2026

v 1.0

Value Proposition

of the Childhood Cancer Collaborative Platform Trials (C3PT) to Industry Partners

Key Issues Identified by Industry

Regulatory Complexity

- US RACE for Children Act and EU Legislation require early paediatric development plans
  - iPSP/PIP submissions often required before adult efficacy data are available
- Differing expectations from FDA and EMA, despite increased coordination

Recruitment Feasibility

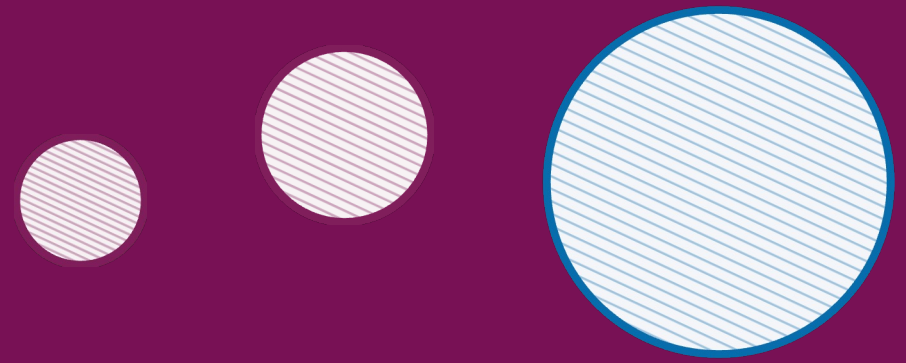
- Small patient populations
- Competing studies
- Rare molecularly defined cancers

Study Delivery

- Paediatric specific dosing, formulation and safety considerations
- Ethical and consent requirements
- Commercial incentives inadequate for compelling business case
- Pressure to deliver rapidly

Our proposed solution is the utilization of

Academic-sponsored Platform Trials



Why Industry Should Engage with Academic Platform Trials?

PATIENT ACCESS:
Faster enrollment and quality data

OPERATIONAL EFFICIENCY:
Proven frameworks reduce setup time and costs

REGULATORY ADVANTAGE:
Supports PIP/iPSP obligations and speeds approvals

ESTABLISHED TRACK RECORD:
Successful global collaborations

PATIENT BENEFIT:
Expedited development of safe and effective treatments for children and young people with cancer

What is a **Platform Trial?**

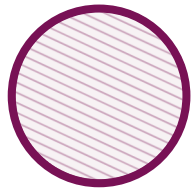
A **platform clinical trial** is designed to **evaluate multiple treatments** at the same time within a single, ongoing trial structure, usually for the same disease or condition.

Treatments are evaluated for safety and /or efficacy in **independent arms**

Academic-led Platform Trials Offer:

- Structured paediatric oncology networks with **disease and site expertise**
- **Established framework** for rapid setup, efficient recruitment, and cost-effective delivery
- **Regulatory endorsement** via EMA and FDA scientific advice
- **Avoidance of competition** for patients between independent industry trials

What is our **Value Proposition?**



Patient Access:
Platform trials deliver quality data in a rare patient population that could lead more rapidly to patient benefit.

Operational Effectiveness:
Platform trials provide Pharma and Biotech access to subject area expertise and an efficient trial delivery mechanism, with clear budgets and timelines

Regulatory Alignment:
Platform trials are an effective mechanism whereby paediatric strategies and development plans can be met efficiently with real time access to regulatory authorities and can support paediatric regulatory obligations

Patient Access

- **Access to patients is unparalleled:**
  - Academic Platform Trials are delivered within well-established academic consortia comprising international networks of leading specialist paediatric oncology centres, facilitating ease of enrollment

- **Access to topic-specific expertise:**
  - Academic Platform Trials are led by KOLs who are experienced clinical investigators with topic-specific expertise and extensive hands–on experience in paediatric oncology patient care and designing trials alongside patient advocates for patient benefit

Operational Effectiveness

- **Experienced Operational Team:**
  - Accelerated site set-up through direct access to clinical operations and methodology experts with specialist paediatric oncology expertise, helping sites navigate complex study-specific and operational challenges

- **Operational Ease and Cost-Efficient Delivery**
  - Track record in delivering adaptive designs
  - Addition of new 'arms' to established and open Academic Platform Trials is time and resource efficient
  - Established platforms have agreed regulatory endorsed endpoints/designs
  - Less time taken overall which translates to less operational costs

VALUE PROPOSITION

Regulatory Alignment

- **Academic Platform Trials can enable companies to:**
  - Deliver paediatric strategies and development plans
  - Meet regulatory obligations for agreed PIPs and iPSPs
  - Access added paediatric oncology expertise

- **Academic Platform Trials' prior engagement with regulatory authorities provides:**
  - Endorsement of the trial specific methodology
    - Assurance on the acceptability of the trial's patient population, trial endpoints and statistical principles
  - Regulatory authority familiarity with the platform trial
    - Potential for more efficient regulatory negotiation timelines

