EPTRI

European Paediatric Transnational Research Infrastructure

EPTRI Paediatric Research Needs Survey

Key Findings and Strategic Implications

Presentation to the EMA Paediatric Committee (PDCO)

Paediatric Research Needs Survey

The survey captures stakeholder-reported research needs, evidence gaps and barriers across the paediatric research and innovation pathway to inform future priorities.

Section A

Respondent profile

Who is responding?

- Organisation **type and size**
- Involvement across **research stages** and **innovation areas**.

Section B

Research priorities, gaps & barriers

What is missing? What blocks progress?

- Priority evidence, knowledge and product-development **gaps**
- Operational, regulatory and system **barriers** to progress

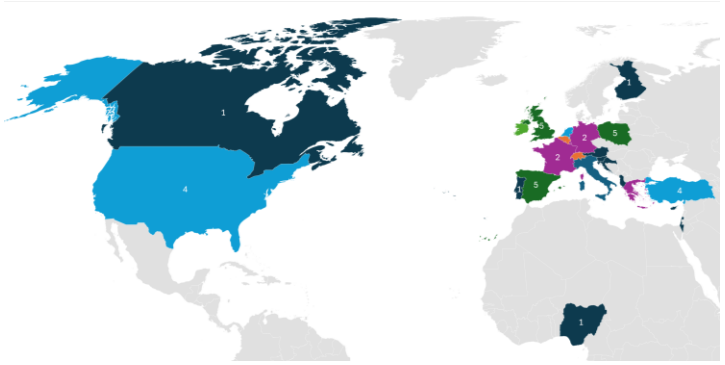
Section C

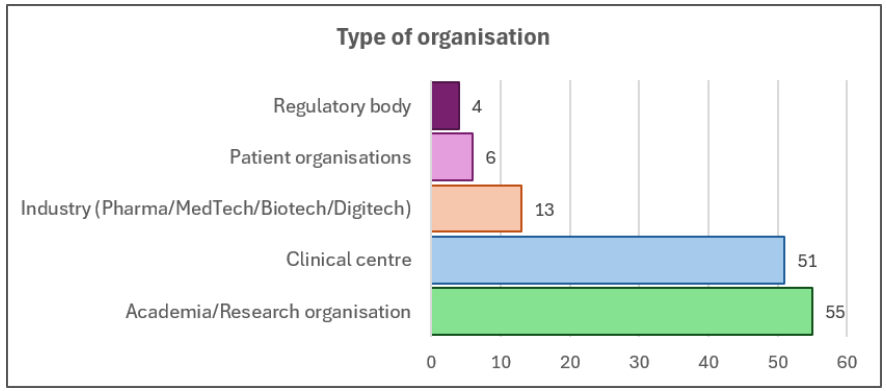
Support needs & implementation priorities

What support could enable progress?

- Cross-cutting **support needs** and preferred **collaboration models**
- Dedicated question modules **for clinical centres, industry, academia, patient organisations and regulatory bodies.**

Additional sections explored **service value and access (D), final insights (E) and optional follow-up (F)**





The current sample is predominantly academic and clinical (**106 of 129 respondents**), with smaller representation from industry, patient organisations and regulatory bodies.

***Note**: The survey is ongoing; findings reflect responses collected to date.*

Key findings across the survey

Current findings show where activity is concentrated, which gaps and barriers persist, and what support is most needed.

SECTION A

Research profile & activity areas

CURRENT ACTIVITY

98 Clinical studies

53 RWD / registries

EARLY & TRANSLATIONAL ACTIVITY

35/32 Early phases drug development

32 Biomarkers

24 Diagnostics

Activity is strongly clinical, with RWD/registries as a second pillar. Early development, biomarkers and diagnostics also show a meaningful translational activity base.

SECTION B

Evidence gaps & barriers

LEADING EVIDENCE GAPS

- RWD & registries · 59
- Validated biomarkers & endpoints · 46
- Paediatric clinical evidence · 45

MAIN BARRIERS TO PROGRESS

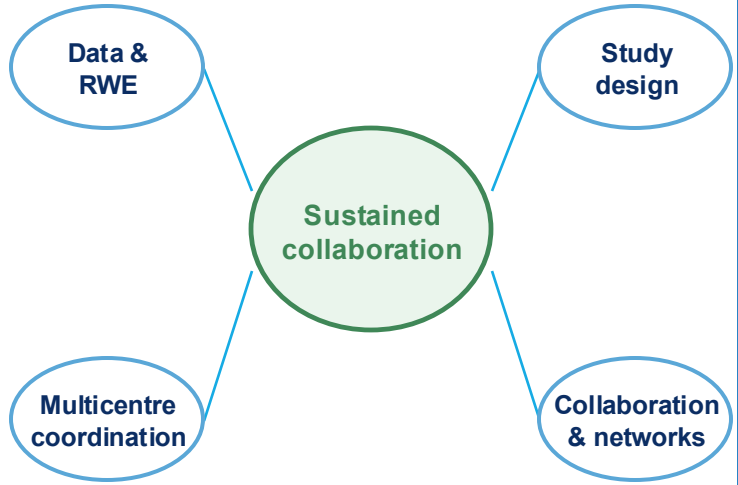
Funding · Recruitment & Feasibility · Regulatory & Ethics

Evidence gaps are compounded by implementation barriers; their relative weight varies across stakeholder groups.

SECTION C

Support needs & collaboration

SHARED SUPPORT PRIORITIES



Shared priorities converge on data/RWE, study design, multicentre support and networking, best delivered through integrated, long-term collaboration.

➢ Across the survey, three connected priorities emerge: **robust paediatric evidence, feasible delivery and sustained collaboration.**

From Paediatric Research Needs to Coordinated Action: Key Conclusions

01

Evidence priorities

Across the research continuum, the clearest gaps concern **paediatric RWD and registries, validated endpoints and outcome measures, and paediatric-specific clinical evidence**. Together, they point to the need for evidence that is sufficiently robust and usable for decision-making.

02

Barriers to progress

Progress is constrained primarily by **limited funding, recruitment and study feasibility, and regulatory and ethics complexity**. Additional barriers related to data, access and patient involvement vary across stakeholder groups.

03

What enables progress

Respondents prioritise practical support for **data and RWE, study design, multicentre coordination and networking**. They favour **long-term, integrated collaboration over one-off advice**, with delivery adapted to stakeholder-specific needs.

Take-home message

The survey points to a continuity gap rather than a single missing capability. Progress requires long-term, coordinated support connecting robust evidence generation, accessible data and feasible multicentre delivery, while addressing stakeholder-specific needs from the outset.

LINK: Paediatric Research Needs Survey