ACT EU as a Catalyst for Paediatric Research Innovation in Europe

How a Multi-Stakeholder Approach Can Accelerate Clinical Trials for Children

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Disclaimer

This presentation has been prepared in the context of the TEDDY Network's participation in the ACT EU Multi-Stakeholder Platform Advisory Group (MSP AG).

It does not constitute an official communication from ACT EU, and the views expressed do not necessarily reflect the positions of ACT EU, the European Medicines Agency, the European Commission, the Heads of Medicines Agencies or the MSP AG members.

ACT EU - Accelerating Clinical Trials in the EU

Better, faster, smarter clinical trials

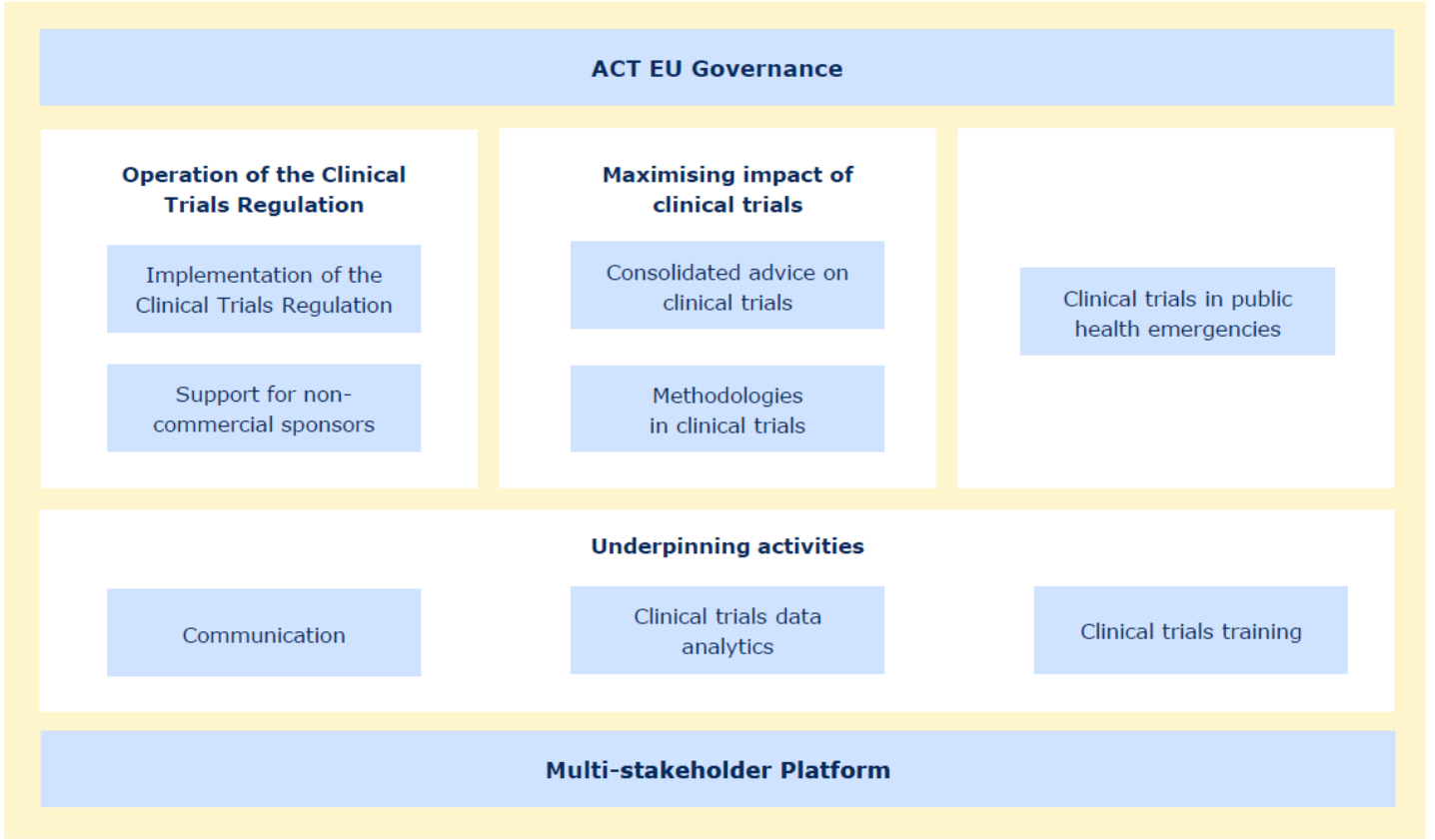
Launched in January 2022 by the European Commission, the European Medicines Agency (EMA) and Heads of Medicines Agencies (HMA)

ACT EU is a multi-annual programme aiming to **create a favourable environment for research and development in life sciences**, through **harmonisation, innovation and collaboration with stakeholders**.