Established Track Record: Delivering for Industry and Patients

Measure	Across the 3 Platforms*
Years of operation	2015-2025+
Industry assets included	21+
Company partnerships	12+
Regulatory programmes supported	Multiple PIPs/iPSPs, FDA and EMA submissions

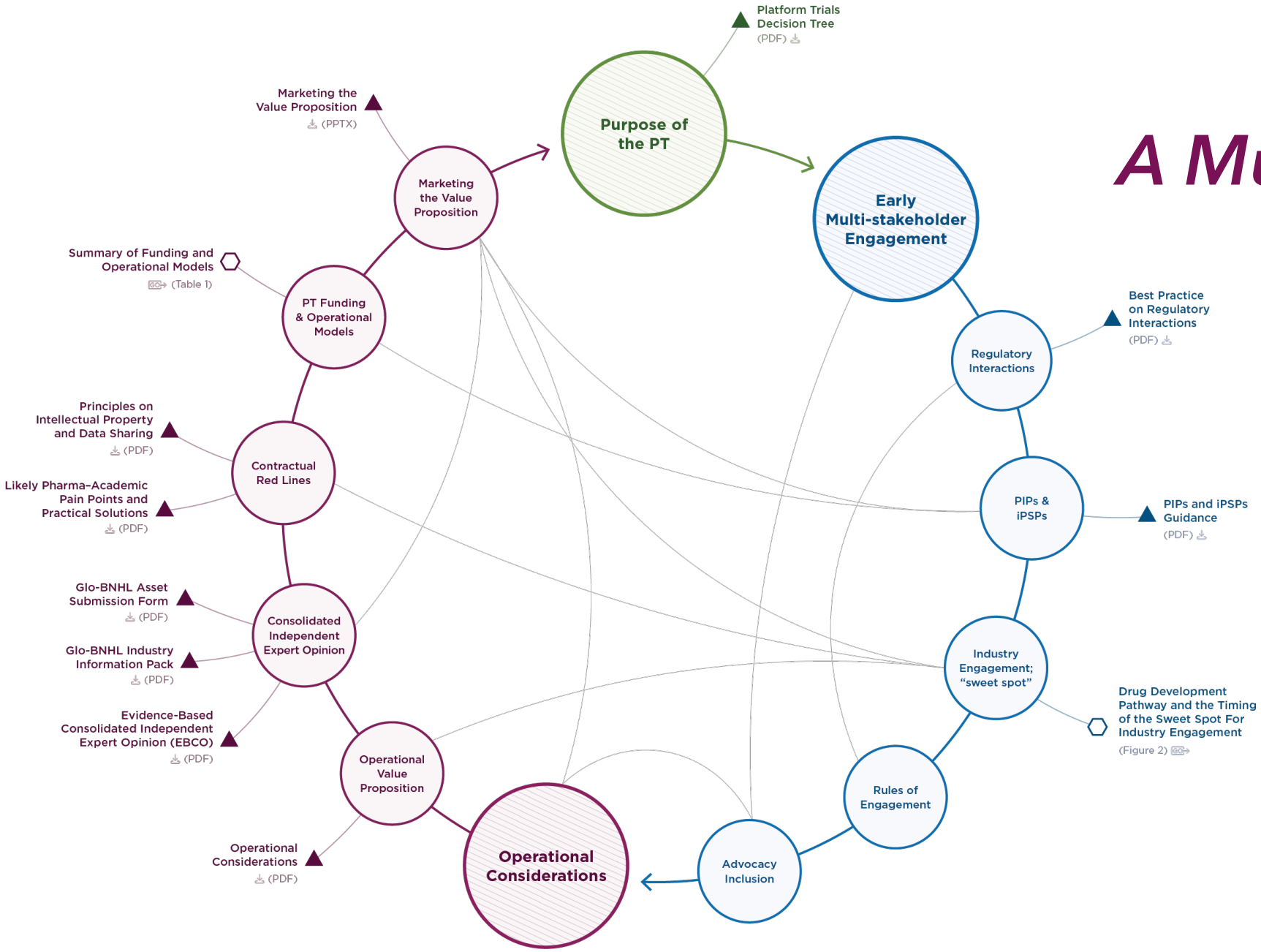
*Based on Exemplar Academic-Industry Collaborative Platform Trials include: AcSe-ESMART, Glo-BNHL, Hemi-iSMART

Industry Engagement with Academic Platform Trials

Transform promising therapies into patient benefit faster through patient access, trial efficiency, regulatory readiness, and proven multistakeholder collaboration

C3PT

A Multistakeholder Collaborative



The path is laid.

Use the C3PT Position Narrative and tools to move from interest to informed action.

For more information on C3PT:

https://MRCTcenter.org/C3PT

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

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