

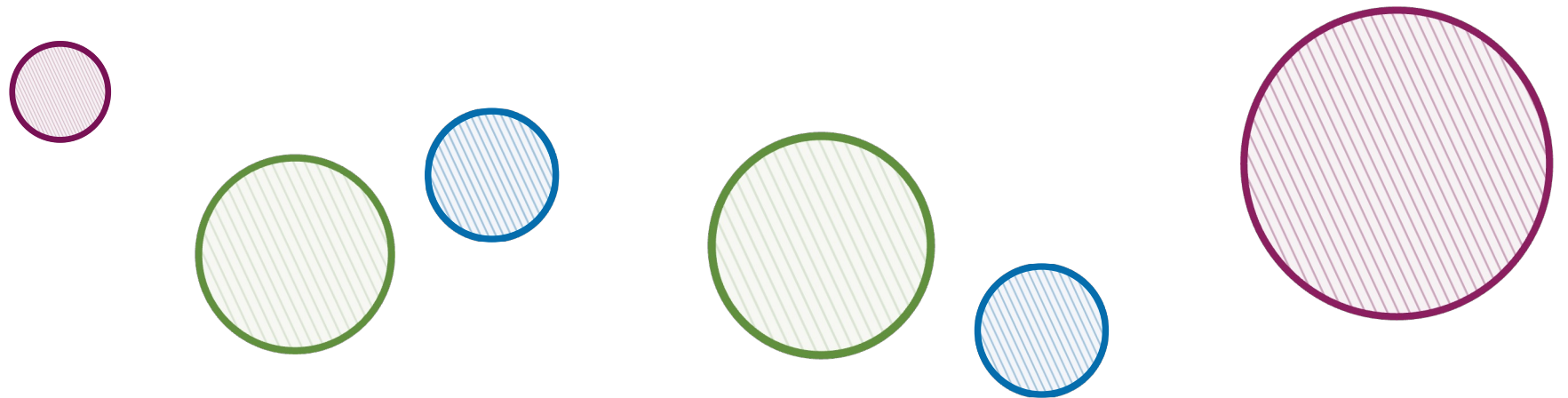


Childhood Cancer Academic-Industry Collaborative Platform Trials (C3PT) Initiative



Disclaimer

This position narrative was developed through a collaborative, multi-stakeholder process involving contributors from multiple disciplines, institutions, and organizations. The views expressed in this presentation represent the views of the C3PT author group as a whole and should not be attributed to any individual author. The views and recommendations do not imply endorsement by, or reflect the positions of, the EMA, FDA, individual organizations, the associated committees or working parties, or any other entity.

