EMA
EUROPEAN MEDICINES AGENCY

The future of clinical evidence

Trials@Home closing event

31 October 2025

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Clinical trials

- Generate evidence to develop medicines, optimise their use and address unmet medical needs
- Location matters:
  - More opportunities for EU patients to access innovative treatments and medicines
  - Faster access to EU market for medicines when clinical trials are conducted in the region
- Positive feedback loop: more research – high-quality healthcare jobs - better healthcare delivery

Excellence of clinical evidence: the heart of well-informed decisions

Randomised clinical trials are the core of clinical evidence.

Clinical evidence 2030: vision

Patient voice guides every step of the way

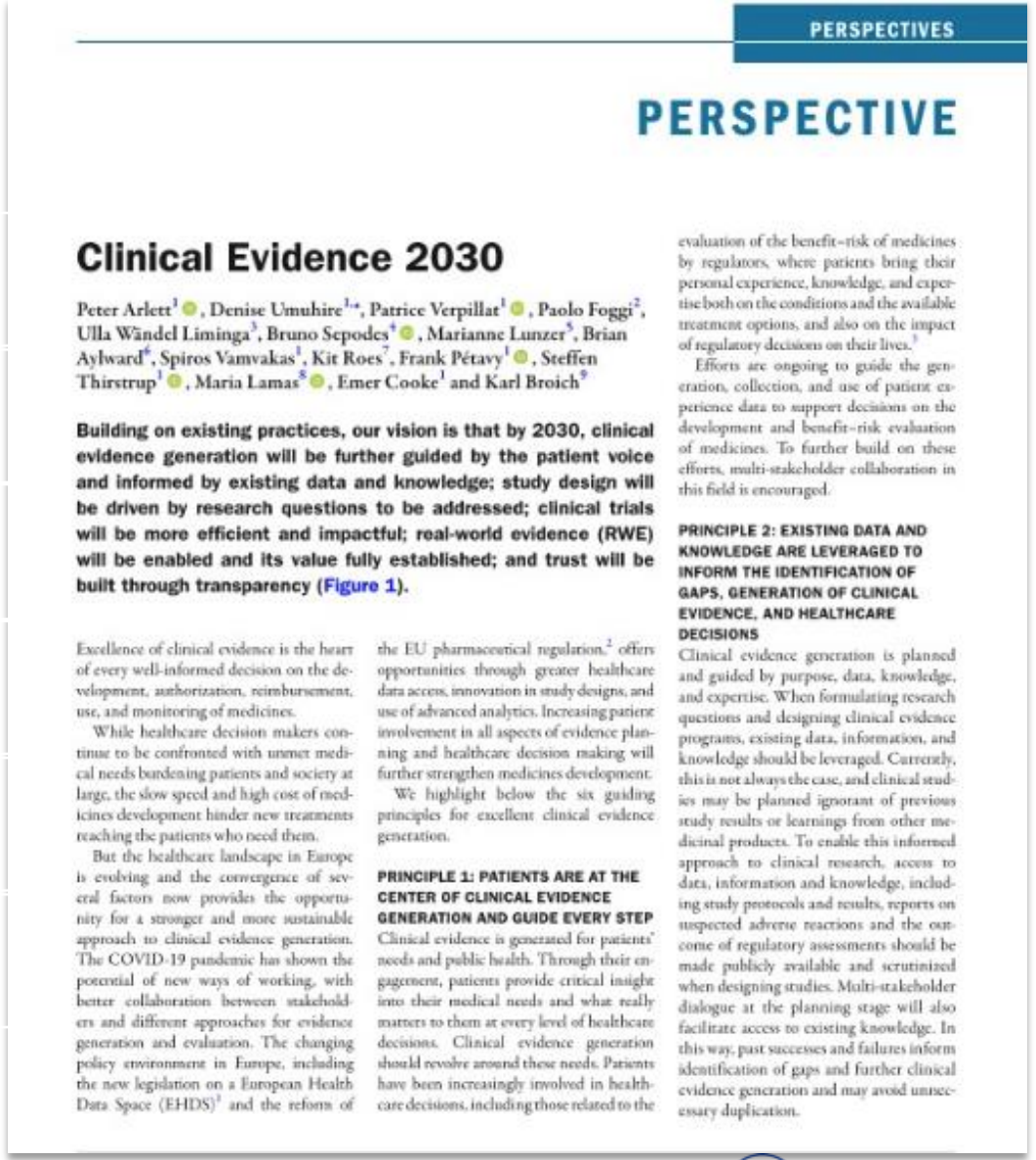
Evidence generation is planned and guided by purpose, data, knowledge and expertise

