2026

EXECUTIVE SUMMARY

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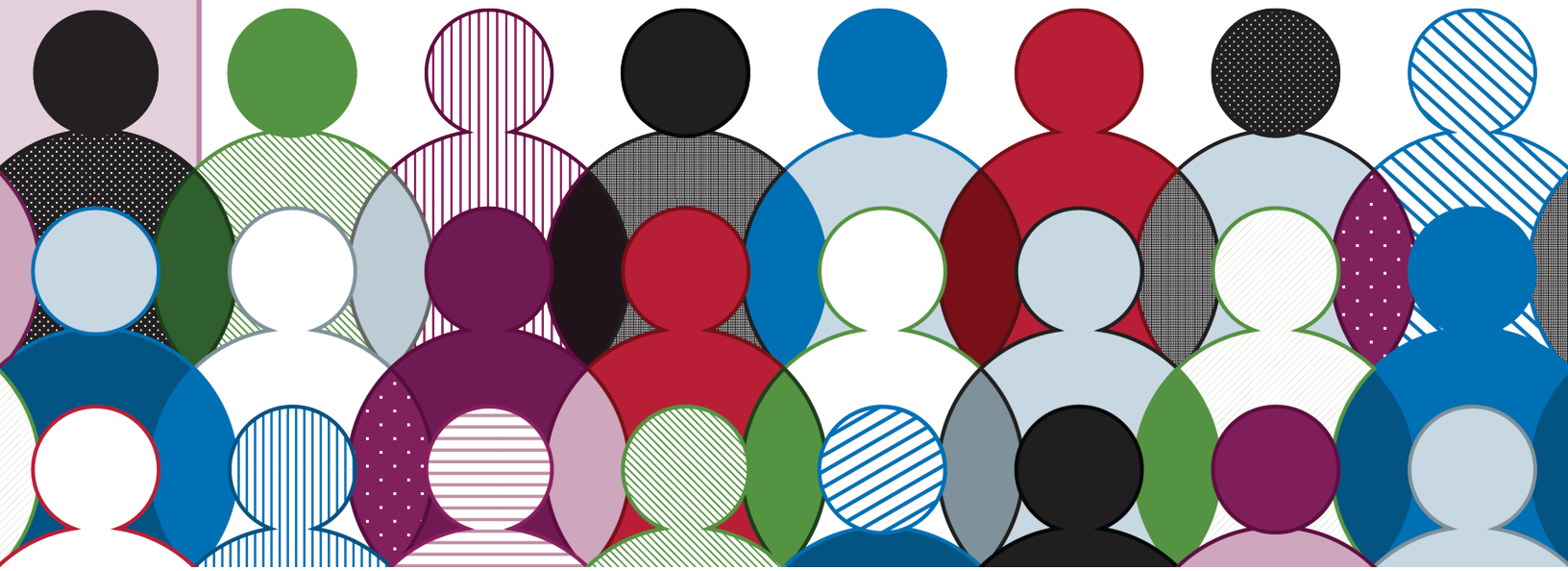
Childhood Cancer Academic-Industry Collaborative Platform Trials (C3PT)



MRCT CENTER
THE MULTI-REGIONAL CLINICAL TRIALS CENTER OF BRIGHAM AND WOMEN'S HOSPITAL AND HARVARD

ITCC
INNOVATIVE THERAPIES FOR CHILDREN AND ADOLESCENTS WITH CANCER

Blood Cancer United

CHILDREN'S ONCOLOGY GROUP

EXECUTIVE SUMMARY

The *C3PT Position Narrative* » presents a multi-stakeholder blueprint for the development and delivery of **Childhood Cancer Academic Industry Collaborative Platform Trials (C3PT). The C3PT Blueprint provides a practical roadmap for establishing effective academic-industry partnerships that can accelerate drug development and improve outcomes for children and young people with cancer.**

The initiative was developed to address the unique challenges of paediatric oncology, where rare and biologically diverse cancers make conventional drug development slow, costly, and fragmented. Platform trials offer a more efficient model by enabling the adaptive evaluation of multiple therapies within a single protocol and infrastructure. Their advantages include shared operational resources, the ability to add or close treatment arms independently, and improved efficiency in evaluating novel medicines for children and young people with cancer.

Despite these advantages, current paediatric oncology platform trials have encountered recurring barriers including limited access to industry assets, regulatory misalignment across jurisdictions, contracting delays, funding challenges, and insufficient academic operational capacity. Addressing these obstacles requires coordinated collaboration between academia, industry, regulators, patient advocates, and international trial consortia

The C3PT Blueprint was developed through extensive engagement with clinicians, patient advocates, regulators (EMA and FDA), industry representatives, and academic organisations including ITCC, COG, MRCT Center, and Blood Cancer United. It provides a consensus framework for designing, funding, governing, and delivering successful academic-industry platform trials, supported by practical implementation tools and guidance.

