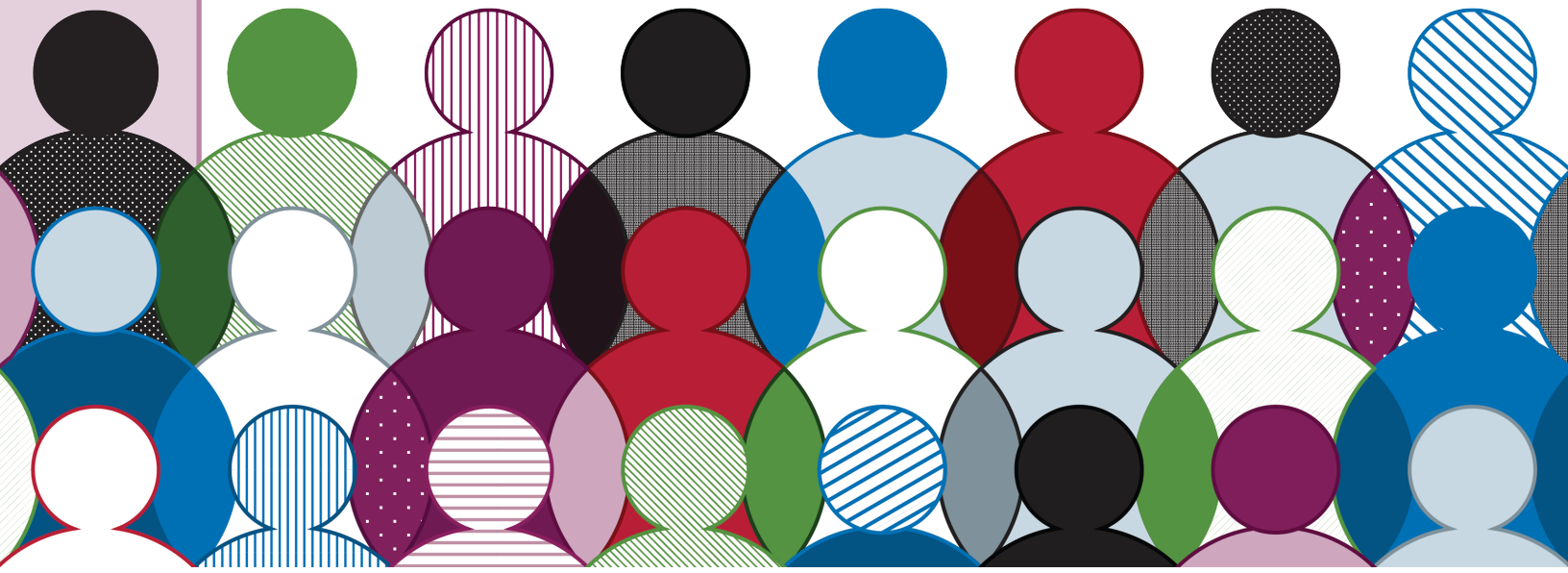
2026

POSITION NARRATIVE v 1.0

Childhood Cancer Academic-Industry Collaborative Platform Trials (C3PT)



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ITCC
INNOVATIVE THERAPIES FOR CHILDREN AND ADOLESCENTS WITH CANCER

Blood Cancer United

CHILDREN'S ONCOLOGY GROUP

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SUGGESTED CITATION

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EXECUTIVE SUMMARY

This 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.

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STATEMENT OF PURPOSE

This Blueprint and its associated tools are intended to provide practical, expert opinion-based guidance for stakeholders planning to develop and conduct a platform trial for childhood cancers, undertaken through collaboration between academia and the pharmaceutical industry.

The intended audience includes academic sponsors, paediatric oncology investigators, patient advocates, and pharmaceutical and biotechnology companies engaged in oncology drug development, particularly those operating under regulatory requirements such as the US FDA's RACE for Children Act, the EU Paediatric Regulation and new EU Pharmaceutical Legisation, which mandate the incorporation of paediatric studies into development programmes.

The Blueprint outlines the value of academic–industry partnerships in the design and delivery of paediatric oncology platform trials and the importance of early engagement between all stakeholders. It supports users in determining whether a platform trial approach is appropriate and feasible and describes key operational considerations to enable efficient and effective trial conduct. The accompanying tools and supporting materials are designed to guide users from the initial decision to adopt a platform trial design through to successful trial delivery.

This document reflects the collective perspectives of contributors at the time of publication and will be updated as evidence, policy, and stakeholder perspectives evolve.

SETTING THE SCENE

The Origins of the Blueprint

In October 2024, the MRCT Center hosted a two day workshop, *Advancing Pediatric Platform Trials: Streamlining Development, Maximizing Impact*, funded in part by Johnson and Johnson, Sanofi and AstraZeneca. This solution-focused workshop sought to explore the strengths of paediatric ***platform trials** by advancing the principles, ethical foundations, and operational considerations for conducting these trials in registration studies of paediatric investigational products. The workshop selected three disease areas to address various challenges and opportunities in paediatric drug development: major depressive disorder, multi-drug-resistant tuberculosis, and paediatric hematological malignancies.

***PLATFORM TRIAL:**
a clinical trial using a single, ongoing master protocol to evaluate multiple treatments simultaneously, allowing interventions to be added or removed over time based on accumulating evidence

Building on the work of the October 2024 workshop, Innovative Therapies for Children with Cancer (ITCC), the Leukemia and Lymphoma Foundation (LLS, now Blood Cancer United, BCU), the Children's Oncology Group, and the MRCT Center collaboratively organized a follow up multistakeholder workshop focused on *Advancing Childhood Cancer Academic-Industry Collaborative Platform Trials*. This multi-stakeholder workshop involved approximately 40 paediatric oncology experts, with balanced representation from academic trialists, industry representatives, patient advocates, and regulatory authorities from the United States and the European Union. The narrative guidance and practical tools presented here are the direct outputs of that workshop.

Generously funded by B+ Foundation, Cancer Research UK, LifeArc; Solving Kids Cancer UK, and Leukemia & Lymphoma Society [Now Blood Cancer United], this workshop sought to develop an implementation framework for academic-industry collaborative platform studies that deliver data that could support a regulatory

requirement **(*PIPs, *iPSPs)** and potentially marketing authorisation applications, specifically focused on paediatric oncology medicines.

The *Advancing Childhood Cancer Academic-Industry Collaborative Platform Trials* workshop led to the development of this narrative and suite of solution-focused of tools, intended as a practical collection of materials that coalesce into a blueprint for those considering developing a platform trial for paediatric oncology.

Academic-Industry Collaborative Platform Trials

The critical need to expedite drug development for children and young people with cancer is recognised by all stakeholders involved. These rare cancer populations face significant unmet medical needs due to limited patient numbers and biological diversity, making it essential that clinical trials are grounded in strong scientific rationale to maximize meaningful outcomes and efficiently identify effective therapies.[1, 2]

There are many factors that feed into delaying paediatric drug development but the concept of 'platform trials' has been proposed as one solution to accelerate progress. Platform trials can provide a framework to evaluate more than one medicinal product in a single trial in parallel arms. This allows arms of the trial to open and complete independently and for new medicinal product(s) to be added for evaluation during the course of the trial.[3, 4] Importantly in an academic-industry collaborative platform trial, the medicinal products can be from different industry partners. **In the context of this initative, platform trial refers to a prospective disease or biologically defined target-focused trial that is adaptive and evaluates multiple, simultaneous or possibly differently timed interventions.**

The adaptive nature of platform trials provides the opportunity of streamlined and efficient evaluation of multiple medicinal products, but they are complex and challenging to deliver.[5] Whilst platform

***(PIPs):**
Paediatric Investigation Plan
An EMA-approved plan describing how a medicine will be studied and developed for use in children to ensure its quality, safety and efficacy.

