



## **Paediatric Clinical Research Infrastructure Network PedCRIN**

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### **Deliverable D1.2 Advisory Board Report #1**

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Working Package: WP1 (ECRIN)

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## 1. Executive summary

The Advisory Board has an important role within the PEDCRIN project and its external, independent analysis and recommendations will ensure the quality and excellence of the project and will support the strategic decisions.

The Advisory Board is composed of 3 experts in neonatal and paediatric clinical research and has its first meeting on the 27 February 2018. The main recommendations provided by the Advisory Board concern the development of tools for paediatric research and their applicability, the pilot trials and the expected learnings, the importance of patient and parents involvement in the development of clinical research, the long term sustainability and business model for project and infrastructure development including the need to avoid duplication or overlaps within the projects and infrastructures.

## 2. The mission of the Advisory Board on PedCRIN

The PedCRIN Advisory Board is an independent body, composed of representatives of major stakeholders in paediatrics and clinical research, providing advice and recommendations for the development of the project to ensure its high quality and excellence.

The Advisory Board will meet once a year and will provide strategic input to the project, particularly regarding the development of interoperable tools for paediatric trials, the optimal use of competences and resources, the expansion strategy and criteria, the access policy, the funding opportunities, and the establishment of international partnerships (Annexe 1).

## 3. The selection process of the PedCRIN Advisory Board

An initial list of experts coming from major stakeholders in global neonatal and paediatric clinical research was established. The experts were selected by PEDCRIN on the basis of proven expertise in neonatal and paediatric clinical research including methodological expertise and good knowledge of the EU research and innovation policy and H2020 programme and availability to fulfil their responsibilities for the duration of the project.

## 4. Members of the PedCRIN Advisory Board

The PedCRIN Advisory Board is now composed of 3 members. Further competencies or expertise may be added during the course of the project, if considered necessary.

A short description of the qualifications of the members is provided below:



**Pr Thierry Lacaze**

Pr. Thierry Lacaze is a Neonatologist, he received his medical degree from the University Paris 5 - René Descartes in 1993 and a PhD in biological sciences at the University Paris 7 - Pierre et Marie Curie in 1995. He completed a fellowship in Neonatology in 1997 and a Master in Epidemiology in 2000. He was appointed professor of Pediatrics at the University Paris 11 in 1997. Thierry moved to Edmonton, Alberta, in 2003 to become the inaugural director of the Women and Children Health Research Institute (WCHRI) in 2006. In 2010, He was recruited as a senior scientist at the Children's Hospital of Eastern Ontario (CHEO) Research Institute and was the scientific director of the Clinical Research Unit at CHEO from 2011 to 2015. Since 2016, Thierry is the section head of Neonatology at the Cumming School of Medicine, and the regional program director of Neonatology at Alberta Health Services. His areas of study include clinical trials with an emphasize on medications. He has led the KidsCAN ([www.kidscantrials.ca](http://www.kidscantrials.ca)) initiative since its inception (2014).

 <p><b>Prof. dr. C.B. (Kit C.B.) Roes</b></p>	<p>Professor of Methods of Clinical Trials, Julius Centrum, UMC Utrecht</p> <p>He leads the methodology group as part of UMC Utrecht Clinical Trial Center. His current research focuses on design and analysis of clinical studies, with emphasis on innovative designs and the bridge between clinical trials and "real world evidence". He participates in the Regulatory Science Network Netherlands and Principal Investigator of the Asterix project: Advances in Small Trials for Significant Innovation and eXcellence (<a href="http://www.asterix-fp7.eu">www.asterix-fp7.eu</a>). He is an advisor to the Medicines Evaluation Board. He has more than 15 years of experience in clinical research and drug development and 10 years of experience in quality improvement and process management in various companies and institutions. He was Vice President of Global Clinical Information at the former Organon, and contributed and developed and registered new drugs in various therapeutic areas</p>
 <p><b>Pr Régis Hankard</b></p>	<p>Professor of Pediatrics at the University François Rabelais in Tours.</p> <p>Specialized in clinical research, he coordinated the Pediatric activity of the Inserm Clinical Investigation Centers (CIC) of the Robert Debré University Hospital in Paris (1997-2004), the CHU de Poitiers (2004-2013) and coordinated that of the University Hospital of Tours since 2013. He is conducting a translational research activity at the UMR Inserm 1069 "Nutrition, growth, cancer" laboratory of Professor Stéphane Chevalier on innovation in metabolic and nutritional investigations using stable isotopes of nitrogen and carbon. Since 2010, he has been coordinating annual nutritional assessment campaigns in hospitals, which each year bring together several thousand patients and more than 60 centers in France and abroad (e-PINUT program). He is the coordinating author of the recommendations for the detection of undernutrition in hospitals of the Nutrition Committee of the French Pediatric Society (SFP). Invested in the promotion of training by and research with young paediatricians, he chairs the Inserm-Pediatrics Interface Committee and the Scientific Advisory Board of the SFP (2010-14). Régis has been director of HUGOPEREN since 2017 and coordinates research axis 2 "Nutrition, Metabolism and Endocrinology". He is also the coordinator of PEDSTART-CIC.</p>

## 5. First Advisory board meeting

### 5.1. Agenda

The first Advisory Board meeting was held in ECRIN Paris on 27<sup>th</sup> February 2018. The objective of the meeting was to update the Advisory Board members about the PEDCRIN projects and the challenges the consortium faces through presentations and discussions. The 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. Participants are listed in Annex 3.