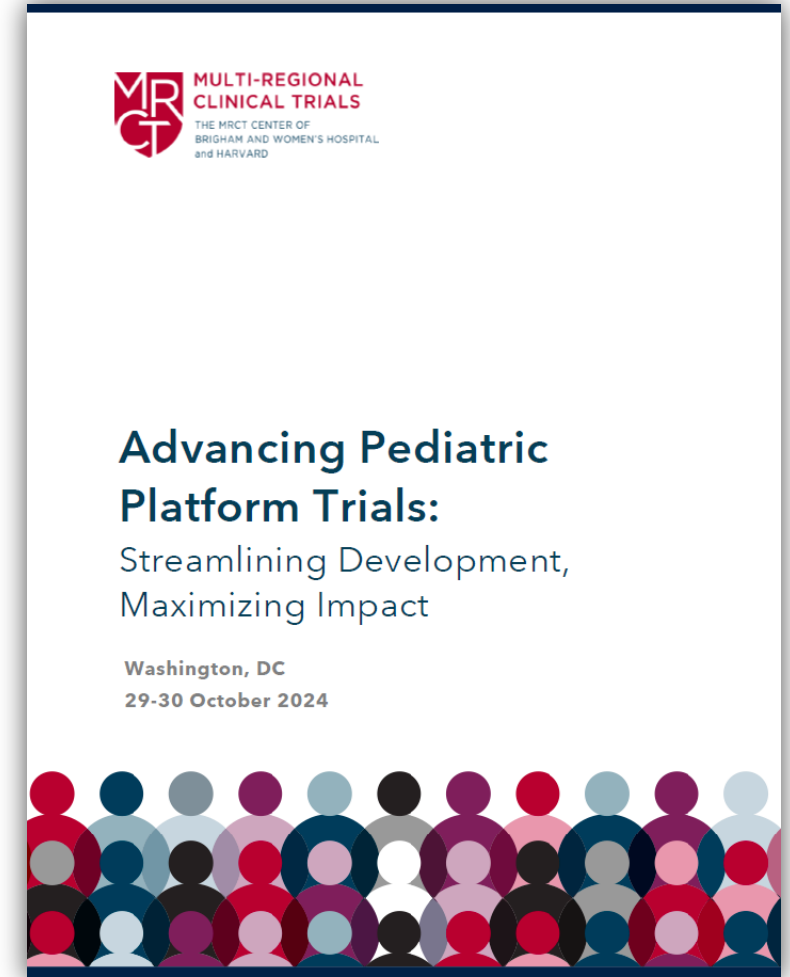


Pediatric Platform Trials: *Where we started*

In **October 2024**, the MRCT Center convened an in-person workshop to discuss **Advancing Pediatric Platform Trials** focused on Oncology, MDR-TB, MDD with ~80 multistakeholder attendees representing academia, industry, patient advocates, and regulators.

October 2024 Advancing Pediatric Platform Trials Workshop Sponsors:

- AstraZeneca
- Johnson & Johnson
- Sanofi



Building on the Foundation from the 1st Workshop

ORGANIZED BY:

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

 **ITCC**
INNOVATIVE THERAPIES FOR CHILDREN AND ADOLESCENTS WITH CANCER

 **25 CHILDREN'S ONCOLOGY GROUP**
1999-2024

 **LEUKEMIA & LYMPHOMA SOCIETY**

Childhood Cancer Academic-Industry Collaborative: Platform Trials Workshop

DATES:
24 July, 2025
8:30 am–6:45 pm EDT
25 July, 2025
7:30 am–2:00 pm EDT

Join us for a dynamic two-day workshop that will bring together an expert group of multi-stakeholder participants focused on developing an implementation framework for academic-industry collaborative platform trials for pediatric oncology. We can't wait to connect and share ideas; join us and be part of the solution!



Challenge identified:

How to Optimize Academic-Industry Collaboration in Pediatric Oncology Platform Trials

Multi-stakeholder Workshop

convened with ~40 attendees representing academia, pharma industry, patient advocates, and regulators (EMA and FDA)

Sponsored by:



Childhood Cancer Academic-Industry Collaborative Platform Trials (C3PT): **Aim** and **Objective**

Aim: Develop a framework of implementation for academic-industry collaborative studies that deliver data that could support a regulatory requirement (PIPs, iPSPs) and potentially marketing authorization applications.



Principal Objective: Agree on a “BLUEPRINT” for future platform trials, building on current experiences and lessons learned from representative academic-industry collaborative platform trials in paediatric oncology.



Definitions used within Programme

Platform Trial

A prospective disease or biologically defined target-focused trial that is adaptive and evaluates multiple, simultaneous or possibly differently timed interventions.

Academic-Industry Collaborative Platform Trial

An academic-sponsored platform trial, potentially involving multiple industry partners, that can evaluate the safety and efficacy of new therapeutic approaches and could generate data to support exploratory evidence, support regulatory decision-making and/or fulfill regulatory obligations.



Methodology



Mapped existing experiences of trial delivery processes | Case studies i) PEDAL ii) **Glo-B-NHL**



Reviewed **what worked**, what we **should continue**, what **needs to be improved** and what **should never be repeated**



Exchanged Stakeholder Perspectives: 'What does each stakeholder want to achieve out of an academic-industry collaborative platform trial?'