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** underpins societal trust



PERSPECTIVES

PERSPECTIVE

Clinical Evidence 2030

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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

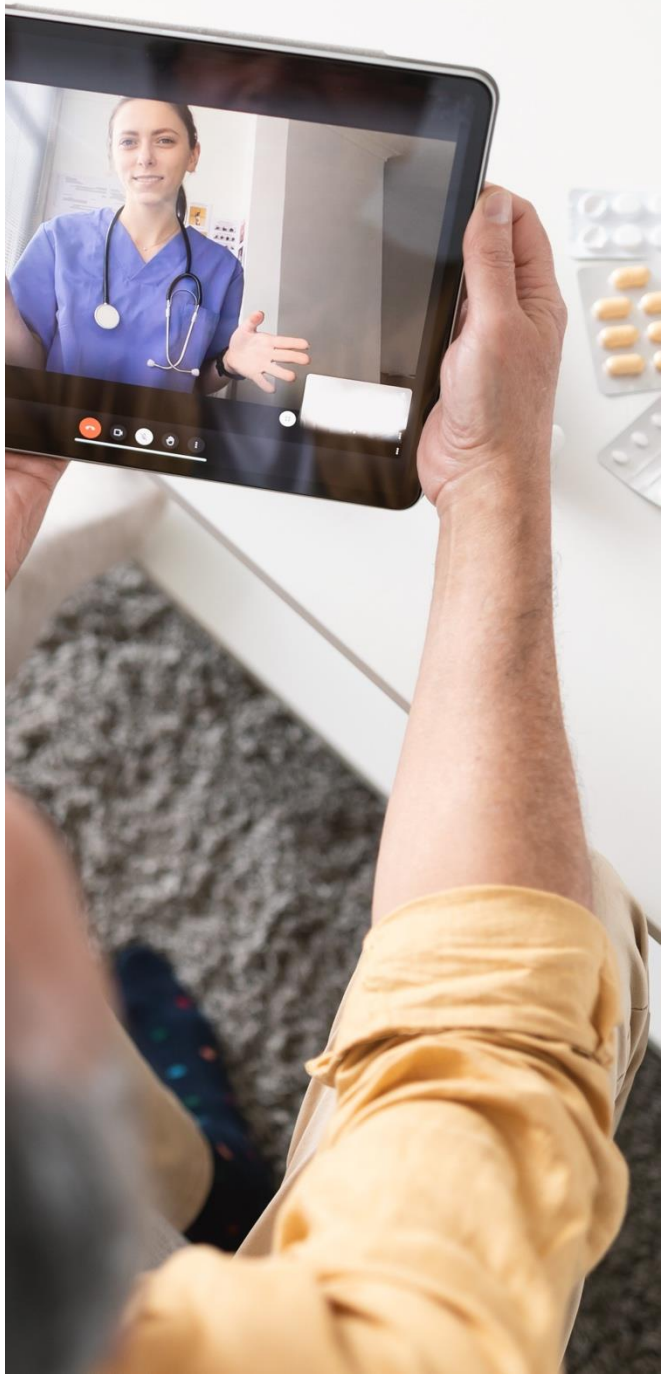
Clinical evidence generation is planned and guided by purpose, data, knowledge, and expertise. When formulating research questions and designing clinical evidence programs, existing data, information, and knowledge should be leveraged. Currently, this is not always the case, and clinical studies may 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.