***(iPSPs):**
Initial Pediatric Study Plan
An FDA-required plan outlining the proposed paediatric studies, timelines and regulatory strategy for a medicine's development in children

[1] (Pearson ADJ et al. 2022)
[2] (Vassal G et al. 2013)
[3] (Geoerger B et al. 2023)
[4] (Adaptive Platform Trials Coalition. 2019)
[5] (Blagden SP et al. 2020)

trials can and are being conducted by industry, as well as by academic and other non-commercial sponsors, this position narrative focuses on **academic-sponsored platform trials** because they can offer multiple efficiencies, including concurrent or sequential evaluation of products that are under development by different industry partners.[5, 6] Depending on their design and objectives, academic-sponsored platform trials can generate data that serve a variety of purposes: assessing the risk/benefit of studied assets for patients; informing go/no go decisions for future pivotal trials; fulfillment of regulatory incentives or requirements; and providing support for a benefit/risk assessment in a marketing application.

[5] (Blagden SP et al. 2020)
[6] (Park JJH et al. 2019)

The workshop attendees concluded that effective collaboration between academia and industry was fundamental, as was early engagement with regulatory bodies and meaningful involvement of patient advocates throughout. However, success is being substantially impeded by multiple hurdles and there is a dearth of practical guidance and easily accessible information to guide stakeholders considering a platform trial approach to drug evaluation.[7]

[7] (McLennan S et al. 2026)

The aim of this position narrative is to present a comprehensive 'Blueprint' with practical tools as a guide to those embarking on an academic-industry collaborative platform clinical trial.

Defining the Challenges from Key Stakeholder Perspectives

Understanding the different perspectives from the clinicians, regulators, pharmaceutical industry representatives and patient advocates (collectively referred to as stakeholders) enabled us to appreciate what would constitute an attractive **childhood cancer academic-industry collaborative platform trial (C3PT) value proposition** and enabled the development of this **C3PT Blueprint**. The Blueprint details the identified challenges and describes the

accompanying practical tools and resources that offer concrete options to solve or mitigate identified roadblocks to facilitate efficient delivery of future collaborative platform trials in paediatric oncology. The **collective views of each of the four stakeholder groups** are summarized below**:**

1. Patient Advocates:

The patient advocates' experience ranged from direct involvement in platform trial design and delivery through membership of trial oversight committees to more focused tasks such as reviewing patient facing information and developing measures for Patient Reported Outcomes (PROs). Their primary drivers were trials where the research question met patient unmet needs, and where the interest of the patient was central throughout and led to accelerated trial impact, ultimately providing children with cancer access to licensed and reimbursed effective safe treatments. Their opinion was trial designs should ensure the fewest children as possible are exposed to unpromising or ineffective medicinal products. The patient advocates identified the vital need for trust-building between stakeholders and transparent knowledge sharing, and advocated for inclusion as equal partners from project inception.

2. Academic Investigators:

All the clinicians present had experience as trial investigators and/or academic sponsors of existing platform trials in paediatric oncology, with a range of objectives and designs, and delivered with different models of partnership with industry. Their perceived challenges were focused on difficulties in accessing their desired medicinal products from industry, complex alignment of national resources and regulatory needs with international/global regulatory and sponsor requirements, and limited core operational and per-trial resources which contributed to slow progress in trial set up.

3. Regulators:

The FDA and EMA were represented by senior representatives with extensive regulatory experience in paediatric oncology. The regulatory representatives emphasised the utility of academic-sponsored platform trials because they provide a mechanism for evaluation of assets of multiple companies. They advised that platform trials need to be based on a strong scientific rationale and emphasized the need for timely engagement with regulatory authorities to ensure that the trial design meets regulatory expectations and the primary goals of the trial (e.g., to provide information for future development versus providing the primary support for a marketing application). They encouraged consideration of innovative designs (e.g., novel statistical methods) for efficient evaluation of medical products in the relevant population, noting that such designs may require more in-depth regulatory interactions. In addition, they identified the need for academic consortia and platform sponsors to define clear and transparent strategies to decide on assets selected for inclusion in the platform and collaborative engagement across all parties.

4. Industry:

Representatives from seven pharmaceutical companies, with a range of experiences of collaborating with academic consortia and sponsors on paediatric platform and non-platform trials, provided the insights from an industry perspective. Two key themes emerged a) time efficiency is critical, therefore partnership with an academic-sponsored platform trials is only viable if tangible time and resource savings are demonstrated; and b) industry's apprehension regarding interactions with regulatory bodies, where they preceived material differences in regulatory processes and decisions, leading to challenges in trans-Atlantic collaboration and inefficiencies in the drug development pathway.

Industry is fully cognisant of the clinicians' desire to access their assets for inclusion in academic sponsored platform trials and the associated frustration when requests are denied. They reflected that there is often a gap in clinicians' understanding of industry's decision making processes and the drivers behind these decisions. Industry representatives also recognised that academic and advocate involvement in ***paediatric investigation plan (PIP)** and ***initial paediatric study plan (iPSP)** discussions remains underutilised by industry and is an area of opportunity.

Whilst the stakeholders differed in their experience of academic-industry collaborative platform trials, they agreed on the following **principal priorities** for a childhood cancer academic-industry collaborative platform trial**:**

PATIENT-FIRST APPROACH emphasising ethical conduct and high-quality data to enable access to safe, effective therapies

ACCELERATED, COST-EFFICIENT TRIALS with global reach and shared infrastructure

EARLY, COORDINATED REGULATORY ENGAGEMENT AND ADVICE to streamline the process of reaching PIPs/iPSPs agreement and to ensure that platform trials are appropriately aligned to investigate new promising treatments in paediatric patients with cancer, with a goal toward generating evidence to support "go/no go" decision-making and support regulatory approval of products that provide clinical benefit to patients

TRUST AND TRANSPARENCY among academia, industry, regulators, and patient advocates, supported by clear roles (data ownership and sharing, intellectual property, trial purpose) and open communication

SUSTAINABLE INFRASTRUCTURE with defined responsibilities and funding models

STRATEGIC PRIORITIZATION of molecules and trial designs

***Paediatric Investigation Plan (PIP):**
An EMA-approved plan describing how a medicine will be studied and developed for use in children to ensure its quality, safety and efficacy.

***Initial Pediatric Study Plan (iPSP):**
An FDA-required plan outlining the proposed paediatric studies, timelines and regulatory strategy for a medicine's development in children

THE BLUEPRINT

Developing a Blueprint for Childhood Cancer Academic-Industry Collaborative Platform Trials

Having defined the **principal priorities** for a childhood cancer academic-industry collaborative platform trial and articulated the challenges to achieving these objectives from different stakeholder perspectives, collective approaches to addressing these challenges were developed and integrated into the following C3PT Blueprint. The aim is for the Blueprint to provide informative and pragmatic tools for all stakeholders involved in academic-industry collaborative platform trials aiming to support drug development in childhood cancer.

This C3PT Blueprint integrates the expertise and perspectives of the multiple involved stakeholders to define the essential components required for the successful delivery of a collaborative, academically sponsored platform trial to accelerate the development of industry-owned medicinal products for paediatric cancers. The Blueprint outlines best practices, core elements, and modification procedures. It is intentionally dynamic: aspirational yet actionable, aligned with the current regulatory landscape and adaptable to future developments. It reflects the shared commitment of the involved patient advocates, regulatory bodies, academia, and industry representatives to advance patient-centred research and innovation.

C3PT Blueprint

The graphic to the right is intended to function as an **interactive visual roadmap** or table of contents for this Consensus Document. The circles are clickable buttons that will jump you to the relevant section within this document. The outer-most ring of labels list the various tools and resources available throughout this document. The key below explains their functionality.