## 5.2. Recommendations from the Advisory Board

The extensive discussions during the meeting were well appreciated by the members of the board as well as by the members of the PEDCRIN consortium.

The main recommendations of the Advisory Board are the following:

- Tools for paediatric trial design: ensure harmonisation across areas (medicinal products, medical devices, other intervention trials) and avoid duplication of tools or initiatives
- Pilot trials: clearly define the expected learnings and the data to be collected to benefit the further paediatrics trials and infrastructure
- Patients and parents involvement: ensure that previous experience especially in adults trials are taken into account and explicate the additional challenges raised by paediatrics and diversity in approaches and culture. To optimize learnings parents and patients from different countries should be involved.
- Sustainability and strategic input: develop a business model for sustainability explaining the added value of the infrastructures, avoid duplication and overlap within projects currently running or in development (PEDCRIN, EPTRI, C4C) and ensure clear communication towards the users.

The feedback provided in writing is appended to the current report (Annex 2).

## Annex1: Invitation to PedCRIN Advisory Board

Prof Jacques DEMOTES-MAINARD  
Director General  
ECRIN  
5 rue Watt  
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**Address**  
XXXXX

**Paris | Date**

Dear Sir/Madam,

RE: Invitation to PedCRIN Advisory Board

The Paediatric Clinical Research Infrastructure Network (PedCRIN) brings together the European Clinical Research Infrastructure Network (ECRIN) and the founding partners of the European Paediatric Clinical Trial Research Infrastructure (EPCT-RI) to develop capacity for the management of multinational paediatric clinical trials. PedCRIN is a four-year project funded by the European Union's Horizon 2020 programme, launched on 1 January 2017 (Agreement Number 731046). One of the major tasks of the PedCRIN project is to create an Advisory Board of recognised experts in global paediatric & clinical research

The Advisory Board will be composed of major stakeholders in paediatric clinical research, to provide strategic input to the project, particularly regarding the development of interoperable tools for paediatric trials, the optimal use of competences and resources, the expansion strategy and criteria, the access policy, the funding opportunities, and the establishment of international partnerships.

It will also provide careful oversight on the distinction between the infrastructure supporting the management of multinational trials (ECRIN), the infrastructure supporting investigation in paediatric trials (EPCTRI) and the scientific communities and sponsors acting as users of these infrastructures (paediatricians, rare disease community, pharma industry, biotechnology SMEs). The Advisory Board will play a significant role in supporting international outreach and connections

We invite you to serve on PedCRIN Advisory Board. You have been recommended as a person with respected knowledge and experience, and as one who will make a valuable contribution to this Board.

The Advisory Board will meet once a year to provide strategic input to the project. The first meeting is scheduled for February 2018.

Yours sincerely,

Regards,

Jacques DEMOTES-MAINARD

## Annex 2: Recommendations of the Advisory Board

Tuesday February 27, 2018

Pr. Thierry Lacaze, Prof dr Kit CB Roes

### Brief summary

During the meeting, the key progress of the PedCRIN project was presented, including development of tools, progress of the three pilot trials adopted in the summer of 2017, patient involvement and communication plan and results. The prepared agenda was followed to a large extent, with main discussions with the Advisory Board already taking place during each presentation. The final discussion was devoted to longer term sustainability of the PedCRIN infrastructures, in the context of the changing environment and related large (pediatric) clinical research infrastructure projects. The general progress of the project and the open discussion with all PedCRIN representatives was well appreciated.

The discussions were extensive and covered many topics. The recommendations below are considered key, but are not a complete summary of all suggestions from the team and the Advisory Board that were discussed

### Recommendations

#### *Tools for paediatric trial design*

- Ensure applicability and terminology for tools across drug, medical device and other intervention trials, where appropriate and possible (e.g., adverse event reporting).
- Ensure that web based information tools (on regulatory aspects e.g.), such as COMPAS, are properly aligned/attuned with others – and avoid duplication. Information can be dynamic; it is worthwhile to think about the updating process and a “one source” type of approach (with referring/linking).

#### *Pilot Trials*

- Comprehensively define the expected/targeted learnings for pediatric trials and infrastructure in general, to be achieved by performing the three pilot trials.
- In line with this, develop a prospective protocol for data collection to support these learnings, as well as support proposals what data needs to be collected and monitored in (future) trials to support efficient and timely execution of trials.