Driving Innovation: Supporting smarter trials through regulatory, technological, and process updates.

Enhancing Collaboration: Fostering coordination among stakeholders, regulators, and ethics committees for cross-border trials.

Improving Healthcare: Transforming trial design and execution to deliver safer, higher-quality treatments to European patients.



ACT EU Objectives

Obj. 1: Accelerate research timelines, optimize ethical oversight, and reduce administrative burdens for academic and SME sponsors. **Increase awareness of available resources, including access to funding and research infrastructures.**

Obj. 2: Strengthen clinical trials by **including under-represented populations** (**children**, pregnant/lactating women) to address **unmet medical needs and rare diseases**.

Obj. 3: Heighten trial impact through excellent, coordinated regulatory and scientific advice throughout the medicine lifecycle.

Obj. 4: Engage stakeholders to deliver **patient-oriented development, integrating patients' voices across the lifecycle**.

Obj. 5: Ensure a clear and unified European position on clinical trials at the international level.

Obj. 6: Support training, regulatory science, and **collaboration between academia and European research infrastructures**.

Obj. 7: Enable **innovation through AI, digital tools, decentralized trial designs, and Real-World Data (RWD).**

PAEDIATRIC RESEARCH AS A STRATEGIC DRIVER

- Address existing structural challenges and methodological needs.
- Children's research as both a beneficiary and a driver of innovation.
- Position paediatric research as a priority use case for ACT EU.

Why Paediatric Research Matters for ACT EU

Paediatric clinical research faces the exact same systemic and structural bottlenecks that ACT EU is mandated to address

FRAGMENTATION ACROSS MEMBER STATES
Substantial variation in trial assessment timelines, ethical standards, and local practices across the EU.

REGULATORY COMPLEXITY
Operational complexity in the use of CTR/CTIS and remaining differences in national implementation and local requirements.

METHODOLOGICAL GAPS
Scarce specialized expertise in complex study designs and multi-stakeholder guideline structures.

RECRUITMENT BARRIERS
Highly limited pool of prospective participants due to small patient populations and rare diseases.

MULTINATIONAL EXECUTION
Severe hurdles initiating and standardizing collaborative trials across borders effectively.

PATIENT & FAMILY ROLE
Critical necessity for early co-design and active partnership with young patients and families to assure feasibility.

Paediatrics is a Priority Implementation Domain, Not a Niche Area

Because children's research operates at the intersection of these systemic challenges, it serves as the ultimate testbed for clinical innovation under ACT EU.

Multi-Stakeholder Approach as a Core Requirement

Clinical research, especially paediatric one, cannot be advanced by a single actor; it requires a fully coordinated ecosystem.

PATIENTS/ CONSUMERS

Engaging **patients, families, and YPAGs** as active partners to co-design feasible, pediatric-centric trials and integrate young voices into development.

HEALTHCARE PROFESSIONALS

Empowering **investigators and clinical networks** to standardise pediatric methodologies and drive high-quality, investigator-led academic studies.

NON-COMMERCIAL RESEARCH ORGANISATIONS & NETWORKS

Leveraging the expertise of **European non-commercial clinical and translational research organisations and networks** to support translational activities, enhance clinical data readiness, and address early-stage operational challenges.

RESEARCH FUNDERS

Aligning **funding priorities** with paediatric unmet needs and **enabling sustainable multinational investigator-initiated research**.

PHARMACEUTICAL INDUSTRY

Partnering with the **commercial sector, industries, and CROs** to unlock targeted funding, de-risk pediatric drug development, and operationalize pipelines.

Multi-stakeholder Platform

Multi-Stakeholder Platform in ACT EU

The Multi-stakeholder platform (MSP) functions as **a vehicle for clinical trials stakeholders and regulators to come together**, voice their views and collaborate to improve the clinical trials environment for European patients and citizens.

