



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

## Decentral(ised) trials (DCTs): discussion topics across EMA interactions

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Decentralized trials: What is the impact on evidence generation?  
6th EFSPi Regulatory Statistics Workshop 13 September 2021



## Disclaimer

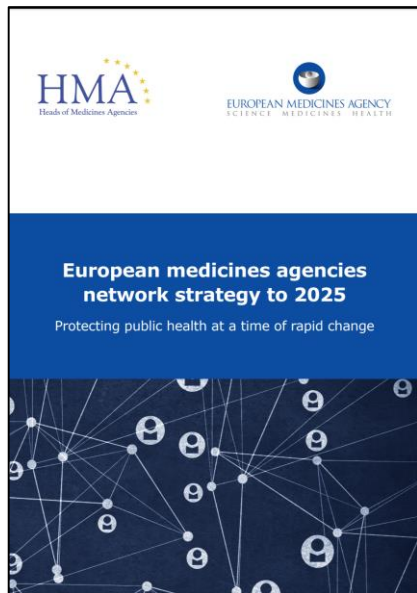
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## Decentral(ised) trials (DCTs) – scope of session discussion

- Clinical trials (interventional trials) executed with the help of telemedicine approaches, mobile or local healthcare providers and/or mobile technologies
- Clinical trial-related activities occur at location separate from investigator's location, e.g. in participants' home or local health care environments



[https://www.ema.europa.eu/en/documents/report/european-union-medicines-agencies-network-strategy-2025-protecting-public-health-time-rapid-change\\_en.pdf](https://www.ema.europa.eu/en/documents/report/european-union-medicines-agencies-network-strategy-2025-protecting-public-health-time-rapid-change_en.pdf)



<https://www.ema.europa.eu/en/about-us/how-we-work/regulatory-science-2025>

## Vision for medicines

“To underpin its mission of protecting human health, EMA must catalyse and enable regulatory science and innovation to be translated into patient access to medicines in evolving healthcare systems.”

**Foster innovation in clinical trials**

- ▶ Work with stakeholders, the EU Medicines Regulatory Network and the European Commission to promote and facilitate the conduct of complex clinical trials and other innovative clinical trial designs;
- ▶ Promote increased information sharing on clinical trial design, conduct, results and best practices. Build on this information and the multi-stakeholder platforms to enable further education, training and sharing of best practice in order to accelerate innovative change;
- ▶ Critically assess the clinical value of new and emerging endpoints and their role in facilitating patients' access to new medicines;
- ▶ Promote the inclusion of neglected populations such as pregnant women, the elderly and those of diverse ethnicity in clinical trials.

**Develop the regulatory framework for emerging clinical data generation**

- ▶ Develop methodology to incorporate clinical care data sources in regulatory decision-making;
- ▶ Clarify questions on data ownership and data security;
- ▶ Modernise the GCP regulatory oversight to enable decentralised models of clinical trials coupled with direct digital data accrual;
- ▶ Develop the capability to assess complex datasets captured by technology such as wearables;
- ▶ Facilitate training and understanding of healthcare professionals and patients to access and participate effectively in such trials;
- ▶ Support the development of robust digital endpoints through qualification, scientific advice, and the establishment of a multi-stakeholder platform to obtain feedback on their utilisation.

## RSS is framework for EMA innovation actions

- Regulatory science strategy (RSS) brings innovation topics together and shows context seen by EMA
- Stakeholders' number 1 recommendation to EMA: Foster innovation in clinical trials
- Regulatory strategy links topics: DCTs, wearables, digital endpoints, PROs, physical functioning, health data ecosystems, ...

p 21 <https://www.ema.europa.eu/en/about-us/how-we-work/regulatory-science-2025>



# Sponsors are welcome to discuss DCTs during development

- Innovation task force briefing meetings
- Business pipeline meetings
- Simultaneous national scientific advice
- EU Scientific advice / protocol assistance (product-specific / broader advice / qualification)
- Paediatric investigation plans
- Marketing authorisation pre-submission meetings with EMA and Rapporteurs

Multi-disciplinary EU approach applied: in-depth involvement of Clinical Trials Facilitation Group, GCP Inspectors, EMA Committees, Working parties and staff

