2026

C3PT TOOL

PIPs and iPSPs

Guidance for Collaborative Contributions to Writing

v 1.0

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

Purpose and Scope of Tool

There are several elements within PIPs and iPSPs where the academic platform trials team can valuably contribute as they the disease and /or target specific scientific and clinical knowledge and expertise. This tool is intended to inform industry of the sections where this expertise could be leveraged.

Introduction

It is proposed that the PIP and iPSP should be written collaboratively with early involvement of the platform trial team. The tool provides suggestions for who (platform trial team vs. company) should contribute to the content of these regulatory documents.

The guidance is divided into the section headers within the iPSP and PIP documents and organized to align sections in the iPSP and PIP that overlap in content. The suggested Contributing Parties are indicated by blue shading with any specific advice for that contribution included. There may be different guidance given dependent on whether the platform trial is disease-specific vs target focused. Please note that this guidance is intended to act as a starting point.

The Contributing Partners will be bespoke for each trial and drug product.

Overview of the Drug or Biological Product

Sections of iPSP	Sections of PIP	Contributing Party		
		Platform Trial Team		Company
		Disease-specific	Target focused	
2.1 Proposed Mechanism of Action of the Drug	2.1 Pharmacology and mechanism of action		Target focused platform may contribute background information on the molecular target and its role in oncogenesis	Company to contribute drug MOA and high-level summary of development program (i.e. indications under development)
2.2 Potential Therapeutic Benefits	2.4 Description of the fulfilment of therapeutic needs and/or significant therapeutic benefit 2.5 Proposed indication(s) in relation to the proposed condition and selected paediatric subsets	Disease-specific platform should contribute to the unmet need and potential benefit		

Background of the Disease

Sections of iPSP	Sections of PIP	Contributing Party		
		Platform Trial Team		Company
		Disease-specific	Target focused	
1.1 Pathophysiology of the disease				Company to provide overview of cancers in the pediatric population that may warrant evaluation based on the target/MOA, beyond the disease(s) encompassed within the platform study
1.2 Methods of diagnosis 1.3 Currently available treatments and/or prevention strategies in the pediatric population	2.3 Current methods of diagnosis, prevention or treatment in paediatric populations			
1.4 Incidence and Prevalence in the Overall Population	2.2 Summary of differences/similarities in the condition between populations (e.g. adult vs paediatric)			

Background of the Disease (continued)

Sections of iPSP	Sections of PIP	Contributing Party		
		Platform Trial Team		Company
		Disease-specific	Target focused	
1.5 Incidence and Prevalence in the Pediatric Population	2.2 Summary of differences/similarities in the condition between populations (e.g. adult vs paediatric)	Include region-specific and global estimates with emphasis on patients available to enroll in a clinical trial		
1.6 Key Differences Between Adults and the Pediatric Population	2.2 Summary of differences/similarities in the condition between populations (e.g. adult vs paediatric)			

Regulatory History

Sections of iPSP	Sections of PIP	Contributing Party		
		Platform Trial Team		Company
		Disease-specific	Target focused	
12. Agreements for Pediatric Studies with Other Regulatory Authorities	2.6 Summary of regulatory advice	Both disease-specific and molecular target-focused platforms and Company to include regulatory history with agencies regarding platform protocol development or other development advice so that all prior regulatory interactions and agreements with the agencies are clearly documented for the review staff. Should note which of the core (asset independent) design elements such as endpoints, definitions etc. have already been agreed.		
	2.7 Feedback received from networks, experts and patient groups	Both disease-specific and molecular target-focused platforms should summarize any advice granted by them and the Company should summarize advice received outside of the platform trial.		

Waivers and Deferrals

Sections of iPSP	Sections of PIP	Contributing Party		
		Platform Trial Team		Company
		Disease-specific	Target focused	
4. Planned Request for Drug Specific Waiver	3. Application for Waivers (being issued based on the PIP condition)	If a waiver is standardized (i.e. a specific age category or disease subset) across the platform.		
5. Plan to Request Deferral of Pediatric Studies 11. Timeline of the Pediatric Development Plan	5. Timelines and Deferrals	Both disease-specific or molecular target-focused platforms should provide estimated rate of enrollment to aid in study completion timelines.		

Proposed Pediatric Plan

Sections of iPSP	Sections of PIP	Contributing Party		
		Platform Trial Team		Company
		Disease-specific	Target focused	
7. Age-Appropriate Formulation Development	4.1.1 Existing pharmaceutical forms 4.1.2 Proposed pharmaceutical forms for pediatric use 4.1.3 Justification of qualitative and quantitative composition			
6. Tabular Summary of planned non-clinical development 8. Nonclinical Studies	4.2 Non-clinical aspects			

Proposed Pediatric Plan (continued)

Sections of iPSP	Sections of PIP	Contributing Party		
		Platform Trial Team		Company
		Disease-specific	Target focused	
6. Tabular summary of planned clinical studies	4.3.2 Graphic Overview of milestones and timelines 4.3.4 Proposed clinical studies			Company should document any other company-sponsored studies in the development plan (outside of the platform) as applicable
9. Clinical Data to Support Design and/or Initiation of Studies in Pediatric Patients	4.3.1 Existing Clinical Data and planned studies	Both disease-specific and molecular target-focused platforms could contribute an overview of same or similar in class agents and basis for the platform study		
3. Overview of Planned Extrapolation of Effectiveness to Specific Pediatric Populations	2.2 Summary of differences/ similarities in the condition between populations (e.g. adult vs paediatric) 4.3.2 Summary of overall strategy and extrapolation plan 4.3.3 modelling and simulation analyses supporting pediatric development	Both disease-specific and molecular target-focused platforms may contribute to the justification of extrapolation (when applicable)		

Proposed Pediatric Plan (continued)

Sections of iPSP	Sections of PIP	Contributing Party		
		Platform Trial Team		Company
		Disease-specific	Target focused	
10. Planned Pediatric Clinical Studies	4.3.4 Proposed clinical studies 4.4 Other studies/ considerations for planned long-term follow-up			Company should document any other company-sponsored studies in the development plan (outside of the platform) as applicable
10.1 Pediatric Pharmacokinetic Studies	4.3.3 Strategy for pediatric dose selection and PK/PD evaluation	Whenever PK studies are included in the platform trial		

References

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