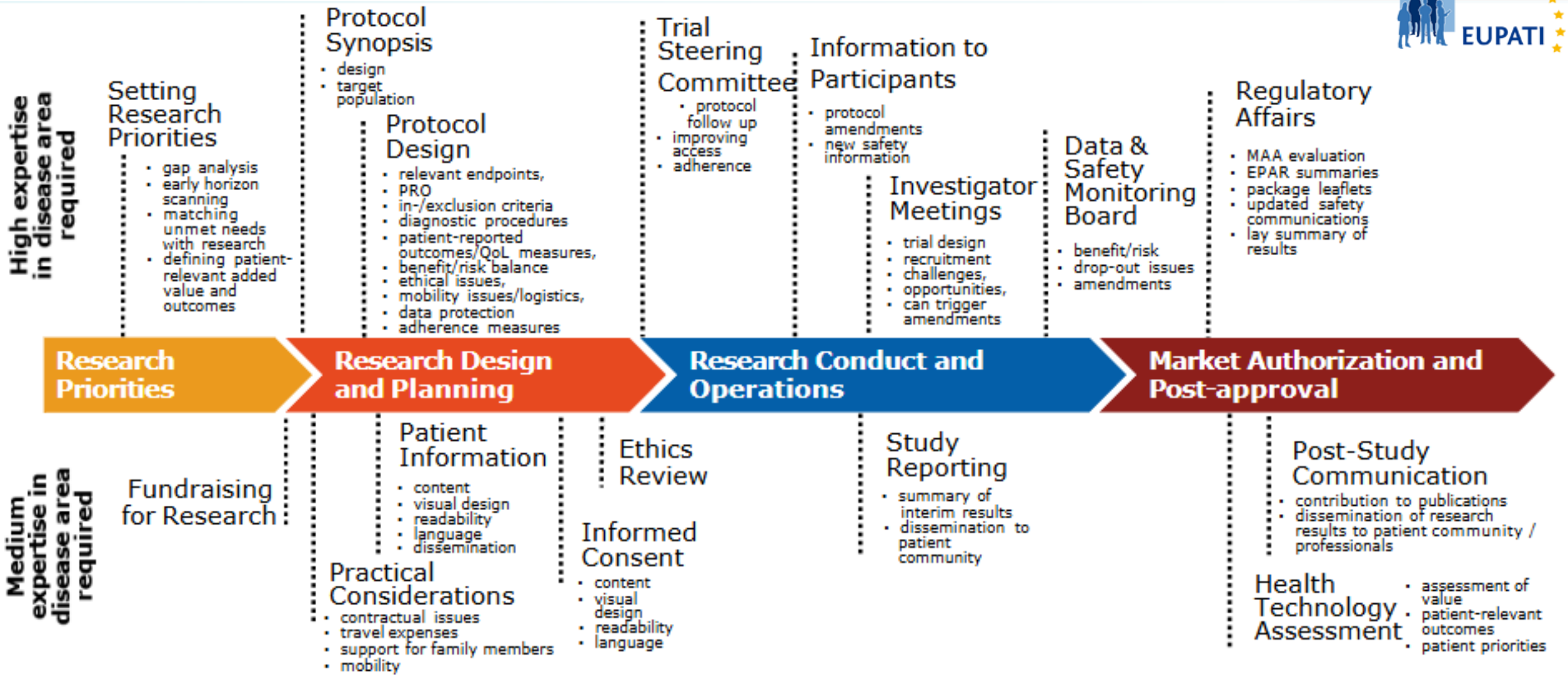

Improving Patient Involvement in Medicines Research and Development: A Practical Roadmap. Geissler, Ryll, Leto, Uhlenhopp, Therapeutic Innovation & Regulatory Science (2017), doi: 10.1177/2168479017706405, and at www.eupati.eu

Trials@Home - EUPATI

- Takeaway 1: Patient involvement in the project itself
- Takeaway 2: Patients' important role in co-design (patient/caregiver preferences), and in recruitment (peer communication)
- Takeaway 3: Training for patients and patient organisations to optimise understanding of Decentralised Clinical Trial approaches (DCTs) in Europe
- Challenge – how to measure the impact of meaningful patient involvement?

