Clinical trials update

PCWP-HCPWP meeting
1 April 2025

Presented by Peter Arlett
Head of Data Analytics and Methods Task Force
European Medicines Agency

Clinical evidence 2030: vision

- Patient voice guides every step of the way
- Evidence generation is planned and guided by purpose, data, knowledge and expertise
- Research question drives evidence choice and embraces spectrum of data and methods
- Clinical trials remain core but are smarter, better and faster
- Real world evidence is enabled, and its value is established
- High transparency level underpins societal trust

PERSPECTIVES

PERSPECTIVE

Clinical Evidence 2030

Peter Arlett[1], Denise Umuhire[1,*], Patrice Verpillat[1], Paolo Foggi[2], Ulla Wändel Liminga[3], Bruno Sepodes[4], Marianne Lunzer[5], Brian Aylward[6], Spiros Vamvakas[1], Kit Roes[7], Frank Pétavy[1], Steffen Thirstrup[1], Maria Lamas[8], Emer Cooke[1] and Karl Broich[9]

Building on existing practices, our vision is that by 2030, clinical evidence generation will be further guided by the patient voice and informed by existing data and knowledge; study design will be driven by research questions to be addressed; clinical trials will be more efficient and impactful; real-world evidence (RWE) will be enabled and its value fully established; and trust will be built through transparency (Figure 1).

Excellence of clinical evidence is the heart of every well-informed decision on the development, authorization, reimbursement, use, and monitoring of medicines.

While healthcare decision makers continue to be confronted with unmet medical needs burdening patients and society at large, the slow speed and high cost of medicines development hinder new treatments reaching the patients who need them.

But the healthcare landscape in Europe is evolving and the convergence of several factors now provides the opportunity for a stronger and more sustainable approach to clinical evidence generation. The COVID-19 pandemic has shown the potential of new ways of working, with better collaboration between stakeholders and different approaches for evidence generation and evaluation. The changing policy environment in Europe, including the new legislation on a European Health Data Space (EHDS)[1] and the reform of the EU pharmaceutical regulation,[2] offers opportunities through greater healthcare data access, innovation in study designs, and use of advanced analytics. Increasing patient involvement in all aspects of evidence planning and healthcare decision making will further strengthen medicines development.

We highlight below the six guiding principles for excellent clinical evidence generation.

PRINCIPLE 1: PATIENTS ARE AT THE CENTER OF CLINICAL EVIDENCE GENERATION AND GUIDE EVERY STEP

Clinical evidence is generated for patients' needs and public health. Through their engagement, patients provide critical insight into their medical needs and what really matters to them at every level of healthcare decisions. Clinical evidence generation should revolve around these needs. Patients have been increasingly involved in healthcare decisions, including those related to the evaluation of the benefit–risk of medicines by regulators, where patients bring their personal experience, knowledge, and expertise both on the conditions and the available treatment options, and also on the impact of regulatory decisions on their lives.[3]

Efforts are ongoing to guide the generation, collection, and use of patient experience data to support decisions on the development and benefit–risk evaluation of medicines. To further build on these efforts, multi-stakeholder collaboration in this field is encouraged.

PRINCIPLE 2: EXISTING DATA AND KNOWLEDGE ARE LEVERAGED TO INFORM THE IDENTIFICATION OF GAPS, GENERATION OF CLINICAL EVIDENCE, AND HEALTHCARE DECISIONS