The MSP provides the opportunity for stakeholders to exchange views and enable dialogue with regulators through:

1. the creation of a **MSP Advisory Group**;
2. multi-stakeholder events;
3. consultations, surveys, and other tools to gather stakeholders' feedback.

PURPOSE

To **enable clinical trial transformation, bring together key stakeholders**, in a neutral forum, for regular, efficient, balanced discussions – putting patients at the centre.

- Enabling innovative approaches through multi-stakeholder collaboration.
- Enhancing trust and understanding between stakeholders and enabling equal, co-creation of change.
- Enable and support pilots of improvement.
- Dissemination of outcomes.

MSP Advisory Group

The MSP Advisory Group (MSP AG) is composed of representatives from key stakeholders who provide:

- strategic advice regarding the ACT EU workplan, identifying stakeholder priorities;
- operational advice for ACT EU initiatives, identifying experts and engagement methodology.

The **TEDDY** Network has been selected as one out of four representatives of the stakeholder group of **non-commercial European clinical data and translational research organizations and networks** within the ACT-EU MSP AG.

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

EPTRI
European Paediatric Translational Research Infrastructure

THE CHILDREN'S MEMORIAL HEALTH INSTITUTE

Support for non-commercial sponsors

Support for non-commercial sponsors

A large proportion of paediatric clinical trials are initiated and led by academic investigators, research networks and infrastructures, and investigator-led consortia, rather than large pharmaceutical companies.

Within ACT EU, this is explicitly recognised under the objective of **strengthening support for non-commercial sponsors**.

CRITICAL BOTTLENECKS

- Complexity of CTIS implementation
- Sponsor legal & operational responsibilities
- Heavy pharmacovigilance requirements
- Difficulties coordinating multinational trials
- Limited dedicated regulatory & operational capacity

ENABLING MECHANISMS

- Regulatory helpdesks
- Stakeholder workshops & targeted support
- Structured assistance for non-commercial sponsors
- Improved coordination across regulatory & clinical systems
- Funding

ACT EU DELIVERED

- Interactive map updates (NCS initiatives)
- ACT EU website network updates
- CTIS/CTR regulatory helpdesk maintenance
- Regular webinars to increase ACT EU & CTR/CTIS awareness
- 6-month CTR/CTIS helpdesk report

ACT EU can become the **operational bridge** between regulatory requirements and academic paediatric research, significantly reducing the administrative burden and improving trial feasibility across Europe.

Support for non-commercial sponsors

Support for non-commercial sponsors

TEDDY Network proposal: Pilots on Co-Sponsorship Models - High Potentiality, Limited Application

Collaborative Framework for Shared Resources, Expertise, and Risks in Clinical Research

Advantages

- **Resource Sharing**
- **Access to Complementary Expertise**
- **Increased Operational Capacity**
- **Enhanced Transparency and Scientific Quality**
- **Risk Sharing**

- Cost reduction and increased sustainability
- Greater study effectiveness and reliability

Limitations

- **Administrative and Contractual Complexity**
- **Conflicts of Interest or Objectives**
- **Regulatory Delays**
- **Increased Managerial Burden**

Although co-sponsorship models offer numerous advantages their use remains extremely limited. A pilot programme would certainly increase their use among Sponsors.

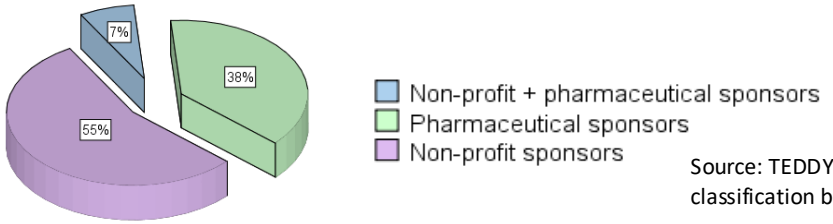
Only **141 out of 9200 (1.5%) Clinical Trials** registered in the **Clinical Trials Information System (CTIS)** were conducted under a **co-sponsorship model**

PAEDIATRIC: 6 out of 1121 (0.5%)

TOTAL: 141 out of 9200 (1.5%)

Out of the **141 co-sponsorship** studies analysed:

- 55% are sponsored **by hospitals, clinics, or other non-profit sponsors**,
- 38% by **pharmaceutical companies**,
- 7% jointly by **pharmaceutical companies and non-profit sponsors**.