Benefits of decentralised elements

A regulatory perspective

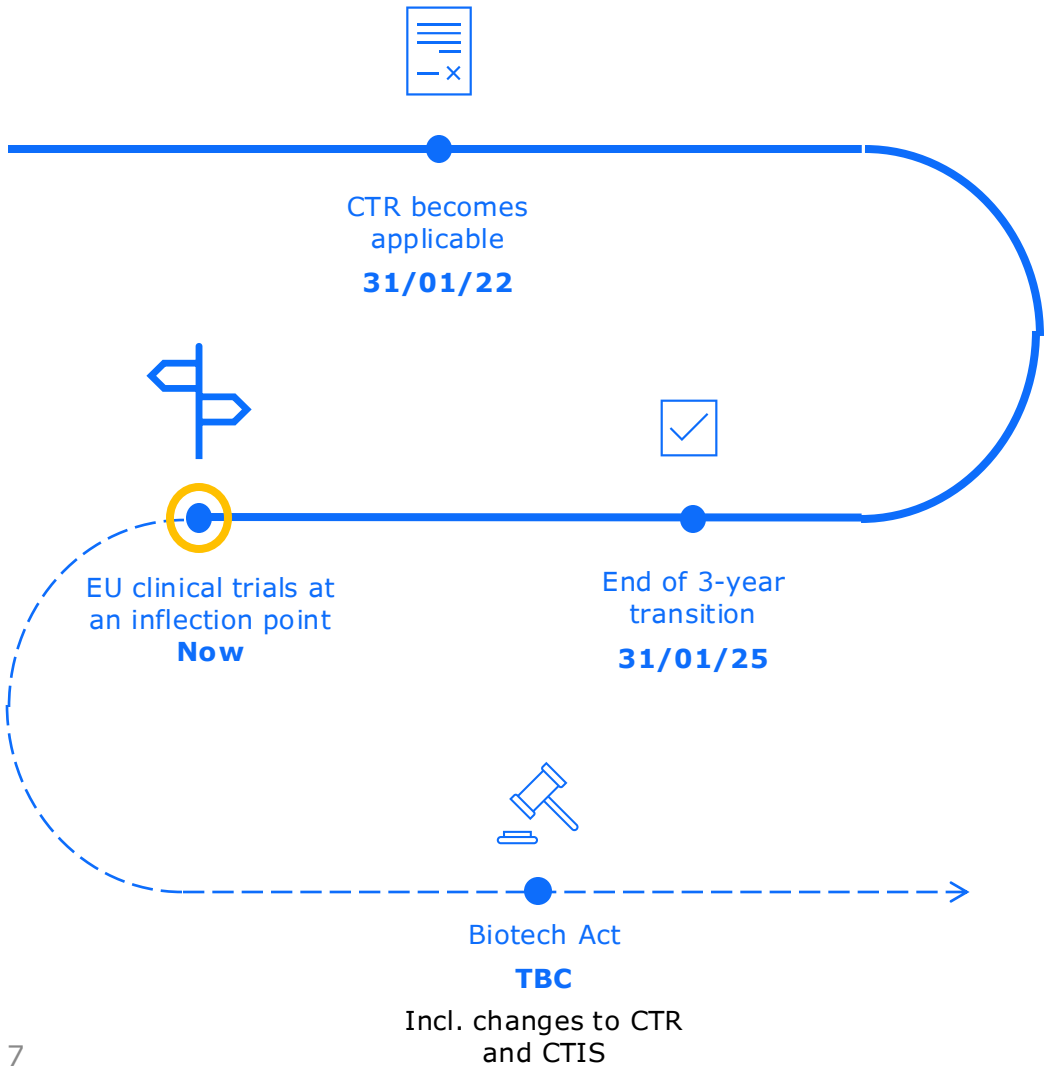
Decentralised elements in clinical trials can:

- Enhance recruitment and representativeness by making trials accessible for a wider range of patients, some with no other opportunity to receive treatment, from a wider range of geographical locations
- Increase convenience which enhances patient retention and engagement, leading to less drop out rates
- Reduce the burden on patients and the healthcare system, potentially reducing costs
- Enable more innovation in their design and conduct



Strengthening clinical trials in Europe

A turning point for clinical trials in the EU



Before the Clinical Trials Regulation (CTR):

- Fragmentation, separate trial applications in each EU/EEA country

Since CTR implementation in 2022:

- CTR and CTIS enable a single application for clinical trial authorisation in all EU Member States
- Trials transitioning from Clinical Trials Directive to CTR

Now:

- The transition is over but there is still work to do, to fully reap the benefits of the CTR

Next:

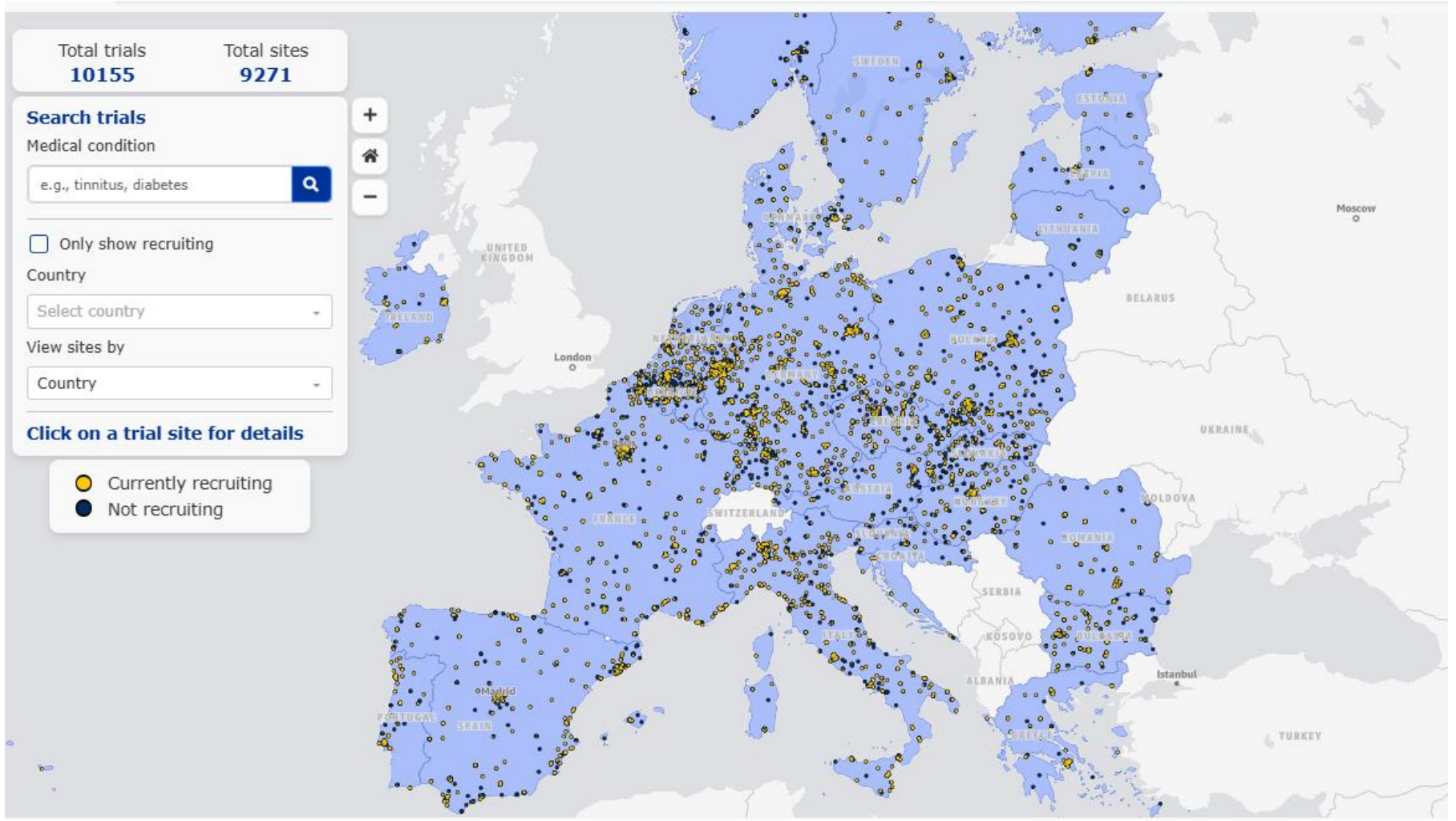
- Working together to optimise processes within current legal framework
- Preparing for legislative change

Accelerating Clinical Trials in the EU (ACT EU)

- A joint initiative by the European Commission, Heads of Medicines Agencies and EMA, established in 2022
- Building on the momentum of the implementation of the Clinical Trials Regulation (CTR), to transform.
- Our vision is to have **better, faster and smarter clinical trials in the EU**, creating a favourable environment for clinical research

……..let's see a few examples of what ACT EU is delivering

Finding clinical trials on the Trial Map



- **Trial Map** developed to empower patients and healthcare professionals
- Integrated with CTIS public portal
- Easy access to information on **more than 10,000 clinical trials** by geographical region and disease area
- STOP PRESS: now available in all official EU languages

[Search for clinical trials – CTIS website]

Pilots on scientific and regulatory advice

Pilot I: Scientific Advice Working Party (SAWP)-Clinical Trials Coordination Group (CTCG)

