Paediatric Clinical Research Infrastructure Network PedCRIN

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Grant Agreement # 731046

Deliverable D1.3
Advisory Board Report #2

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Working Package: WP1 (ECRIN)

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1. Executive summary

The current deliverable is the second report on the activities of the PedCRIN project's External Expert Advisory Board (EEAB). The members of the EEAB were contacted either through physical meetings or via email. All the activities carried out with the PedCRIN EEAB fall under WP1-Project management, which is led by ECRIN. The first EEAB deliverable 1.2 summarized the selection of the EEAB members and any feedback acquired from them from the beginning of the project until M12 (27th February 2018). The current deliverable summarizes the PedCRIN activities in which the EEAB members participated and the meetings organized with them, along with their recommendations to the project consortium for the period M13 (March 2018) until M28 (27th May 2019).

As regards the structure of the current document, a short introduction on the role of the EEAB, the activities performed during the second half of the project, and the members of the PedCRIN EEAB are presented. The activities carried out during the second half of the project and finally, some of the most important recommendations of the experts to the PedCRIN partners are summarized.

2. Introduction

Besides the communication between the PedCRIN partners, it was very important throughout the whole duration of the project to convey ideas, research and outcomes of the project to relevant stakeholders, to receive their feedback, and make sure that the final outcomes of the project were aligned with the vision of the PedCRIN users. In order to achieve that, an External Expert Advisory Board (EEAB) was established early in the project to review the project activities and outcomes, identify the strong and weak points with respect to the objectives of the project, and provide recommendations during all the project's phases.

The procedure followed for the selection of the experts who were invited and joined the PedCRIN EEAB has been described in deliverable D1.2 (1st Report of Advisory Board meetings (27 February 2018), accompanied with the role of these experts in the PedCRIN project. The member list of the EEAB has remained unchanged since then, as the experts involved from the beginning of the project were capable to cover all the needs emerged.

The PedCRIN EEAB members are shortly presented below along with their updated expertise:

Pr Thierry Lacaze	Neonatologist and the section head of neonatology at the Cumming School of Medicine. And the regional program director of Neonatology at Alberta Health Services.
Prof. dr. C.B. (Kit C.B.) Roes	Leads the methodology group UMC Utrecht Clinical Trial Center.
Pr Régis Hankard	Professor of Pediatrics at the University François Rabelais in Tours and the coordinator of PedStart-CIC.

A short description of their qualifications and position can also be found on the PedCRIN website: https://www.ecrin.org/pedcrin-advisory-board

3. Activities of the PedCRIN Advisory Board

Throughout the whole duration of the PedCRIN project, the consortium has kept a continuous and fluent communication with the members of the EEAB. It should be noted that for the 1st EEAB meeting focused on the refinement of the PedCRIN Project. Whereas the 2nd EEAB meeting provided a careful oversight regarding the development of the tools for neonatal and the paediatric clinical trials, the progress of the PedCRIN funded pilot trials (WE study, OTBB3 & POPART) and on the distinction between the infrastructure supporting the management of paediatric multinational trials (PedCRIN), the project supporting investigation in paediatric trials IMI-C4C(C4C (Collaborative Network for

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European Clinical Trials For Children) and the scientific communities and sponsors acting as users of these projects (paediatricians, rare disease community, pharma industry, biotechnology SMEs).

4. Meeting Overview

The scope of the PedCRIN Advisory Board meeting was to update the board members about PedCRIN project and the challenges the consortium faces, and to offer the PedCRIN consortium the opportunity to draw some initial crucial recommendations for the project's implementation. The Second Advisory Board meeting was held in ECRIN Paris on 27th May 2019. During the meeting the following points were taken in to consideration:

- Provide strategic input to the project
- The development of interoperable tools for the management of paediatric trials
- The optimal use of competences and resources
- The expansion strategy and criteria
- The funding opportunities for multinational paediatric trials
- Rare diseases and paediatric trials
- Infrastructure versus investigation network distribution of roles
- Establishment of international partnerships

Leaders and co-leaders of each work package (WP1-5) provided an update to the advisory board members about their activities (PedCRIN funded pilot trials, the sustainability, strategy, governance & business plan, development of tool for the multinational neonatal and paediatric clinical trials and communication and dissemination of the project. Following the update by the WPs several interesting and useful recommendations were provided by the PedCRIN Advisory Board members.

5. Advisory Board Recommendations

General discussion and recommendations by the Advisory Board focused on the following topics:

- Tools for paediatric trials
- Patient involvement
- Communication with patients and parents
- Communication with paediatric community
- Funding for paediatric trials
- Sustainability of the Infrastructure
- Rare diseases and paediatric trials
- International cooperation

Recommendations by the Advisory Board

Monday May 27, 2019

Pr. Thierry Lacaze, Pr Régis Hankard, Prof dr Kit CB Roes (excused)

Brief summary

During the meeting, the key progress of the PedCRIN project was presented, including development of tools, progress of the three pilot trials, patient involvement and communication plan and outcomes achieved during the second year of the project. Short presentations were given by partners of the Consortium for each topic, leaving space at the end of each presentation to EAB members for discussing results and collect their comments and recommendations. The key recommendations are given below.

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Recommendations

Tools for paediatric trial design

Advisory board would like to congratulate the PedCRIN WP3 team for its achievements and for developing outstanding set of the tools for the paediatric clinical research. The work done for tool development is impressive particularly for neonates. After the end of the PedCRIN project, it is critical to ensure these tools remain available for further projects and adapted to future standards in paediatric clinical research.