Source: TEDDY analysis of CTIS data, accessed in 2025; classification based on predefined search terms

ePTRI European Paediatric Translational Research Infrastructure

The Children's Memorial Health Institute

Co-sponsorship foster cooperation among academic centers, community sites, industry, and patient groups *- enhancing integration across the entire clinical ecosystem, from adult to pediatric care.*

Methodologies in clinical trials

Innovative Approaches in Clinical Trials

Because traditional large randomized trials are often unfeasible, paediatrics serves as a primary driver of novel methodological approaches.

Obj. 7: Enable **innovation through AI, digital tools, decentralized trial designs, and Real-World Data (RWD).**

Priority

ACT EU PRIORITY AREAS

- **Innovative Methodologies:** Integrating advanced clinical designs in official guidance development.
- **Regulatory Alignment:** Coordinating joint platforms to build bridges between trial sponsors and regulators.
- **Knowledge Sharing:** Organizing joint workshops on platform trials and complex designs.

Scientific Need

ADVANCED METHODS

- **Adaptive & Bayesian:** Maximizing statistical power and flexibility in small, rare patient cohorts.
- **Master Protocols:** Running platform trials and basket designs to study multiple treatments in parallel.
- **RWD & Synthetics:** Implementing external control arms to reduce the burden of active control recruitment.

Action

TEDDY Network CONTRIBUTIONS

- **Pilot "Sandboxes":** Testing novel frameworks in early-phase design scenarios.
- ***In-silico* Strategies:** Promoting advanced modelling and simulation to optimize trial protocols.

Paediatrics: The Testing Ground for European Methodological Innovation

The severe constraints of children's research naturally accelerate and validate the adoption of alternative, highly-efficient designs under the ACT EU workplan.

Methodologies in clinical trials

Innovative Approaches in Clinical Trials

Advantages

- **Efficiency** increase
- **Sample size** decrease
- Easier patient **access**
- Higher **retention**
- Ensure **feasibility** and **validity** of data
- Offer **flexibility** in trial design
- **Optimization** of resource use

- Improvement of Ethical Standards
- Patient-Centric Approaches
- Safety Monitoring

Limitations

- **Conservatism** and **Resistance** to change
- **Regulatory Concerns**
- **High Cost** of Specialized Expertise
- **Limited Familiarity**
- **Lack of Training**

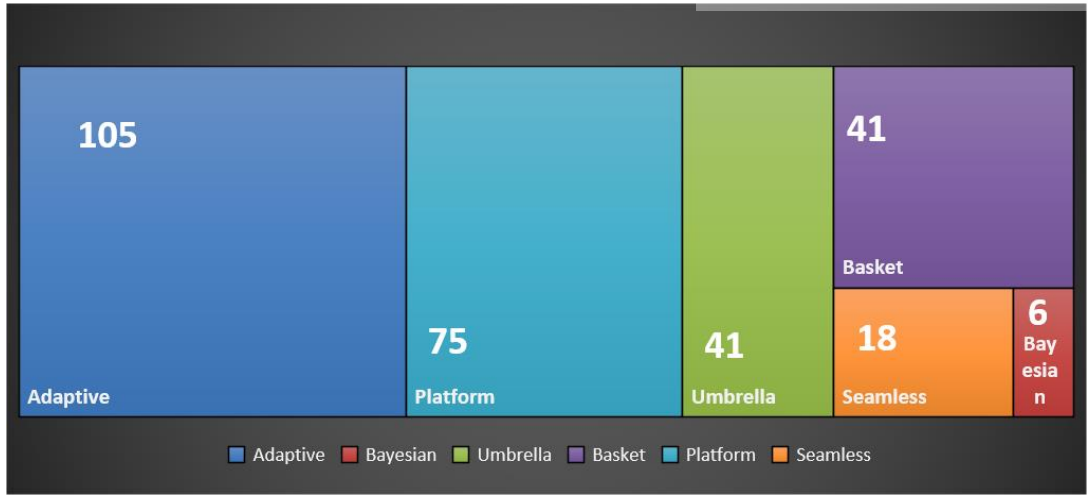
High Potentiality, Limited Uptake

Master Protocols (Umbrella, Platform, Basket)