The grey lines within the circle are representative, not prescriptive, of potential connections between tools within the blueprint.

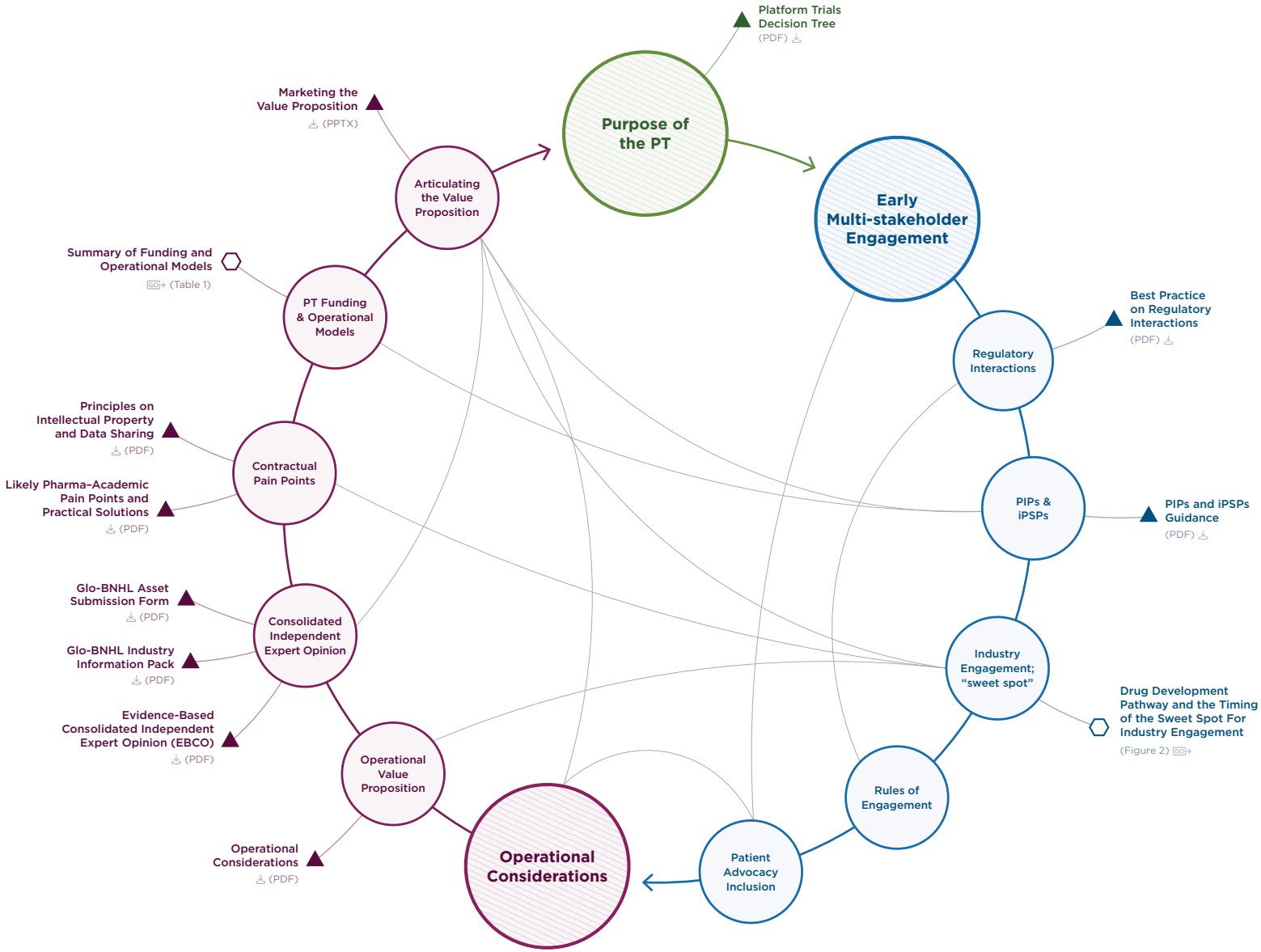
Key for the outer-most ring of labels (tools and resources):

▲ = a linked **external** resource

⬡ = a linked **in-doc** resource

= click to **download** resource

GO = click to **jump** to resource



PATIENT-FIRST APPROACH

ACCELERATED, COST-EFFICIENT TRIALS

Agreeing on the Purpose of the Platform Trial: The Decision Tree

While the overall aims of a childhood cancer academic-industry collaborative platform trial are to evaluate the safety and efficacy of new therapeutic approaches, there will be differences in the specific objectives of each platform. Select considerations include: Is the purpose hypothesis-generating or toward pivotal evidence generating? What is the delivery model? What is the potential value proposition for different stakeholders? These and other factors will inform the decision as to whether an academic-sponsored platform trial model is the right approach. To guide these considerations, a decision-making framework has been developed for evaluating whether a multi-arm platform trial for paediatric cancer patients is suitable. It explores the benefits of using a platform trial, such as efficiency in studying multiple therapies, and adaptability for rare populations, and outlines scenarios where platform trials may not be appropriate. The stepwise decision tree guides stakeholders through questions on clinical objectives, industry collaboration, regulatory considerations, infrastructure, and feasibility to determine the best trial design. If a platform trial design is considered the appropriate approach, further considerations for successful development and conduct of the academic-industry collaboration are detailed throughout this tool. Key aspects of early multi-stakeholder engagement, the critical elements necessary for an attractive operational value proposition, and a proposed blueprint framework for optimal trial delivery are highlighted.

TOOL: Platform Trial Decision Tree

EARLY, REGULATORY ENGAGEMENT

Early Multi-stakeholder Engagement

REGULATORY INTERACTIONS

The successful development and conduct of collaborative platforms can be facilitated by improved multi-stakeholder understanding and adoption of best practices for regulatory interactions. This includes collaborative working, transparent communication, and maximising the potential for alignment between major regulatory agencies. We focused on the FDA and EMA, who collaborated in this initiative, although recognise the applicability to many other regulators globally. Both FDA and EMA strongly support a multi stakeholder approach—including academia, patient advocates, industry sponsors, and international regulators—to accelerate development of effective cancer therapies for children.

A central theme is the importance of early, regular, and transparent engagement between academic sponsors and regulators, with inclusion of all relevant parties in these interactions whenever possible. This includes coordinated discussions on platform trial design, timely sharing of information across agencies, and ensuring all stakeholders are represented in key meetings. Industry partners are encouraged to incorporate information about their platform trial participation within paediatric plans (PIPs and iPSPs) and proactively seek regulatory advice when needed.

To support trans-Atlantic regulatory alignment, the FDA and EMA use mechanisms such as paediatric cluster calls, parallel scientific advice, and requesting common commentaries on PIPs/iPSPs to harmonize development strategies. These efforts help identify areas of agreement and acknowledge where alignment is not feasible. Transparency from industry partners regarding global submission plans enables both agencies to coordinate effectively and avoid delays.

The Best Practice on Regulatory Interactions tool outlines pathways for regulatory engagement with the EMA—primarily through Scientific Advice and PIPs—highlighting the need for clear scope, iterative interactions, and appropriate sequencing of procedure types. It also describes support available to academic and non commercial sponsors, including fee waivers and opportunities for informal presubmission discussions.

On the FDA side, *Best Practice on Regulatory Interactions tool* provides an overview of meeting types (A, B, C, D, F, INTERACT, and Parallel Scientific Advice), their purposes, timelines, and relevance to academic platform trial sponsors. A key message is the value of strategic communication planning with the FDA review division to ensure efficient, responsive dialogue throughout development.

Collectively, the practices described aim to foster a coordinated, efficient, and scientifically rigorous regulatory environment that supports innovative paediatric oncology trials. Through structured engagement, transparency, and alignment between global authorities, sponsors can better navigate regulatory processes and accelerate the delivery of novel therapies to paediatric patients.