Brainstormed what was needed to **streamline and expedite delivery**



Formulated the process map for academic-industry collaborative platform trials



Agreed on what good looks like: The C3PT Blueprint

Thank you to all C3PT Blueprint Contributors

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Cornelis van Tilburg

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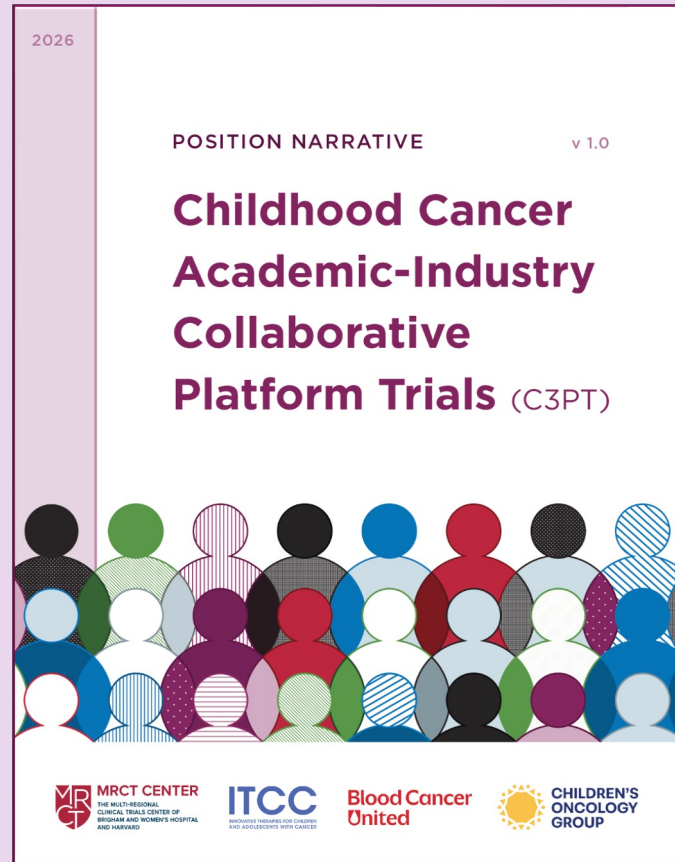
Michel Zwaan*