Pilot Trials

- In terms of recruitment POPART recruited 93/250 in 4/6 countries only, WE study 40/96 (Poland and France (Nice), 1/0) and OTBB3 trial no inclusion, formulation to be finalized.
- Complex relationships between investigators and sponsors may have been a limiting factor accounting for delayed start date and slow recruitment.
- To continue supporting OTBB3 is questionable. As the OTBB3 trial is not progressing therefore, it is recommended to allocate funds to support the other two running projects and accelerate recruitment by opening more sites if possible or alternatively by offering a study nurse support to the PIs.
- The lessons learnt within PedCRIN funded pilot trials are of prime importance and should be taken into account in current and future European initiatives. Nevertheless, challenges that were encountered in setting up these trials can be used as valuable lessons in the future. PedCRIN must develop a plan for describing lessons learnt from the pilot trials and how these issues and challenges can improve the management (regulatory submissions, monitoring, pharmacovigilance etc.…) of the future paediatric trials. PedCRIN can also recommend the benefits, strengths and weakness of such type of funding calls.

Patient and parents involvement

- Comparison of YPAG feedback with those of patients groups would be interesting
- Building a patient and parent database for involving in other trials is recommended

Sustainability

- There is a need to establish a PedCRIN sustainability board and later to plan a meeting by inviting the government representatives along with other stakeholders (organisations, EU paediatric society, ERNs, European hospital children organisation). It is very important to know what government representatives think about the series of projects initiated in parallel (c4c, EPTRI, EJPRD/ERNs) and to see whether it is acceptable for them to or not later to support more than one infrastructure.
- Many fields are in common with other projects such as c4c. Interface between all these EU projects must be organized in order “not to reinvent the wheel”, speed up the processes and valorize work that already had been done. For instance, the pharmacovigilance “POV” in c4c project on neonate may benefit from presented work as well as issues regarding pharmacovigilance.

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Annex1: Agenda of the PedCRIN Advisory Board 2019

PedCRIN Advisory Board Face-to-face Meeting
Paris 27th May 2019

Introduction
The meeting aims to discuss: • Provide strategic input to the project • The development of interoperable tools for the management of paediatric trials • The optimal use of competences and resources • The expansion strategy and criteria • The funding opportunities for multinational paediatric trials • Rare diseases and paediatric trials • Infrastructure versus investigation network distribution of roles • Establishment of international partnerships

Meeting title:	Date(s)/time:	Location(s):
PedCRIN Advisory Board Face-to-face Meeting	27th May 2019	ECRIN Offices Salle de Conseil 5 rue Watt, Paris, 75013

PedCRIN Advisory Board Face-to-face meeting agenda: 27 May 2019

Time:	Description:	Who:
10h30-11h00	**Arrival/Coffee**	
11h00-11h20	**WP1: PedCRIN Project overview**	Prof Jacques Demotes
11h20-11h40	**WP3: Tools for paediatric trials**	Donato Bonifazi Adriana Ceci Evelyne Jacqz-Aigrain
11h40-12h00	**WP4: Pilot trials**	Saskia De Wildt Lepola Pirkko Christine Kubiak
12h00-12h20	**WP2: Sustainability, strategy, governance, business plan**	Mark Turner
12h20-12h40	**WP5: Communication, dissemination, empowerment**	Joana Claverol Torres Eric Vermeulen
12h40-13h40	**Lunch**	
13h40-16h50	**General discussion and recommendations by the Advisory Board** Tools for paediatric trials Trial design and methodology Patient involvement Communication with patients and parents Communication with paediatric community Funding for paediatric trials Sustainability Infrastructure versus investigation network Rare diseases and paediatric trials International cooperation	Pr. Thierry Lacaze Prof dr Kit CB Roes Pr Régis Hankard
16h50-17h00	**Any other business**	

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Annex 2 : List of the PedCRIN Advisory Board participants

Project Title:	PedCRIN(Paediatric Clinical Research Infrastructure Network)-Grant Agreement # 731046
Work Package:	Advisory Board Meeting 2019
Name of the organising Project Partner Organisation:	ECRIN

LIST OF PARTICIPANTS

PedCRIN WP1 Advisory Board Face-to-face Meeting 27th May 2019

Salle du Conseil Ecrin-Eric Office Paris BioPark, 5 Rue Watt, Paris, 75013

Salle du Conseil (11h00-17h00)

	Board Members	Organisation	Available
1	Pr Régis Hankard	INSERM	Yes
2	Pr. Thierry Lacaze	Alberta Health Service (AHS)	Yes
3	Prof Dr Kit CB Roes	University Medical Center Utrecht	Excused
4	Mark Tuner	University Liverpool (WP2)	Gotomeeting
5	Sabah Attar	University Liverpool (WP2)	Gotomeeting
6	Donato Bonifazi	CVBF (WP3)	Yes
7	Cristina Manfredi	CVBF (WP3)	Yes
8	Tessa VanderGeest	Radboudumc (WP4)	Yes
9	Saskia deWildt	Radboudumc (WP4)	Gotomeeting
10	Janna Kallio	HUSFI (WP4)	Gotomeeting

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11	Pirkko Lepola	HUSFI (WP4)	Gotomeeting
12	Evelyne Jacqz-Aigrain	INSERM (WP3)	Excused
13	Valery Elie	INSERM (WP3)	Yes
14	Beate Aurich	INSERM (WP3)	Yes
15	Joana Claverol Torres	FSJD (WP5)	Gotomeeting
16	Begonya Nafria Escalera	FSJD (WP5)	Gotomeeting
17	Eric Vermeulen	VSOP (WP5)	Yes
18	Jacques Demotes	ECRIN (WP1)	Yes
19	Christine Kubiak	ECRIN (WP1)	Yes
20	Salma Malik	ECRIN (WP1)	Yes

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