TOOL: Best Practices for Regulatory Interactions GO

EARLY, REGULATORY ENGAGEMENT

STANDARDIZED DISEASE OR TARGET SPECIFIC SECTIONS FOR PIPS AND IPSPS FOR INCREASED EFFICIENCY

A disease-specific platform trial offers the opportunity to evaluate different medicinal products independently in a single trial framework and each medicinal product will have its individual paediatric development plan, detailed in a PIP or iPSP. Here we aim to provide practical guidance on elements within the PIP and/or iPSP where

harmonised text could be provided to the industry partners by the academic platform leads, bringing more efficiency to the development of these documents.

The sections within a PIP or an iPSP relating to the background knowledge of the condition or molecular target are factually consistent and would not vary significantly in PIPs or iPSPs for different medicinal products. The expert clinical leadership of an academic-industry collaborative platform trial could provide increased efficiencies in the development and regulatory review of these documents if harmonised text could be provided for inclusion in the relevant sections of the iPSP and PIPs. Where we had access to both the PIP and iPSP on the same medicinal product or condition, the information contained was almost identical supporting a recommendation for synchronous drafting of PIPs and iPSPs.

A review of the pathway of PIPs and iPSPs from industry partners for the ongoing childhood cancer platform trial Glo-B-NHL[8] revealed variations in when and how much of the drug development documents were shared with the academic platform trial leadership and the resultant outcomes. In all cases, industry partners shared elements of or the entire PIP, but only half of the iPSPs were shared.

[8] (Seaford E et al. 2023)

It is evident that early engagement between the industry partner and the academic trial leadership would provide the opportunity to add this common core background and design elements to PIPs and iPSPs.

TOOL: PIPs and iPSPs: Guidance for Collaborative Contributions to Writing

TRUST AND TRANSPARENCY

TIMING OF INDUSTRY ENGAGEMENT; HITTING THE 'SWEET SPOT'

Academic clinicians often find it difficult to navigate the paths to access the novel medicinal products that are a prerequisite for the conduct of academic-industry collaborative platform trials. Improved stakeholder understanding of where a medicinal product is in its development pathway and insight into industry decision-making processes are vital to facilitate successful engagement and collaboration. Clarity on the who, how and when of industry engagement during the course of the medicinal product's development plan will guide academic clinicians to the optimal engagement window, or the 'sweet spot' for industry-academic engagement

It is critical to determine who the decision maker is within a company and gain access to the individual who typically will have control of a budget necessary for such a collaboration. Some have assumed that the industry representative they encounter in initial discussions is the final decision-maker, however individual industry paediatric experts often do not have the authority, financially or otherwise, to make the final decisions for the company to engage in a platform trial. Rather, industry representatives typically shape strategy and provide recommendations while formal approval rests with the industry partner's governance boards, who will weigh paediatric development opportunities in the context of overall medicinal product development and broader considerations.

For most companies, paediatric platform trial proposals follow the same governance pathway as adult oncology studies (e.g., development team → global programme team → senior governance). Large paediatric initiatives are reviewed by governance bodies that prioritise drug development and resource allocation across the portfolio. Typically, the company's paediatric experts advise the medicinal product team and partner with the medicinal product team to bring proposals forward within the company.

Nevertheless, in some companies, the paediatric team has broader authority to independently seek senior governance approval. When engaging with a pharmaceutical company representative, an understanding of their company's paediatric decision-making process is critical to managing expectations and effective communication.

Organisational Models For Paediatric Decision-Making:

There are two primary organisational models that detail **how** paediatric decision-making is organized in pharmaceutical companies (FIGURE 1). Across both models, governance bodies remain the ultimate decision-makers on platform trial participation.

FIGURE 1:

Organisational Models For Paediatric Decision-Making

Embedded Advisory Model	Dedicated Paediatric Team Model
Paediatric experts are integrated into adult asset teams, providing strategic input and advocating within the governance chain (development team → global program team → senior governance). Proposals are advanced by the asset team.	A specialised paediatric team monitors the pipeline, develops proposals, and is accountable for developing and delivering an asset's paediatric strategy. While asset teams must endorse strategy and plans, the paediatric team presents proposals to governance.

Industry Considerations for Academic Platform Trials:

Industry sponsored clinical trials are an established standard for generating data suitable to support regulatory submissions, reflecting the industry sponsor's accountability for study conduct, data integrity, and regulatory interactions. This model is particularly well established for adult indications. For paediatric oncology, academic sponsored platform trials represent an alternative development approach that

may offer potential efficiencies but require careful evaluation within the context of the sponsor's governance and regulatory frameworks.

From an industry perspective, consideration of participation in an academic platform trial is informed by the extent to which the platform can demonstrate that it can deliver data of appropriate quality, reliability, and regulatory acceptability, while offering potential advantages in time or resource utilisation. **Key considerations** include evidence that the platform can support**:**

ACCELERATED, COST-EFFICIENT TRIALS

- An overall resource profile that is proportionate relative to a conventional single product sponsor led trial

SUSTAINABLE INFRASTRUCTURE

- Predictable and well coordinated trial start up processes, including regulatory alignment, contracting, protocol finalisation, and site activation

PATIENT-FIRST APPROACH

- Feasible recruitment and retention in rare paediatric populations

EARLY, REGULATORY ENGAGEMENT

- Robust data quality, integrity, and governance, including clear data access arrangements that support regulatory review and sponsor accountability

For newly established platform trials, the absence of a demonstrated operational track record is recognised. In such cases, indicative performance data from comparable, well established platforms may inform assessment, including timelines from initial scientific engagement to contract execution, progression through governance and regulatory processes, and achievement of key milestones such as first patient enrollment.

Optimal Timing of External Engagement—The 'Sweet Spot':

Engagement between academic groups and industry sponsors is most effective when aligned with internal paediatric development planning and established governance timelines. Given variability across sponsor development models, this may occur when supportive adult data are

emerging—often around signal seeking (phase II) development—or earlier when paediatric strategy is being considered during safety and dose finding (phase I) trials.

Early scientific dialogue with paediatric experts within industry can support evaluation of platform trial concepts and relevance to a sponsor's portfolio. The **'Sweet Spot'** for engagement is the optimal window, not a fixed point in time, within a medicinal product's development pathway. **Ideally, dialogue should be during the safety and dose finding (phase I) development in adults but will vary depending on company development models and program specific timing; engagement should be no later than at the start of signal seeking (Phase II) development.** Each company's decisions regarding participation will remain subject to evaluation of compliance with sponsor governance, data requirements, and regulatory obligations.

FIGURE 2 (next page):

Drug Development Pathway and the timing of the Sweet Spot for industry engagement

FIGURE 2.1. Typical sequential stages in the clinical development pathway of an oncology medicinal product for adult indications.

FIGURE 2.2. Key points along the adult oncology drug development pathway at which paediatric development should be considered, aligned with European Union (EU) and United States (US) regulatory requirements.

FIGURE 2.3. The "Sweet Spot" denotes the preferred period for initiating discussions with pharmaceutical sponsors regarding inclusion of a medicinal product in a paediatric platform trial. It is depicted as a window rather than a discrete timepoint, recognising that optimal timing may vary according to individual company development strategies and programmes.