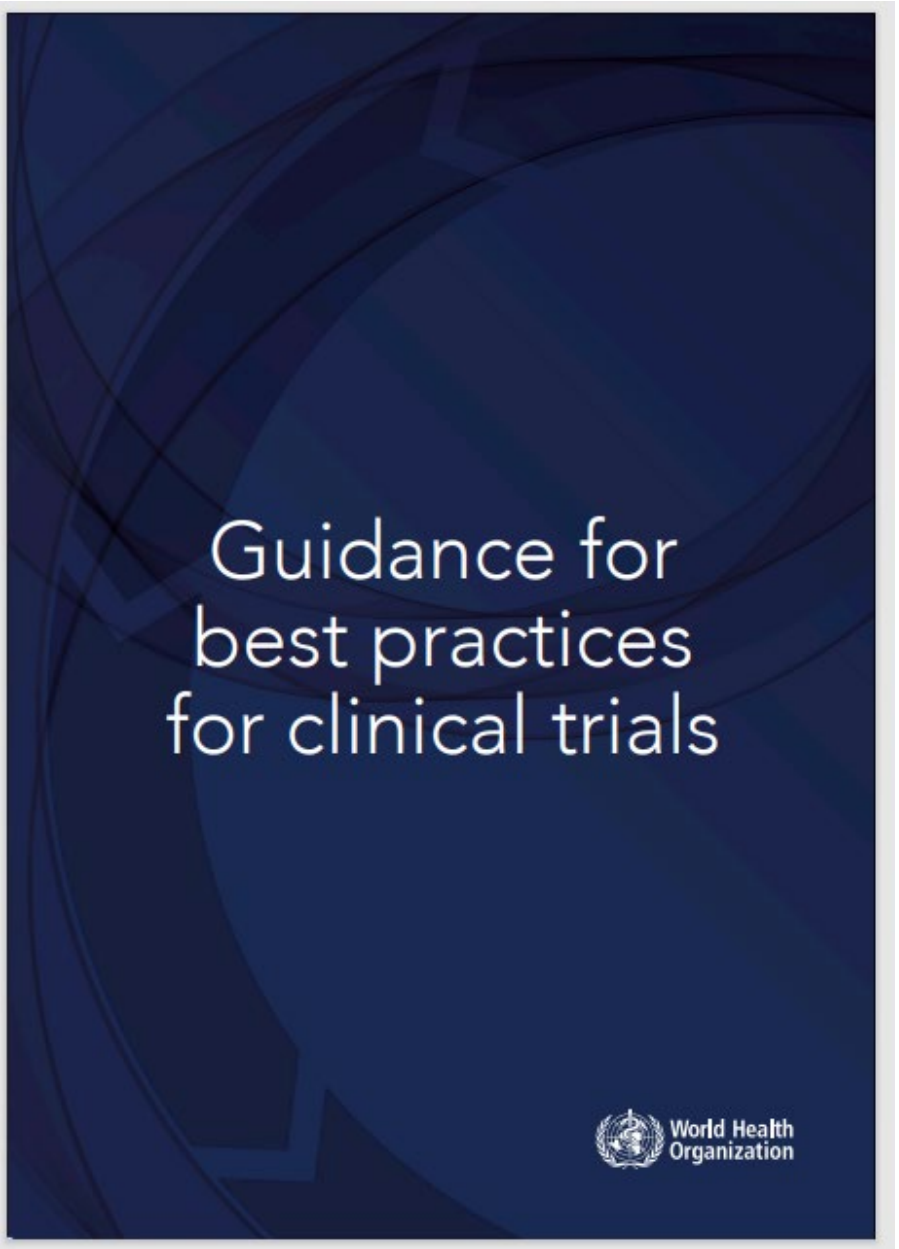
Clinical evidence generation is planned and guided by purpose, data, knowledge, and expertise. When formulating research questions and designing clinical evidence programs, existing data, information, and knowledge should be leveraged. Currently, this is not always the case, and clinical studies may be planned ignorant of previous study results or learnings from other medicinal products. To enable this informed approach to clinical research, access to data, information and knowledge, including study protocols and results, reports on suspected adverse reactions and the outcome of regulatory assessments should be made publicly available and scrutinized when designing studies. Multi-stakeholder dialogue at the planning stage will also facilitate access to existing knowledge. In this way, past successes and failures inform identification of gaps and further clinical evidence generation and may avoid unnecessary duplication.

Excellent clinical evidence: the heart of every well-informed decision

Randomised clinical trials remain at the core.

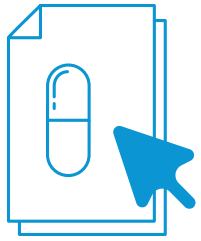
Aligned with WHO activities to strengthen clinical trials

- In May 2022, World Health Assembly adopted a resolution to strengthen clinical trial ecosystems
- In response, WHO in collaboration with stakeholders developed the Guidance for best practices for clinical trials
- Good clinical trials:
  - are designed to produce scientifically sound answers to relevant questions
  - respect the rights and well-being of participants
  - are collaborative and transparent
  - are feasible for context
  - manage quality effectively and efficiently



Clinical Trials Regulation (CTR)

- The 3-year CTR transition period ended on 30 January 2025
- All new and ongoing trials in the EU now fall under the CTR
- The CTR fosters innovation and research in the EU, facilitating the conduct of larger clinical trials in multiple EU / EEA countries
- EU clinical trials in CTIS since the start of CTR implementation (January 2022 – February 2025):



10,867

Submitted clinical trials

8,882

Authorised clinical trials

The power of data: finding a clinical trial for me

[Search for clinical trials – CTIS website]

- **Trial Map** developed to empower patients and healthcare professionals
- Integrated with CTIS public portal
- Easy access to information on clinical trials by geographical region and disease area
- Provide your feedback or suggestions

Strengthening the EU clinical trial ecosystem: What's next

Simplifying and modernising CTIS

Enhancing harmonisation of clinical trials authorisation (CTR Collaborate)

Supporting collaboration with ethics committees (MedEthicsEU)

ACT EU Multi-stakeholder platform at the core of EU clinical trial activities

Supporting efforts to streamline trials of medicines & medical devices (COMBINE programme)

Implementing the ACT EU workplan

Thank you