#### *Patient and parents involvement*

- Ensure that experience of models for involvement known from adult clinical trials is properly included, and explicate the additional challenges that pediatric trial add.
- Design the interaction groups such:
  - that investigators responsible for developing the protocols are actively involved;
  - That there is diversity/contrast in approaches/culture included to optimize learning, e.g. by having (at least 2) groups for each pilot trial from different countries (6 countries across 3 studies could be covered).

#### *Sustainability*

The (longer term) sustainability of infrastructures is preferably addressed by the different initiatives jointly.

- Sustainability could be made explicit for PedCRIN specifically, by ensuring that achieving the objectives of the PedCRIN project indeed contributes to a sustainable (part of) the infrastructure. It may need a further reflection on these objectives.
- PedCRIN must develop a plan describing how the Pilot projects will inform on issues and barriers; which methodology will be used to collect data and ensure a relevant analysis, which tools will be piloted, particularly when it pertains to patient and families engagement. It may require PedCRIN to invest some of the \$ grant in some extra human resource to ensure that both projects (the trials themselves and the scholar description and understanding of the barriers/as well as the assessment of tools) are run in synergy and not in conflict.

- It needs to be noticed that the total investment (from EU and industry) in infrastructures for pediatric clinical trials for the coming years is (unprecedented) very high. It is thus a reasonable expectation, that following these investments a sustainable system is achieved, that would not (primarily) depend on continued governmental funding of the infrastructures, independent of the trials actually conducted by the infrastructures.
- A substantial part of sustainability would thus have to come from the primary activities (“services”) and infrastructures that remain attractive after completion of the projects. The attractiveness should translate to “willingness to pay” from the primary process of designing and conducting pediatric clinical trials. This should be worked out in a business model, where the primary aim is to develop a model in which the infrastructures are willing and able to be “self” sustainable, i.e. without structural government funding. In developing such a model, it may transpire that the specific circumstances for pediatric clinical research put structural (financial) limits on the extent with which this can be achieved. E.g. a much larger part (compared to adults) of pediatric clinical trials investigates already available medicinal products, with very tight financial margins. These structural limitations could then be quantified and used as basis for additional governmental funding that would still (reasonably) depend on success of the infrastructures.
- Underpinning these considerations, it will be very helpful to explicate the added value of the infrastructures in terms that are relevant for health care policy, e.g. impact of improving health of pediatric patients (with impact on a hopefully long life), increasing speed of new treatments reaching patients, reducing costs of clinical research, reducing waste (i.e., evidence based abandoning ineffective and/o costly treatments), etc.

Prof Régis HANKARD

PedCRIN Advisory Board meeting was an opportunity to learn about PedCRIN. Being a French representative of C4C and PEDSTART (French Paediatric network), I think there is a risk of overlap between the two projects and structures which is not efficient and bring confusion on the role of all participants. One example is the Pharmacovigilance, such a service is also planned in C4C project led by France.

Additionally, these projects (PedCRIN, C4C, and EPTRI) generate complexity therefore, a special consideration should be paid when communicating to potential users, investigators, industry or the public and for this purpose a proper communication plan is pivotal.

The young person advisory groups (YPAGS) across Europe should be involved in the activities of PedCRIN and information, consent and communication should not only to be restricted to participating countries/centers

It would also be interesting to explore the relationship of European paediatric networks with the Canadian and American networks, like a network of the networks with potential shared projects. To strengthen the affiliation further this has already been discussed with T Lacaze and others from Canadian networks.

### Annex 3: Participants in the first Advisory board meeting

#### PedCRIN-Advisory Board

Name	Members (major stakeholders in global paediatric and clinical research)	Contact details	Email	Phone & website
Pr Thierry Lacaze-Masmonteil	University of Calgary & Alberta Children's Hospital Research Institute (ACHRI)	Section Chief, Neonatology, Department of Pediatrics Cumming School of Medicine	<a href="mailto:Thierry.Lacaze@albertahealthservices.ca">Thierry.Lacaze@albertahealthservices.ca</a>	Phone: (403) 944-4638 Website: KidsCAN Clinical Trials for Children
Kit Roes	University Medical Center Utrecht	Professor Clinical Trial Methodology   Julius Center for Health Sciences and Primary Care, Biostatistics and Research Support University Medical Center Utrecht   Room number C01.124   Internal mail no D01.343   P.O. Box 85500   3508 GA UTRECHT	<a href="mailto:K.C.B.Roes@umcutrecht.nl">K.C.B.Roes@umcutrecht.nl</a>	T: +31 88 75 682 95   M: +31 6 2245 74 96   <a href="http://www.umcutrecht.nl">www.umcutrecht.nl</a>
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#### Pediatric Clinical Research Infrastructure Network (PedCRIN)

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