Drug Development Pathway and the Timing of the Sweet Spot For Industry Engagement

FIG 2.1. Adult Drug Development Timeline

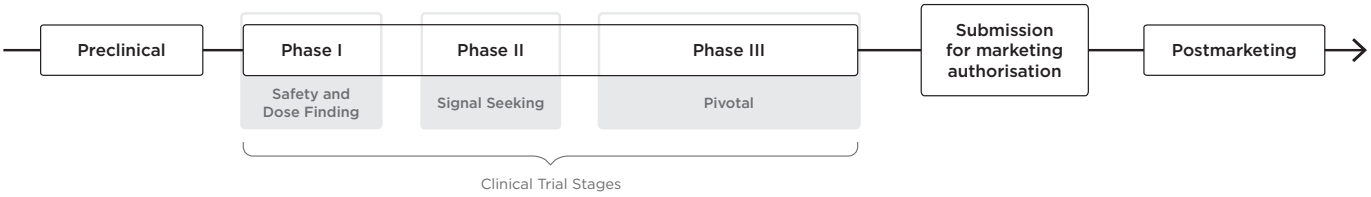


FIG 2.2. Paediatric Regulatory Key Time Points

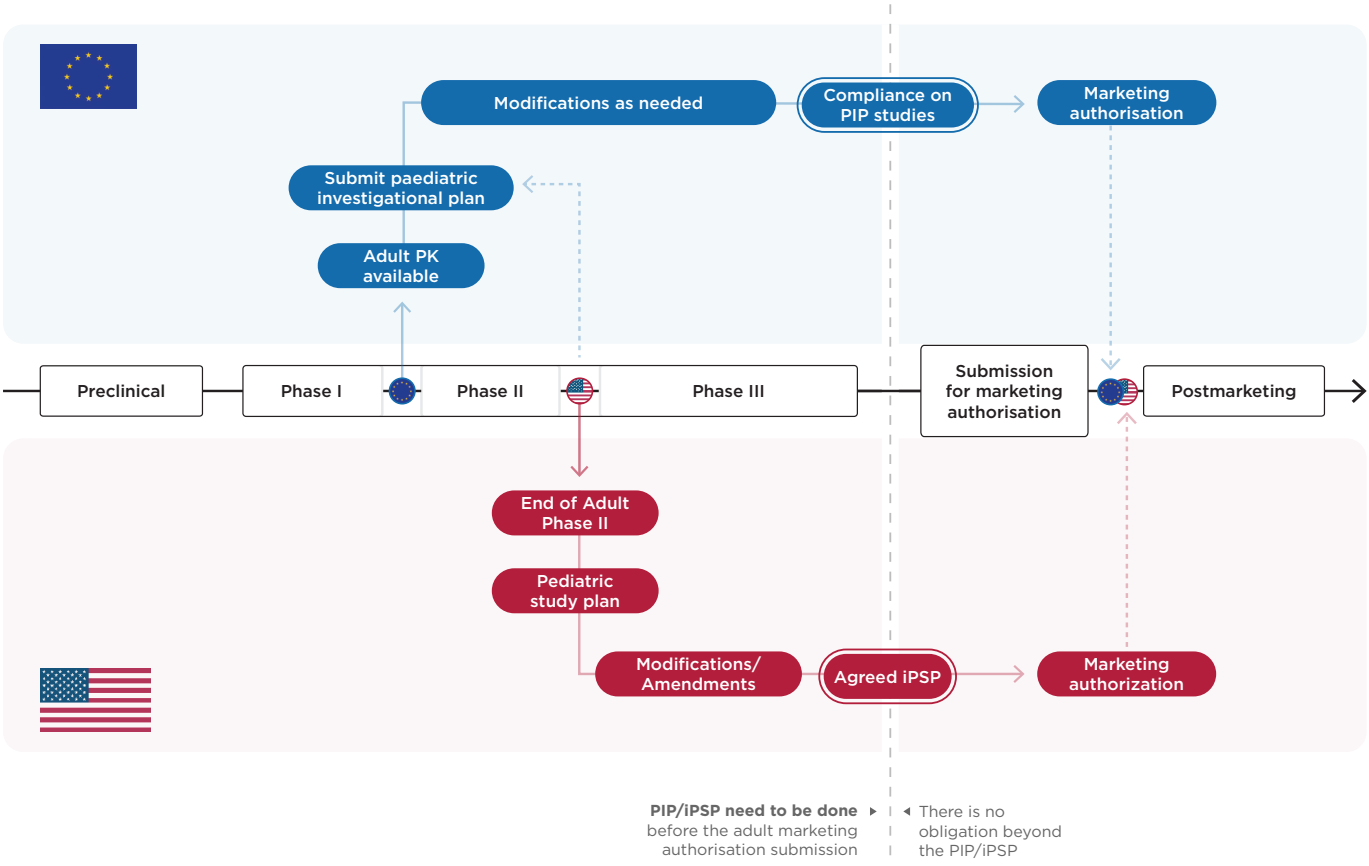
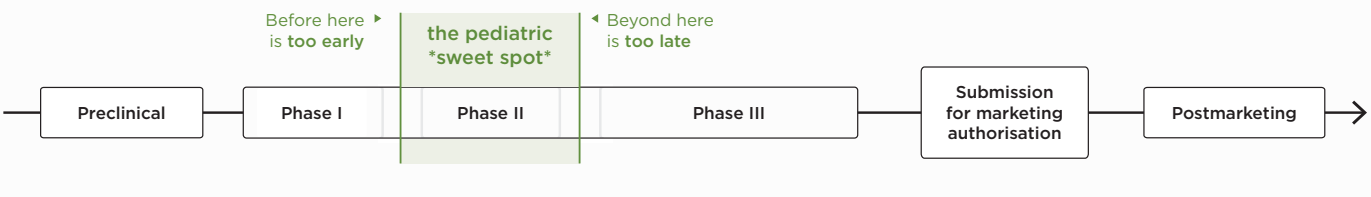


FIG 2.3. The Ideal **"sweet spot"** Within the Adult Drug Development Timeline



TRUST AND TRANSPARENCY

RULES OF ENGAGEMENT WITH INDUSTRY

We considered above the question of **how** and **when** the academic sponsor for the platform and the industry partner should interact to optimise development and delivery of a platform trial. The mechanisms for how academic paediatric oncologists can provide industry partners with advice are considered here. They can provide a range of expertise, including an understanding of the most pressing challenges in childhood cancer, knowledge of competing clinical trials under development, and access to networks of experienced institutions that treat a large proportion of patients with uncommon malignancies. Academic medical oncologists treating adult cancers typically require financial compensation when they provide advice to pharmaceutical partners at all stages (beyond initial informal discussion) of medicinal product review and study development. Historically, academic paediatric oncologists have been reluctant to request financial compensation for their expertise, in part driven by their desire to encourage industry partners to support paediatric clinical trials, which is not always a priority for industry. In practice, this results in academic paediatric oncologists providing free advice in comparison to medical experts consulting on drug development programs servicing adults.

Since academia-sponsored platform trials can be used by industry to meet regulatory requirements, there should be an expectation of fair market value compensation to academic paediatric oncologists by pharmaceutical partners for the advice they provide, recognising there may be national or regional differences in this value. Initial informational discussions between academic paediatric oncologists and pharmaceutical partners prior to execution of a confidential disclosure agreement can be done without compensation. However, as the discussions become more formal, the execution of an exploratory collaboration agreement detailing specific services provided for fair market value compensation is warranted. We propose that this type of exploratory collaboration agreements would be specific to the platform in question and may support activities such as PIPs/iPSPs

submission and/or protocol development as agreed between the parties prior to a full agreement covering all trial costs. Ideally, these agreements would include an international paediatric community perspective and patient advocate input.

The optimal timing and scope of an initial collaboration agreement for a platform trial is complex. A start-up agreement would provide compensation for early activity even if a clinical trial is not ultimately activated. However, the terms of a start-up agreement may be as legally complicated as a clinical trial agreement and its execution could delay initial work on a clinical trial if it is an absolute prerequisite. Alternatively, a full clinical trial agreement could include compensation for early advice, protocol design, and initial set-up activities, all of which would be done "at-risk" by the academic consortium without a start-up agreement. For these reasons, an individualised approach may be needed to balance the competing goals of providing early compensation and meeting study development timelines. At a minimum, compensation should be linked to completion of key milestones, including providing initial advice on relevance in paediatric populations and completion of a study design agreement.