11 applications received:
- ✓ 8 concluded
- ⌛ 3 ongoing

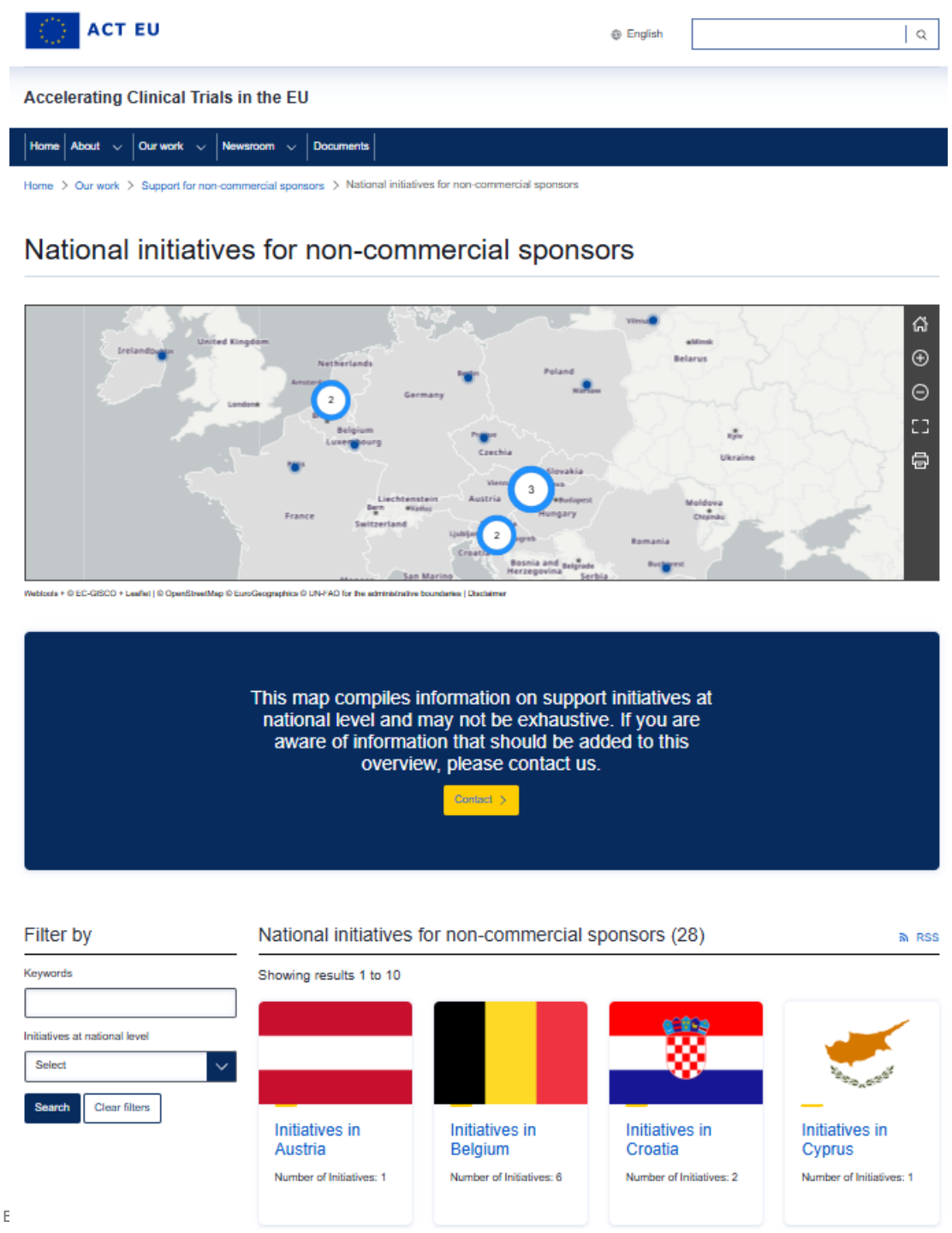
Pilot II: Pre-CTA (clinical trial application) advice from CTCG

17 applications received:
- ✓ 14 concluded
- ⌛ 1 ongoing
- ✗ 2 rejected

- Launched by ACT EU in June 2024
- 28 applications received so far
- Very positive feedback from applicants
- Currently exploring if the pilots should be a permanent offering

Support for non-commercial sponsors

- Tailored technical assistance on CTIS functionalities and regulatory requirements provided under regulatory helpdesk
- Up-to-date map of national support initiatives on [ACT EU website]
- National webinars on fostering clinical research by non-commercial sponsors delivered in IE, AT, BE, with more in 2026



Monitoring the EU clinical trials environment

[New targets for clinical trials in Europe | European Medicines Agency (EMA)]

Increased attractiveness of the EU

Target: 500 more multi-national trials authorised by 2030

Faster access to treatment

Target: 66% of trials start recruitment in under 200 days from application submission

ACT EU's multi-stakeholder platform (MSP): What do stakeholders want?