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DCTs - Regulatory evolution and pediatric applications

Dr Martine Dehlinger-Kremer
President, EUCROF

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Improving Accessibility, Representativeness, and Data Quality

Enhanced Accessibility & Inclusive Participation

Decentralized Clinical Trials (DCTs) **reduce geographic and logistical barriers**, making participation more accessible to a broader range of patients.

This inclusivity strengthens recruitment—especially for traditionally underserved populations such as **children, older adults, and those affected by rare diseases**.

Real-Time, Reliable Data Collection

Wearables, mobile apps, and remote monitoring tools enable continuous, real-time data capture, enhancing both data richness and reliability.

Technologies must be **validated, user-friendly, and interoperable** across diverse hardware and operating systems to reflect real-world conditions.

It is essential to ensure that devices **do not create additional burden** for participants.

Participant Support & Communication

Provide clear, accessible instructions for all digital tools and procedures.

Offer a responsive **"help desk" or support interface** to assist participants throughout the trial.

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Considerations for the Future – A Patient-Centric Approach

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Shaping the Future of DCTs: Practical Implications from Trials@Home

Trials@Home recommendations are already influencing how sponsors, regulators, and trial sites approach DCTs

Protocol Design	• Sponsors are adopting **hybrid trial models** that combine remote visits, home sampling, and digital endpoints to enhance flexibility and patient engagement.
Regulatory Strategy	• **Early dialogue with regulators** has become standard practice for DCTs, particularly to address data integrity and patient safety. • It is advisable to engage early via the **EMA–HTAb Joint Scientific Assessment (JSA)** to ensure data generated through innovative approaches is acceptable to both regulatory and HTA bodies.
Infrastructure Investment	• CROs and trial sites are **upgrading technology platforms and logistics capabilities** to support remote operations and ensure seamless trial delivery.
Policy Influence	• Trials@Home findings are informing **EU-level initiatives such as ACT EU**, which aim to modernize and harmonize clinical trial regulation across Europe.

TRIALS @HOME

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Lucía's Story: A Case for Decentralized Clinical Trials

Lucía is a 10-year-old girl from Barcelona, Spain.

At age 8, she was diagnosed with Neuronal Ceroid Lipofuscinosis, CLN5 variant (Batten disease)—a genetic, ultra-rare, neurodegenerative condition that primarily affects the nervous system. As of today, there is no approved treatment or cure.

The clinical trial had sites in New York and London. Lucía was enrolled at the London site.

To participate, the entire family had to relocated to London for 8 months—this meant finding new schools for both children, adapting to a different language and culture, and facing the challenges of being far from family and friends during an already difficult time.

Upon returning to Barcelona, some tests had to be repeated, and results from the London site were not shared locally, adding further strain.

Incorporating decentralized trial elements could have significantly reduced the burden on Lucía and her family—allowing them to navigate this difficult period with greater ease, continuity, and support.

TRIALS @HOME

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Thank You

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Dr Martin Johnson

FutureMeds UK
Medical Director

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Top Line Recommendations

- Numerous issues with technology during study: (‘Select & Test Technologies early’)
- Issues with oversight & management of contracted home teams
- In general, needs clarification of process/framework of PI oversight
- Flexibility of Protocols to allow for Home Visits (which in turn needs the flexibility & vision of sponsors)

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What Was Missed?

- Lack of Stipend payments to participants who have home visits
- Will DCT cause a further increase of web sites to log-in?
- Flexibility of Regulatory Approvals to allow for home visits
- Some participants do really like to attend clinic visits!
- Practical aspects of Home Working: dogs, safety, insurance etc
- The effect of DCT on site workforce (talk of ‘fragility of Work Force’)

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A Final Quote…..

“Patients don’t live around research sites. They live in communities, homes, and realities where a one-size-fits-all model doesn’t work. An ecosystem flexes to its environment. Clinical trials should do the same, adapting across sites, homes, and digital touchpoints under one quality system.”

Cameron Glen VP of BD FutureMeds, Oct 2025

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Q&A

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