Adaptive Designs - Seamless and Decentralized Trials

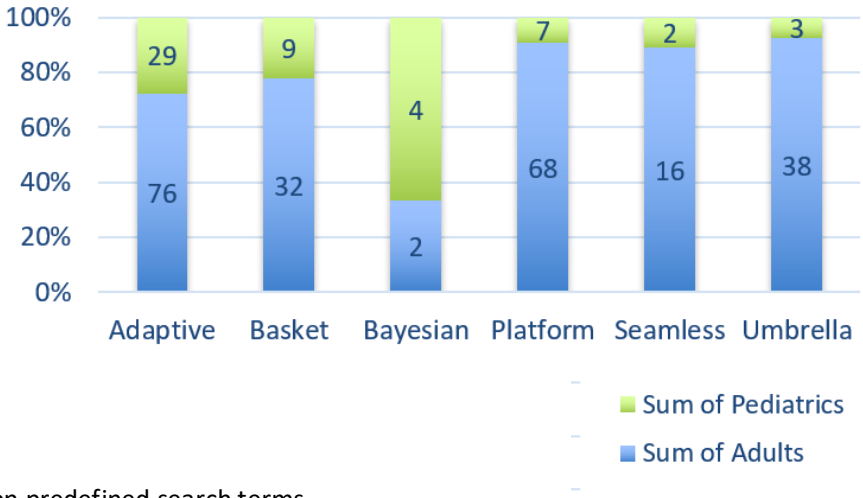
Only **286 out of 9200 (3%) clinical trials** registered in the **Clinical Trials Information System** (CTIS) were classified under terms related to innovative designs.

IN PAEDIATRIC POPULATION:
54 out of 1121 (4.8%)



Improving Identification of Innovative Trial Designs: SPIRIT & CONSORT guidelines

- **Current Limitation:** Lack of filtering options prevents identification of clinical trials with innovative designs.
- **Proposed Solution:** Adopt international standards, **SPIRIT** (for protocols) and **CONSORT** (for trial reports), to classify studies by methodological features, enabling better filtering and selection.
- **Paediatric Focus:** SPIRIT-C 2026 and CONSORT-C 2026 provide paediatric-specific recommendations to improve the completeness, transparency and usefulness of paediatric trial protocols and reports.



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Source: TEDDY analysis of CTIS data, accessed in 2025; classification based on predefined search terms.

Methodologies in clinical trials

European Initiatives Driving Clinical Translation

Strengthens transnational health research by supporting joint programming, investigator-initiated clinical studies, and the uptake of health technologies

ERA4Health Partnership

ERAMET

Promotes the adoption of modelling and simulation methods by providing validated tools, data standards, and regulatory-ready frameworks.

invents

Develops innovative clinical trial designs, *in silico trials*, and real-world data approaches to improve the evaluation of medicines for rare diseases and support regulatory decision-making.

Facilitate the transfer of innovative approaches from research settings into clinical development and healthcare practice.

Realise D

Develops operational and methodological solutions to optimise clinical trials and accelerate drug development for rare and ultra-rare diseases.

ERDERA

European Rare Diseases Research Alliance

Provides methodological support to promote the integration of innovative approaches into research and clinical pathways

ePTRI

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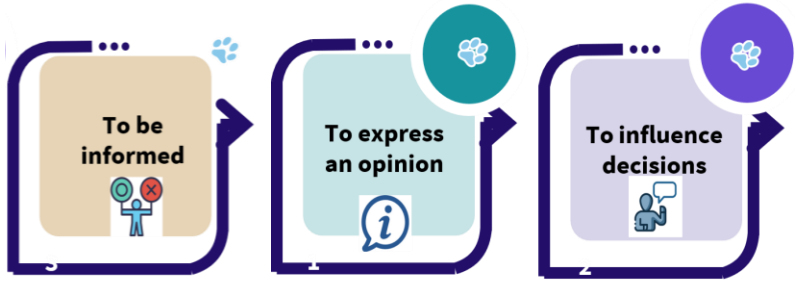
Meaningful Patient Involvement

Obj. 4: Engage stakeholders to deliver **patient-oriented development, integrating patients' voices across the lifecycle.**

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

Maximizing the impact of patient engagement activities by deploying established paediatric infrastructures and methodologies.

The involvement of children in health and biomedical research is a progressive process, aligned with the development of their capacities, and involves different levels of participation:



Involvement of young patients

ERDERA European Rare Diseases Research Alliance
To train patients and young researchers in understanding rare diseases and in the development of innovative and effective therapeutic solutions.