Risk based approach

Initiatives to support recruitment

Model contracts

Faster authorisation of trials

Streamlined ethical input

Only critical questions during assessment

Increased flexibility

Improving the use of decentralised elements in clinical trials

ACT EU's multi-stakeholder platform (MSP)

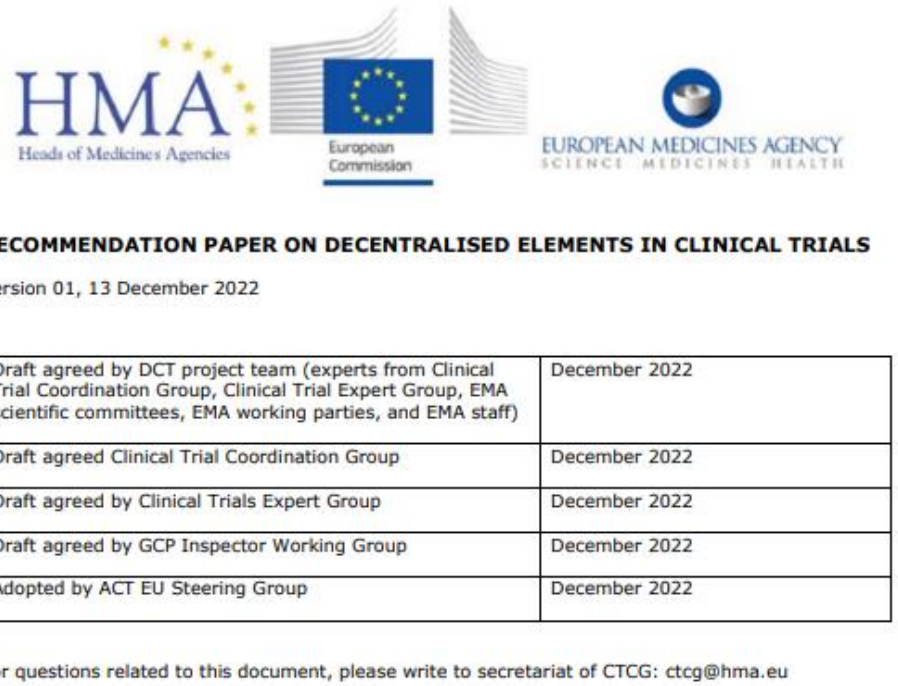
MSP can act as a vehicle to further discuss how to improve the use of decentralised elements in trials, through:

- Multi-stakeholder **workshops** on key clinical trial topics
- **Consultations and surveys** to gather stakeholder feedback
- Regular meetings of the **MSP Advisory Group** of stakeholder representatives

Updated DCT recommendation paper

Includes a disclaimer on cross border trials, and importantly, an updated annex of national provisions – published 29 Nov.

- EudraLex - Volume 10 - Public Health - European Commission



HMA Heads of Medicines Agencies

European Commission

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

RECOMMENDATION PAPER ON DECENTRALISED ELEMENTS IN CLINICAL TRIALS

Version 01, 13 December 2022

Draft agreed by DCT project team (experts from Clinical Trial Coordination Group, Clinical Trial Expert Group, EMA scientific committees, EMA working parties, and EMA staff)	December 2022
Draft agreed Clinical Trial Coordination Group	December 2022
Draft agreed by Clinical Trials Expert Group	December 2022
Draft agreed by GCP Inspector Working Group	December 2022
Adopted by ACT EU Steering Group	December 2022

For questions related to this document, please write to secretariat of CTCG: ctcg@hma.eu

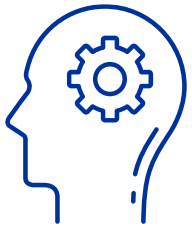
European Commission | EMA HMA Heads of Medicines Agencies

Legal, policy and technical opportunities

Biotech Act

Boosting innovation and competitiveness

Improving and simplifying the regulatory landscape, incl. through changes to the Clinical Trial Regulation



AI

Optimising processes and increasing productivity

CTIS modernisation

Enhancing user experience and improving efficiency

Delivering a more responsive system that can adapt to the evolving environment

Clinical trials 2030

Patients central
engagement enabled, evidence co-created

Innovation enabled
novel methods, decentralised elements, RWD enriched, AI-enabled processes.

Agile regulation
A single entry point for clinical trials advice, submission, fast authorisation

Data-driven decision making
Access to high quality data on clinical trials enables stakeholders to take decisions

better, faster and smarter clinical trials in the EU:
…….creating a favourable environment for clinical research and delivering for the patients of Europe

EMA

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Thank you

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