Each interaction contributes to inform regulators' discussions on DCTs

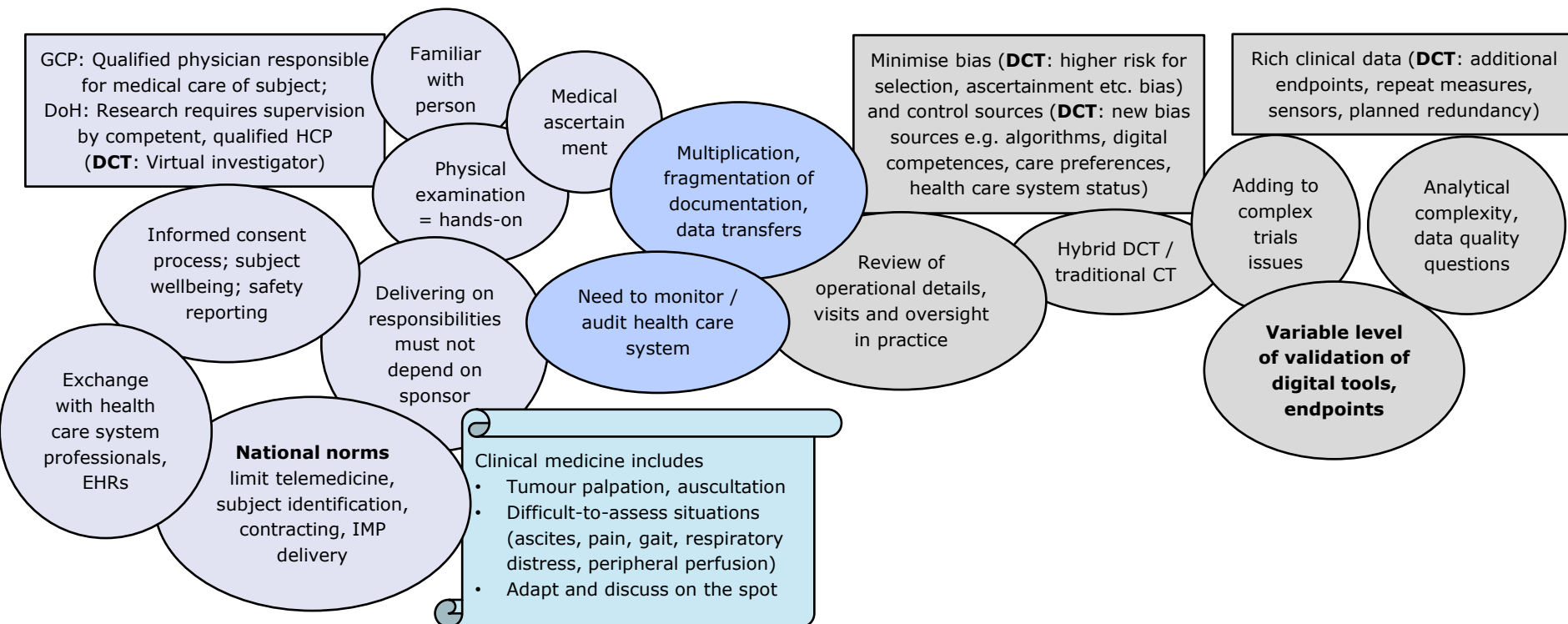


## Recent contributions relevant to DCTs

- ICH: [E8\(R1\) draft](#), [E6 Principles draft](#), E6(R3) Annex-2 on designs that bring trials to patients
- Fergus Sweeney (EMA): Regulatory Perspectives and DCT Implementation Post-Pandemic; Kirstine Moll Harboe (DKMA): DCT – a regulator's perspective (2021-08-25 CTTI event)
- [EMA Guideline on computerised systems and electronic data in clinical trials](#) (draft, 2021-06)
- [Danish Medicines Agency's guidance on implementation of decentralised elements in clinical trials with medicinal products](#) (2021-05-04)
- [EC EMA HMA Guidance on the management of clinical trials during the COVID-19 \(coronavirus\) pandemic](#) (last update 2021-02-04)
- [ICMRA Informal Innovation Network Horizon Scanning Assessment Report – Artificial Intelligence](#) (2021-08-06)



# Potential issues flagged in EMA interactions on DCTs







## For DCTs, fundamental relations such as...

- Subject / patient ↔ Investigator ↔ Sponsor
- Data ↔ CRF ↔ Computerised system ↔ Sponsor's database
- Source documents ↔ Trial master file Investigator ↔ Trial master file Sponsor

...become more complicated, but remain identifiable and this is a basis for learning about and advancing DCTs

# Summary

- In evolving clinical research enterprise, DCTs can address relevant experimental questions
- Traditional RCT useful yardstick, but DCT needs re-implementing scientific questions & principles
- Multi-disciplinary discussions needed about DCTs, with methodologists
- Regulators want operational model and conduct of DCTs to be approached in holistic way
- Trial integrity and data credibility remain difficult to anticipate and evaluate
- Re ICH GCP modernisation: what are critical-to-quality factors for DCTs?
- Trial results need to be informative for regulators, health professionals and patients = need to be onboard in multi-disciplinary discussions, facilitated with estimand framework



# Acknowledgments

- EU Regulatory network and internal experts for comments in interactions on DCTs
- Frank Pétavy, Falk Ehmann, Camelia Mihaescu, Fergus Sweeney, Eftychia-Eirini Psarelli, Maria Antonietta Antonelli, Myriam Chapelin



Sponsors are welcome to use:

- Innovation Task Force: <https://www.ema.europa.eu/en/human-regulatory/research-development/innovation-medicines>
- Scientific Advice: <https://www.ema.europa.eu/en/human-regulatory/research-development/scientific-advice-protocol-assistance>

## Further information

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