OrphaDev4Kids
To identify unmet therapeutic needs and develop pediatric medical devices for rare diseases.

invents
To develop innovative methods with regulatory relevance in clinical research on pediatric and rare populations (e.g., juvenile idiopathic arthritis and pediatric multiple sclerosis).

Funded by the European Union

- ✓ **KIDS-YPAG Groups & Child Participation:** Promoting pediatric patient involvement through **KIDS-YPAG groups** to empower children and young patients as active participants in healthcare decision-making processes.
- ✓ **Validated Frameworks for Families:** Deploying field-tested methodologies to involve parents and caregivers, systematically minimizing trial burden while optimizing clinical recruitment and retention.
- ✓ **Ready-to-Use Materials & Tools:** Providing a mature repository of age-appropriate, digitally interactive informed consent/assent templates, ready to be scaled across the European Union.

Strategic Alignment with European Priorities

ACT EU does not operate in a vacuum.
It directly aligns clinical trial execution with Europe's key strategic priorities:

- **The Proposed EU Biotech Act:** Modernising the European life sciences ecosystem and driving the scale-up of biotech innovation.
- **Clinical Research Investment Plan:** Attracting capital, boosting competitiveness, and optimizing European trial frameworks.

A competitive European ecosystem is impossible without an agile framework for its youngest and most vulnerable populations.

Paediatrics and Rare Diseases are the ultimate sandboxes for global innovation.

SYSTEMIC ENABLERS

Faster Translation
Accelerating the transition from lab to bedside, drastically narrowing the historical gap between adult and paediatric therapy availability.

Attractive Conditions
De-risking the ecosystem to incentivize biotech and pharma developers to invest early in dedicated pipelines and orphan and paediatric drug developments.

Regulatory Predictability
Harmonizing scientific advice across Member States to eliminate bureaucratic friction, backed by senior representation in ACT EU and Enpr-EMA.

Innovative Methodologies
Fostering advanced trial designs (master protocols, *in silico* simulation) for small populations, as applied in INVENTS and ERAMET Projects.

Strengthened Infrastructures
Deepening the operational integration of pan-European networks to seamlessly support complex trials.

Role of Research Infrastructures & Networks

Research infrastructures and networks are essential implementation partners for **ACT EU** bridging European high-level policy visions with concrete clinical execution.

BUILDING EUROPEAN CAPACITY

Strengthening a sustainable European paediatric research infrastructure that connects **expertise, resources and people** across borders.

PROMOTING EXCELLENCE IN PAEDIATRIC TRIALS

Raising the **standard of paediatric research** across Europe through harmonised practices, training and shared expertise.

BRIDGING RESEARCH AND REGULATION

Aligning paediatric research with **regulatory science** to facilitate innovation, access and evidence generation for medicines.

PUTTING CHILDREN AT THE CENTRE

Ensuring **children's voices** inform research priorities and trial design across a collaborative European research framework.

Mature research infrastructures and networks act as the critical structural engine to turn ACT EU's policy visions into sustainable, high-quality, and ethical clinical trials across Europe.

TEDDY NETWORK
European Network of Excellence for Paediatric Clinical Research

ePTRI
European Paediatric Transnational Research Infrastructure

EnprEMA
European Network of Paediatric Research at the European Medicines Agency

conect 4children
COLLABORATIVE NETWORK FOR EUROPEAN CLINICAL TRIALS FOR CHILDREN

Transforming European Clinical Research

ACT EU provides the master operational roadmap,
but the ultimate success of the EU strategy depends on mature, collaborative execution at the patient level.

THE OPERATIONAL FRAMEWORK

ACT EU acts as the critical engine to unify European clinical trials.
Together with research infrastructures, it bridges high-level policy guidelines with standardized, cross-border clinical trial execution.

THE STRATEGIC CATALYST

Paediatric research is the sandbox of clinical trial innovation.
From advanced adaptive designs to established patient-centric frameworks (YPAGs), children's studies drive ecosystem-wide standards.

Policy-to-Practice

Harmonization

Patient-Centric

Methodological Pioneer

Paediatric research should not merely adapt to ACT EU standards; it must be treated as a key strategic driver of innovation, patient-centricity, and methodological advancement across all European clinical research ecosystem.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EPTRI General Assembly and Scientific Meeting
Warsaw, 28-29 July, 2026
THANK YOU FOR YOUR ATTENTION!
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