Planning Committee

Task Leads*



The C3PT Blueprint comprises



Position Narrative



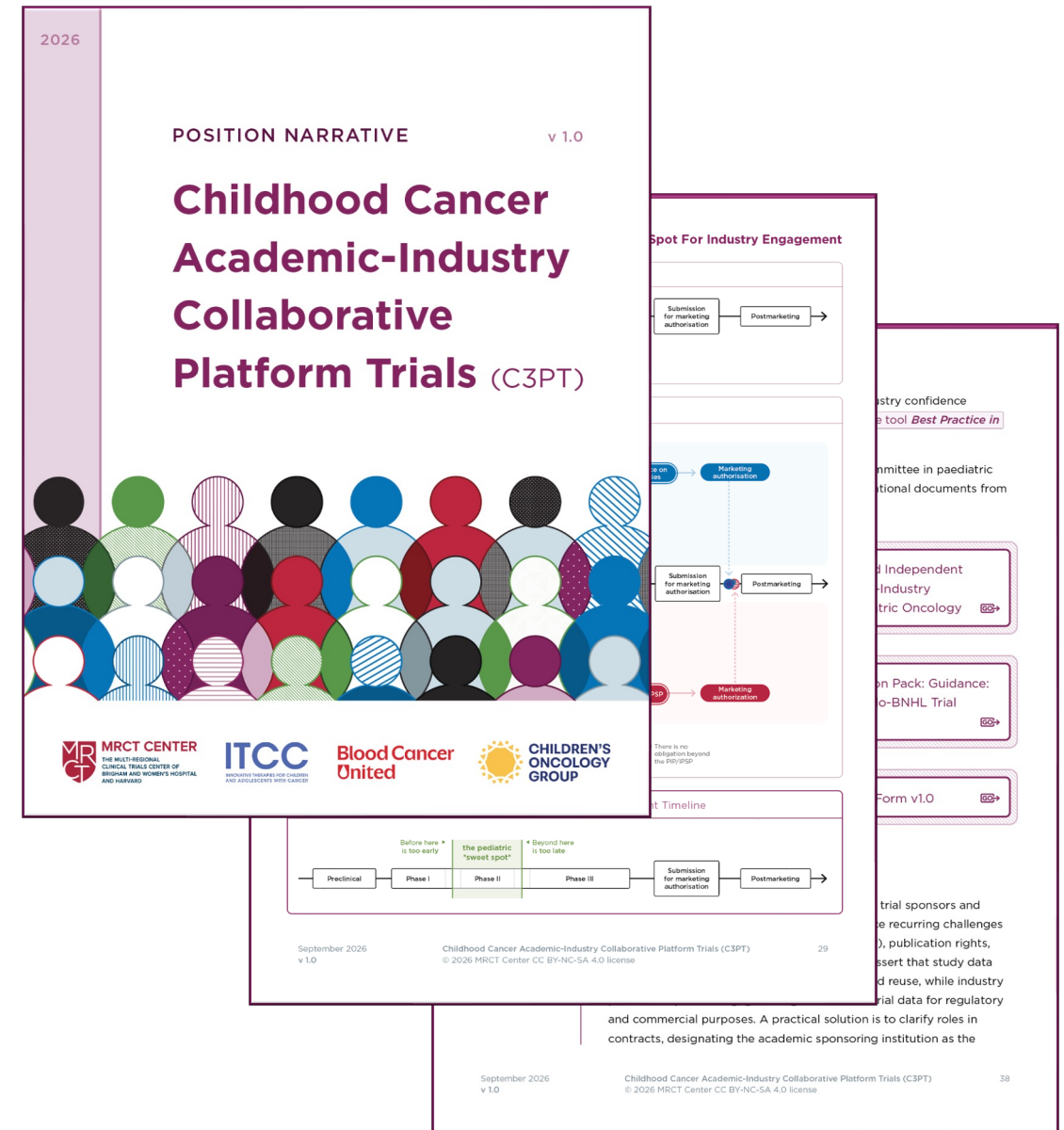
A Suite of Tools

C3PT Position Narrative