The specific level of financial compensation should be based upon the typical costs related to industry-led medical advisory boards and reflect the time devoted by academia in developing a platform trial. Since academia-led platform trials are designed by consortia and delivered by an academic sponsor, usually a clinical or higher education institution, it is an opportunity for the company to receive collective and independent advice (see section **Evidence Based Consolidated Independent Expert Opinion**). Under these circumstances, the financial compensation would be to the platform sponsor or consortia not to the individual members of an advisory board. Academic consortia and industry partners should prospectively define the scope of their initial interactions and the deliverables to ensure an alignment of assumptions. Academic paediatric oncologists

selected by an academic consortium or trial platform academic leadership to participate in the platform trial design activities should reflect international and institutional diversity, and they should participate on behalf of the academic consortium and platform trial sponsor rather than as individuals or representatives of their local institutions. Convening a multistakeholder advisory board will optimally accomplish these goals. The scope should be defined in advance of the engagement. These 'advisory boards' providing advice to pharmaceutical partners in the development of platform studies must include patient advocates representatives, who should have a broad interest in drug development in children as well as expertise in particular patient groups.

PATIENT-FIRST APPROACH

START TO FINISH PATIENT ADVOCACY

Patient advocates are vital in paediatric oncology research, with unique expertise of their lived experience and consequent important perspectives on the disease and its treatments. They bring a strong commitment to improving outcomes for children. In a complex research landscape, they provide an essential bridge between stakeholders, promote accountability, and have historically driven meaningful change in the field.

Several systemic gaps hinder the effective integration of patient advocates within platform trials for childhood cancer. First, trial governance structures do not routinely incorporate advocates into key trial decision-making bodies such as Trial Steering or Data Monitoring Committees. Consequently, roles, responsibilities, and the decision-making authority remain poorly defined for patient advocates, and multi-arm platforms introduce additional ambiguity regarding accountability for advocate engagement.

Second, there is a lack of standardised training programs to equip advocates with the skills necessary to navigate scientific, regulatory, and operational complexities. Limited resources in academic sponsored

and publicly funded trials further constrains sustained involvement. Third, equity and representation challenges persist, with diverse patient and caregiver voices significantly underrepresented and no clear strategies to ensure inclusive participation across demographics and geographies.

Operational shortcomings include the absence of documented patient advocate engagement plans within trial protocols and the lack of dedicated budgets or resource allocation for advocacy support. Finally, accountability mechanisms are insufficient, as there are no established metrics to evaluate the impact of advocacy on trial design, conduct, or outcomes.

To address these gaps, the following **critical actions** are required**:**

- Development of standardised 'best practice' operational models that clearly define advocate roles, decision rights, term limits, and communication pathways.
- Creation of platform-level charters to guarantee sustained involvement, resource allocation, and bidirectional feedback.
- Implementation of comprehensive training frameworks and capacity-building initiatives.
- Establishment of measurable outcomes, including a requirement for every trial protocol to incorporate an "Advocate Plan."

Additional Resources:

Advocacy Papers References, Access Routes to Patient Advocates

Role of Patients and Advocates in Cancer Therapeutics Development: https://doi.org/10.1007/978-3-031-06357-2_9

The Crucial Role of Patient Advocates in Pediatric Oncology Research—Insights From ACCELERATE
https://doi.org/10.1001/jamapediatrics.2024.1877

CTTI Recommendations: Patient Group Engagement
https://ctti-clinicaltrials.org/wp-content/uploads/2021/06/CTTI_Patient_Group_Engagement_Recs.pdf

Patient Advocates and Researchers as Partners in Cancer Research: A Winning Combination
https://ascopubs.org/doi/10.1200/EDBK_100035

Patient-Centered Drug Approval: The Role of Patient Advocacy in the Drug Approval Process
https://www.jmcp.org/doi/full/10.18553/jmcp.2017.23.10.1078

OPERATIONAL CONSIDERATIONS

SUSTAINABLE INFRASTRUCTURE

Operational Value Proposition

Academic-sponsored platform trials, conducted in collaboration with industry partners, require clear agreements on operational responsibilities to ensure scientific rigor, regulatory compliance, and efficient execution. We have outlined the primary areas of responsibility for academic sponsors and shared functions that may involve industry partners.

Academic Sponsor Responsibilities:

Academic sponsors typically lead the following:

- **Scientific Leadership:** Disease-specific expertise, formulation of key research questions, and prioritization of therapies for evaluation.

- **Governance & Oversight:** Trial Steering Committees, Data Monitoring Committees and leadership teams to guide trial strategy.
- **Regulatory Role:** Acting as the ***legal sponsor of the platform trial**, holding ***IND** (US), interacting with FDA/EMA, EU and other competent authorities (e.g. Canada, Australia and New Zealand etc.) in relation to seeking scientific advice, clinical trial approval and managing safety requirements.
- **Operational Infrastructure:** Project and trial management, site qualification and training, vendor selection, and trial implementation.
- **Data and Quality Management:** Biostatistics, data management systems, pharmacovigilance, auditing, and quality oversight.
- **Documentation & Compliance:** Development and maintenance of protocols, consent forms, safety memos, and regulatory submissions.
- **Public Transparency:** Managing trial registration and public-facing information (e.g., ClinicalTrials.gov, EU Clinical Trials Register).
- **Patient Engagement:** (see section Start to Finish Patient Advocacy).

*** SPONSOR** *as defined by the EU Clinical Trials Regulation (No 536/2014):*

An individual, company, institution or organisation which takes responsibility for the initiation, for the management and for setting up the financing of the clinical trial

*** IND:**

Investigational New Drug (Application)

A regulatory submission made to the FDA requesting permission to test a new drug or biologic in humans through clinical trials.

Shared Responsibilities:

Certain functions may be undertaken by either academic or industry partners and may involve additional contracted third parties. The arrangements for shared or delegated responsibilities needs to be negotiated between the academic and industry partners:

- Biostatistical support
- Correlative studies, biomarker analysis, and pharmacokinetics
- Monitoring activities
- Drug distribution and labeling
- Response assessment and specialized expertise (e.g., surgical or radiation)

Industry Responsibilities:

Certain responsibilities remain the responsibility of the Industry partners throughout the trial, for example, PIP/iPSP submissions to regulatory Authorities and oversight of compliance. PIP/iPSP submission and fulfillment of PIP/iPSP obligations are the sole obligation of the industry owner of the asset, therefore close collaboration and transparency within the agreed contract frameworks between industry and the academic sponsor are key. See tools *Likely Pharma–Academic Pain Points and Practical Solutions »* and *Intellectual Property and Data Sharing Contract Principles for Platform Trials »*. Successful platform trials depend on robust collaboration and clearly defined roles across scientific, regulatory, operational, and quality domains, clearly agreed upon at the onset of co-development of the platform.

TRUST AND TRANSPARENCY

TOOL: Operational Considerations for Academic-Industry Collaborative Platform Trials in Paediatric Oncology GO

STRATEGIC PRIORITIZATION

Evidence Based Consolidated Independent Expert Opinion

Selecting medicinal products for inclusion in a platform trial is a critical step. While a “first come, first served” approach—based solely on the timing of industry partner engagement—may seem straightforward, it does not guarantee that the most appropriate products are chosen. This approach also fails to address situations where multiple products from the same drug class are proposed by different partners.