Principal Recommendations

1. Define the Trial Purpose Early

A clearly articulated trial purpose is fundamental to platform trial success. Academic sponsors should determine at the outset whether the platform is designed to generate exploratory evidence, support regulatory decision-making, or fulfill regulatory obligations. Alignment around trial intent informs stakeholder engagement, governance structures, operational design, and regulatory strategy. A dedicated C3PT decision tool supports this process. (See the ***Platform Trial Decision Tree*** » Tool.)

2. Engage Key Stakeholders as Early as Possible

Meaningful engagement should begin during platform conception and continue throughout development and delivery.

- **Regulators:** Early, transparent, dialogue with EMA and FDA is essential. Seeking early (joint) scientific advice can align regulatory expectations, support paediatric plans across jurisdictions, reduce unnecessary amendments, and improve efficiency. C3PT Tools advise on ***Best Practice in Regulatory Interactions*** » and provide ***PIPs and iPSPs: Guidance for Collaborative Contributions to Writing*** ».
- **Patient Advocates:** Patients and families should be integrated as active partners rather than consulted intermittently. The Platform's governance structures should define roles, responsibilities, communication pathways, and training opportunities to support sustained involvement throughout the trial lifecycle.
- **Industry Partners:** Academic groups and pharmaceutical companies should transition from informal interactions to structured partnerships with defined expectations, transparent deliverables, fair compensation for expertise, and inclusion of patient perspectives in decision-making.

3. Improve Access to Investigational Assets

One of the greatest barriers to platform trials is securing access to suitable medicines. Academic leaders should engage industry partners strategically and

at the appropriate stage of drug development, when development decisions remain influenceable. Successful collaborations depend on understanding industry decision-making processes and demonstrating the platform's ability to generate robust, regulatory-relevant evidence efficiently.

4. Optimise Operational Infrastructure

Academic sponsors require sufficient expertise and capacity across scientific leadership, regulatory sponsorship, governance, data management, and site operations. While operating models may vary, successful platforms require critical mass, sustainability, and readiness across all operational functions. A C3PT ***Operational Considerations*** » checklist has been developed to support self-assessment and planning.

5. Sustainable Funding Models

If industry partners recognise the value of platform trials, they will be willing to invest when costs and expected returns are transparent. Although platform trials often require significant upfront investment, they can deliver long-term efficiencies through shared infrastructure, streamlined recruitment, and reduced duplication of effort. Transparent costing frameworks are therefore essential.

6. Independent Asset Prioritisation

To avoid a "first-come, first-served" approach, asset-selection decisions should be guided by an independent expert review process. The Blueprint recommends an ***Evidence-Based Consolidated Independent Expert Opinion (EBCO)*** » framework, with practical examples from the GLO-BNHL trial, to provide transparent, objective, and scientifically robust recommendations regarding asset inclusion. (See the ***Glo-BNHL Industry Information Pack*** » and ***Glo-BNHL Asset Submission Form*** ».)

7. Contractual Principles

Recurring challenges around data ownership, intellectual property, publication rights, and governance can delay trial initiation. The Blueprint provides tools recommending transparent contractual principles that preserve academic

independence while providing industry partners with appropriate access to data for regulatory purposes. Clear governance and publication procedures are essential to maintaining trust and partnership effectiveness. (See the ***Intellectual Property and Data Sharing Contract Principles for Platform Trials*** » Tool and the ***Likely Pharma–Academic Pain Points and Practical Solutions*** » Tool.)

8. Articulating a Strong Value Proposition

Many pharmaceutical companies continue to view academic platform trials as equivalent to traditional investigator-initiated studies and therefore underestimate their strategic value. Platform leaders should clearly communicate the benefits of participation, including access to rare patient populations, shared infrastructure, operational efficiencies, high-quality data generation, predictable timelines, and support for regulatory objectives. The C3PT toolkit includes resources to assist with stakeholder communication and partnership development. (See the ***Value Proposition of C3PT to Industry Partners*** » slide deck.)

Future Directions

The C3PT Blueprint calls for continued evolution of policy, regulation, and operational processes to better support academic-industry collaborative platform trials. It advocates for streamlined amendment pathways, ongoing refinement of implementation tools, and stronger international alignment. Above all, future development must remain patient-centred. Meaningful communication, equitable access, long-term follow-up, and sustained involvement of patient advocates will be essential to ensuring that platform trials translate into improved survival, better quality of life, and more rapid access to innovative therapies for children and young people with cancer.

Access the full C3PT work here: https://MRCTcenter.org/C3PT