Presents a multi-stakeholder blueprint for the development and delivery of **Childhood Cancer Academic-Industry Collaborative Platform Trials (C3PT)**

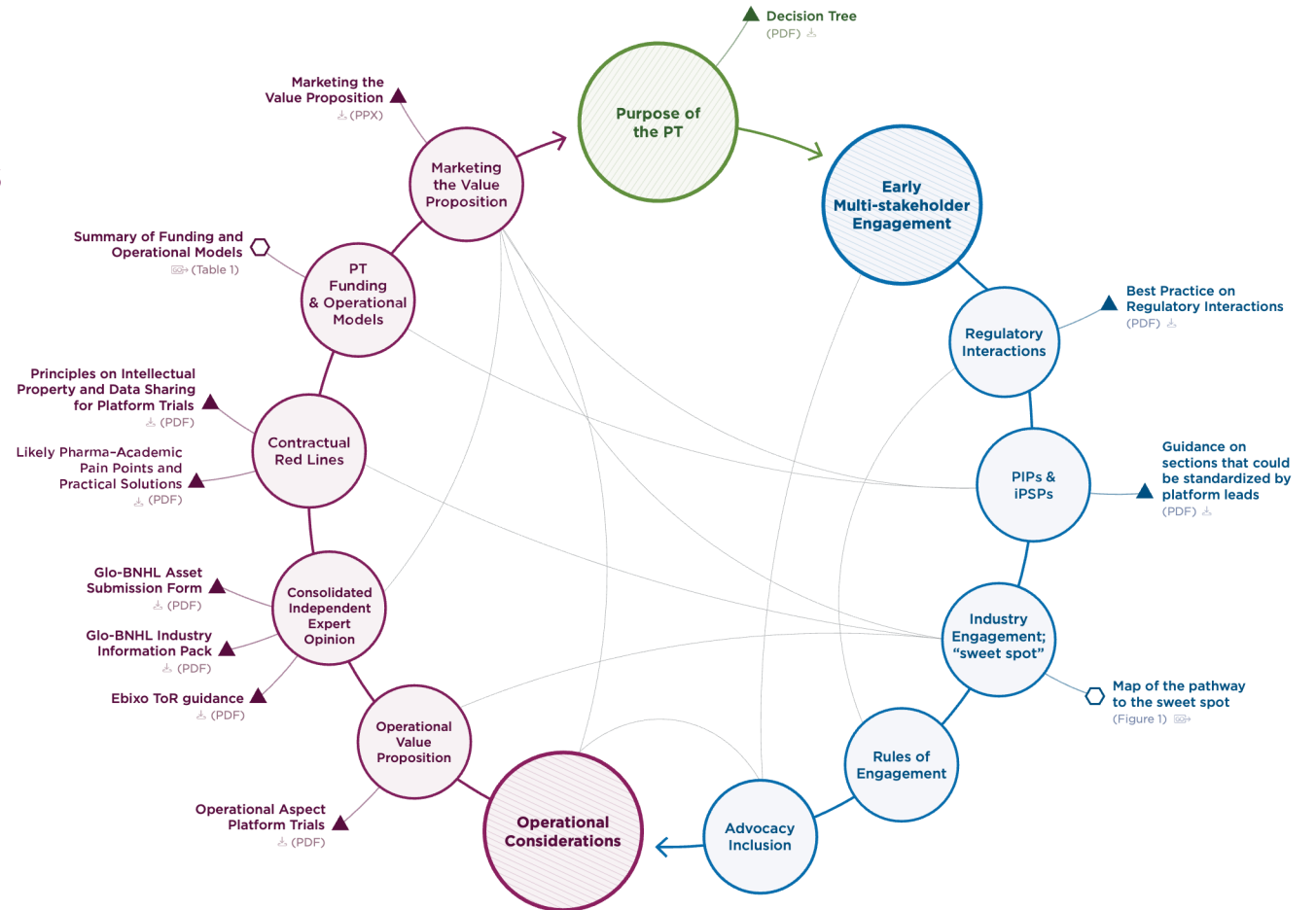
Principal Recommendations:

1. Define the Trial Purpose Early
2. Engage Key Stakeholders as Early as Possible
3. Improve Access to Investigational Assets
4. Optimise Operational Infrastructure
5. Sustainable Funding Models
6. Independent Asset Prioritisation
7. Contractual Principles
8. Articulating a Strong Value Proposition



The C3PT Toolkit

- Platform Trial Decision Tree
- Early Multistakeholder Interactions
 - Best Practices for Regulatory Interactions
 - PIPs and iPSP Guidance
 - The 'Sweet Spot' for accessing assets from Industry
 - Start to Finish Patient Advocacy
- Operational Considerations
 - Operational Value Proposition
 - Evidence-Based Selection of Assets for the platform trial
 - Pain points in Contracting
 - Funding and Operational Models
 - Articulating a Strong Value Proposition



Panel Discussion

Multi-Stakeholder Panelists

Patient Advocates:

- Amanda Monteiro
- Nicole Scobie

Regulators:

- Dominik Karres (EMA)
- Martha Donoghue (FDA)

Industry:

- George Kirk
- Kerri Nottage

Academia:

- Amos Burke
- Elizabeth Fox

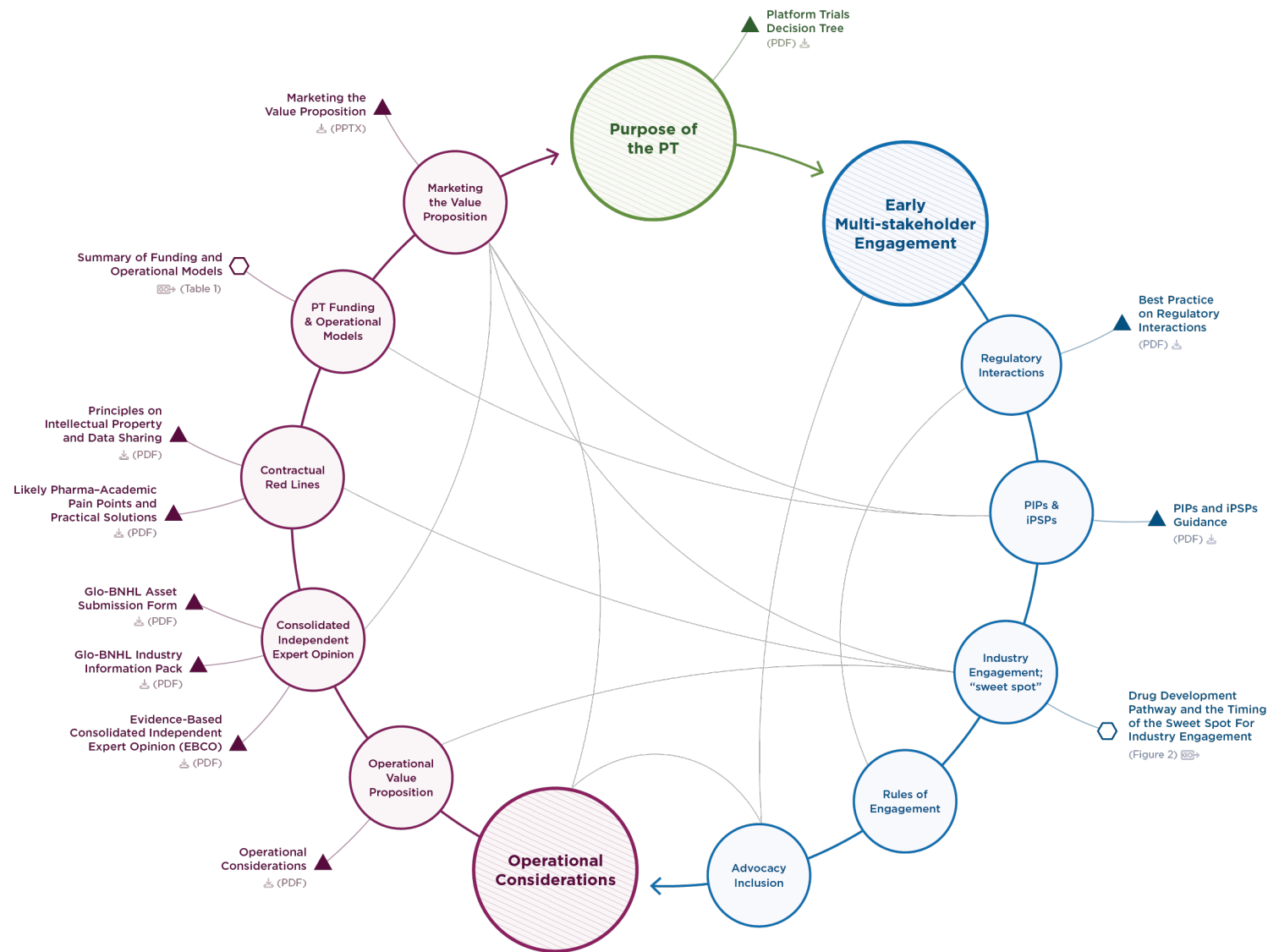
Please post your
questions in the
chat

C3PT

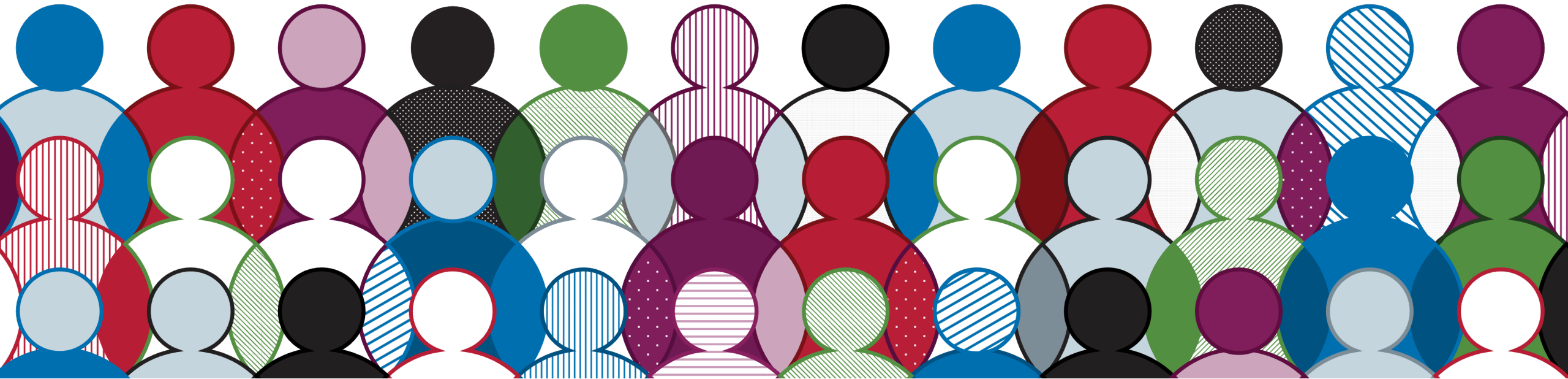
A Multistakeholder Collaborative

The path is laid.

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



Learn more at: <https://MRCTcenter.org/C3PT/>



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BRIGHAM AND WOMEN'S HOSPITAL
AND HARVARD

ITCC

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AND ADOLESCENTS WITH CANCER

**Blood Cancer
United**



**CHILDREN'S
ONCOLOGY
GROUP**