Traditionally, the pharmaceutical industry convenes advisory boards to gather expert opinions from their selected key opinion leaders on

their medicinal products. For an academic-sponsored platform trial, we propose adapting this model by establishing an advisory committee for platform trials led by the academic sponsor. This committee would review all available pre-clinical and clinical data for each proposed medicinal product and provide an independent, evidence-based assessment of its priority in paediatric drug development and its for inclusion in the platform. This committee's role is to deliver topic-specific expertise and issue a consolidated, unbiased recommendation—referred to as an Evidence-Based Consolidated Independent Expert Opinion (EBCO).

The committee providing the EBCO will review each pharmaceutical partner's drug information pack promptly, following a published timetable, for example: [Glo-BNHL collaboration process]. The review will assess the scientific merit, evidence of activity in the relevant indication (paediatric and adult data where applicable), feasibility of delivery, and mechanism of action to determine prioritisation for platform inclusion of the medicinal product. A formal decision statement with rationale will be issued to the industry partner within the agreed defined timeframe after receipt of the complete information pack

The EBCO committee will comprise internationally recognised disease experts representing the trial's geographic scope, including experts external to the trial platform where necessary. Members should be active leaders in major international study groups or consortia.
A conflict-of-interest policy, (such as the example adapted from the Glo-BNHL trial), must be implemented with a managed register available upon request. See tool ***Evidence-Based Consolidated Independent Expert Opinion (EBCO) for Academic-Industry Collaborative Platform Trials in Paediatric Oncology »***.

In summary, the EBCO committee should provide authoritative guidance on the development pathway of a medicinal product and its inclusion in the platform. This type of effective review committee providing formal scientifically justified opinions, alongside regulatory

endorsement of the trial platform increases industry confidence and alignment with regulatory requirements see tool ***Best Practice in Regulatory Interactions »***.

Suggested Terms of Reference for an EBCO Committee in paediatric oncology platform trials and exemplars of operational documents from the GLO-BNHL trial are provided in the toolkit.

TOOL: Evidence-Based Consolidated Independent Expert Opinion (EBCO) for Academic-Industry Collaborative Platform Trials in Paediatric Oncology

TOOL: Glo-BNHL Industry Information Pack: Guidance Submitting Asset Information to the Glo-BNHL Trial Steering Committee v4.0

TOOL: Glo-BNHL Asset Submission Form v1.0

TRUST AND TRANSPARENCY

Pain Points in Contracting

Collaborative platform trials involving academic trial sponsors and pharmaceutical companies as partners often face recurring challenges around data ownership, intellectual property (IP), publication rights, and governance. Academic clinicians typically assert that study data should remain under their control for sharing and reuse, while industry partners require strong, global rights to use all trial data for regulatory and commercial purposes. A practical solution is to clarify roles in contracts, designating the academic sponsoring institution as the

custodian of study data and granting companies a broad, perpetual license for regulatory and IP-related uses. Rules for onward sharing and potentially a joint data access committee can help ensure transparency and protect product-specific confidentiality.

IP is another sensitive area. Academic sponsors worry that companies might claim platform innovations as product IP, while industry partners fears losing ownership of product-specific discoveries. To address this, resulting IP should be split into two categories: product-specific IP, owned by the industry partner with academic inventorship acknowledged, and platform or methodology IP, owned by the academic sponsor but licensed for company's use. A decision matrix and joint invention review processes can provide clarity and fairness.

Publication control also requires balance. Academic sponsors seek timely dissemination of results, whereas Industry partners need to safeguard trade secrets and patentability. Standardised timelines for Industry partner review of publications, limited to confidentiality and factual accuracy, combined with commitments to publish all results and fallback mechanisms for delays, can prevent undue influence while protecting legitimate interests.

Data sharing policies should reflect both openness and risk management. A tiered access model ranging from restricted company-specific data to aggregated public summaries, along with robust data use agreements and pre-defined "no-go" zones for sensitive analyses, can reconcile transparency with competitive concerns, remaining mindful that trial data should be used to maximal patient benefit.

Platform trial governance structures must separate scientific independence from operational oversight. Considerations for negotiable rights with regards to trial steering committees' autonomy on scientific decisions, while Industry partners retain rights to monitor safety and data quality. Joint charters, clear voting rights, transparent documentation, and clear definitions with mutual understanding of "by whom" and "by when" help avoid perceptions of undue influence.

Finally, consistent terminology and clear definitions are essential to prevent confusion and mistrust. A shared glossary across agreements and drafting checklists can standardise language. It is recommended to also consider future data use, which can be categorised by risk. A potential model could be designed to enable low-risk academic analyses and publications, while requiring collaboration or consent for higher-risk projects. Structured collaboration opportunities and safeguards for proprietary information can support academic freedom without compromising industry's freedom to operate.

These principles are captured in these C3PT tools and aim to guide constructive dialogue and form the basis for a shared IP and data governance framework, including a model clause set for future platform collaborations.

TOOL: Likely Pharma–Academic Pain Points and Practical Solutions

TOOL: Intellectual Property and Data Sharing Contract Principles for Platform Trials

SUSTAINABLE INFRASTRUCTURE

Platform Trial Funding and Operational Models

Whilst a direct comparison is not possible without knowing the full cost of an equivalent industry sponsored trial, it was agreed that transparency on the elements that contribute to the cost to industry of an academic-sponsored platform trial would help Industry assess the fair market value of the proposition.

The workshop discussants reviewed the funding and operational models of five childhood cancer collaborative platform trials (Glo-BNHL, PedAL, ESMART, MATCH, Hem-iSMART), collated to create a comprehensive table outlining funding sources, operational models, and regulatory processes for these five platform trials. The objective was to clarify cost drivers and delineate responsibilities for each trial and arm. Terminology was standardised, including definitions for funding categories (e.g., charity = philanthropy), sponsor roles, and legal entities to ensure consistency (TABLE 1).

Academic-sponsored platform trials can present unique cost and operational considerations for industry partners. The timing of the costs incurred is also a consideration with the need for frontloaded set up costs for academic platform trials. The funding structures for the existing platforms reviewed varied significantly. For example, the core infrastructure for trials such as Glo-BNHL and PedAL is largely supported through philanthropic contributions, whereas ESMART and MATCH employ mixed funding approaches. At the individual trial arm/protocol level, funding typically combines resources from industry, grants, and philanthropy, reflecting the collaborative nature of these studies.

Operational delivery models also differed by academic sponsor and by trial design, incorporating approaches such as delegating responsibilities to contract research organizations (CROs), national coordinating centres (NCCs), or hybrid systems. Monitoring and safety oversight are managed through diverse arrangements, including academic sponsor-led models, CRO contracts, or joint frameworks.

Regulatory submissions and advice also follow different pathways. Responsibilities were adopted by academic sponsors and/or delegated to CROs, or NCCs, with regional differences in engagement with agencies such as the EMA and the FDA. The way the role of the academic sponsor was adopted differed between trials: for example, the charity BCU (previously LLS) was the trial sponsor for PedAL in

North America with the Princess Maxima Centre in the Netherlands taking the role of European sponsor, whereas the NCI/COG was the trial sponsor and co-ordinating centre for MATCH delivered solely in North America. Other international trial platforms relied on academic institutions as the international sponsors, for example University of Birmingham as global sponsor for Glo-BNHL.

Additional cost drivers include central laboratories, database management, imaging review, statistical analysis, clinical study report ***(CSR)** drafting, and pharmacokinetic (PK) laboratory services. Correlative studies and sample logistics often depend on charitable or public funding, adding further complexity to the financial and operational landscape.

In summary, academic-sponsored platform trials require collaborative funding models and operational flexibility. Clear agreements on operational roles, responsibilities, and cost-sharing are essential to ensure efficiency and regulatory compliance. Transparency in these cost drivers will help industry partners assess fair market value and make informed decisions about participation.

***(CSR):**

a Clinical Study Report

A CSR is the integrated scientific and regulatory report of a completed clinical trial, documenting how the study was conducted and what results were obtained.

CSRs are typically prepared according to the ICH E3 Guideline on Structure and Content of Clinical Study Reports and form a key component of regulatory submissions.

TABLE 1:

Summary of Funding and Operational Models

Trial	Glo-BNHL	PedAL	ESMART	MATCH (enrolment complete)	Hem-iSMART
Infrastructure Funding	Philanthropy (Charity)	Philanthropy (Charity)	Philanthropy (Charity), INCa	NCI	Philanthropy grants
Arm Funding	Industry	Industry/ Philanthropy	Industry (drug), Grant awards, INCa	NCI	Philanthropy/ Industry
No. of Arms (Dec 2025)	2	3	13	13	3
Use of CRO	No	Yes	No	No	Academic arms: No; Intent-to-file: Yes
Use of NCCs	Yes	Yes	Yes	No	Yes

Trial	Glo-BNHL	PedAL	ESMART	MATCH (enrolment complete)	Hem-iSMART
Monitoring	Sponsor-out-sourced	CRO contracted by sponsor	Sponsor	NCI/COG	CRO contracted by sponsor
Safety Reporting	Sponsor-outsourced	CRO (US), Academic sponsor/ CRO (EU)	Sponsor/ NCC	NCI/COG	Sponsor
Regulatory Submission	Sponsor/NCC	Sponsor/CRO	Sponsor/NCC	NCI	Academic arms: Sponsor/NCC; ITF arm: CRO
Regulatory Advice	EMA/FDA	Sponsor	EMA (EU), FDA (US)	Some arms	FDA; Sponsor with EU Competent Authority
Sponsor	Academic	Independent (US, Canada, Australia)	Academic (EU, UK, Israel)	NCI	Academic
IND Holder	Sponsor	Independent Sponsor	Sponsor	NCI	NA
Central Labs	Industry partner	Sponsor	Sponsor	Sponsor	Academic labs for PK/response; ITF arm includes PK, PD
Central Review	Sponsor-outsourced	NA	Imaging and sequencing reanalysis	Imaging response review	Academic arms: central response; ITF: central response
Database	Sponsor-procured (commercial)	CRO cloud-based	Sponsor	Trial Database COG; NCI database for matching participants genomics to sub-protocols	Sponsor database for all arms
CSR Author	Industry partner	Sponsor for CTIS; Industry for RA submission	Sponsor	Industry partner	Sponsor
PK Lab	Industry partner	Sponsor	Industry PK lab/ CRO; academic lab	COG and industry (subprotocol-dependent)	Academic lab; ITF arm uses commercial lab
Subject Insurance	Sponsor-procured	EU: Sponsor; US: Independent sponsor	Sponsor	Patient private/ public third party payer; COG trial insurance; NCI insurance for genomic matching database.	Sponsor

Articulating a Strong Value Proposition

The potential value proposition of a childhood cancer academic-industry collaborative platform trial is not always visible to potential industry partners. Approaches to potential industry partners from academic clinicians are usually considered under the conventional ***investigator-initiated trial (IIT)** banner and the possibilities for collaborative working to mutual advantage under the banner of an academic sponsored academic-industry collaborative platform trial is not realised. To promote the platform trial's value proposition, the toolkit proposes an informative marketing slide set template to aid the academic team in its marketing of the platform to industry. The purpose of this slide set is to outline the advantages of a structured platform trial road map for evaluating medicinal products for paediatric cancers using a platform trial model. This slide set outlines how the platform trial can support the regulatory mandates that require pharmaceutical and biotechnology companies to evaluate the safety and efficacy of oncology drugs in paediatric populations. It describes how the platform trial model can support compliance with the requirements of regulatory bodies such as the FDA and EMA, including submission of PIPs and iPSPs and promotes the platform's ability to deliver efficient trial design, address ethical considerations, and data extrapolation from concurrent trial data. The slide set fosters collaboration among stakeholders to accelerate the development of paediatric-specific oncology therapies.

The intended audience primarily comprises pharmaceutical, and biotechnology companies engaged in oncology drug development, particularly those compelled by regulations (e.g., the FDA's RACE for Children Act, EU Pediatric Regulation or the new EU Pharmaceutical Legislation) to incorporate paediatric studies into their pipelines. This includes research and development teams, regulatory affairs specialists, clinical trial managers, and executives in mid-to-large pharma/biotech firms who must navigate these requirements to avoid delays in adult

***IIT:**

Investigator-Initiated Trial (sometimes called an Investigator-Initiated Study)

An IIT is a clinical trial in which the investigator or academic institution, rather than a pharmaceutical company, takes responsibility for the design, conduct, management, and sponsorship of the study.

drug approvals or face penalties for non-compliance. The content is tailored for professionals with a working knowledge of oncology but seeks to bridge gaps for those less experienced in paediatric-specific challenges, such as age-appropriate dosing, rare disease subsets, and long-term survivorship monitoring.

The use of the slide set template to promote an academic sponsored clinical trial contributes to a unified road map that introduces companies to the inherent value proposition of the academic-industry collaborative platform trial model in evaluating their medical product. They will have an understanding of the process, timelines, cost, data sharing, and regulatory success rates. By promoting access to evidence-based strategies, it could expedite access to innovative treatments for paediatric cancer patients, minimize ethical risks, and support global harmonization efforts, ultimately leading to better outcomes in a field where childhood cancers remain a critical unmet need.

TOOL: Marketing the Value Proposition (slide-set)

FUTURE PERSPECTIVES

The C3PT Blueprint provides guidance for all stakeholders engaged in drug development for children and young people with cancer, demonstrating how Academic-Industry Collaborative Platform Trials can accelerate the evaluation of innovative therapies and improve patient outcomes.

Stakeholders across academia, industry, regulatory agencies, funding organisations, and patient advocacy groups recognise that current processes can be lengthy and resource-intensive, with individual

amendments often requiring separate review and compliance activities across multiple parties. To fully realise the efficiencies offered by platform trials, meaningful policy, regulatory, and legislative developments may be needed to support more streamlined and proportionate amendment pathways. As the paediatric oncology community gains further experience in the design, governance, and delivery of academic-industry collaborative platform trials, the C3PT Blueprint should be viewed as a living framework that can evolve iteratively in response to emerging evidence, operational learning, and stakeholder feedback.

From the perspective of patients and families, the promise of platform trials will only be fully realised if the systems that support them are designed with the same care and innovation as the science they seek to advance. Patient advocates should be embedded as equal partners in platform trial governance, with clearly defined responsibilities, appropriate resourcing, and meaningful involvement from protocol development through to data sharing and dissemination of results. The complexity of multi-arm, adaptive studies should never create additional burden for families already facing the challenges of a childhood cancer diagnosis. Clear and accessible participant information, user-friendly digital engagement tools, and transparent communication regarding the value and impact of participation should become standard practice.

Looking ahead, the paediatric oncology ecosystem must also invest in sustainable infrastructure for long-term follow-up, ensuring that safety, survivorship, and quality-of-life outcomes are captured with the same rigour as efficacy endpoints. Such investment will be essential to understanding the full impact of novel therapies across the lifespan of children and young people treated within these studies.

Ultimately, the success of the C3PT Blueprint will not be measured solely by its ability to accelerate trial activation, regulatory decision-making, or access to innovative medicines. Its true success will be

judged by whether it enables more effective collaboration, generates robust evidence more efficiently, and delivers treatments that meaningfully improve survival, quality of life, and long-term outcomes for children and young people with cancer. Through continued partnership, innovation, and commitment across all stakeholders, Academic-Industry Collaborative Platform Trials have the potential to transform the development of paediatric cancer therapies and establish a new paradigm for bringing better treatments to patients faster.

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