British Journal of Pharmacy

https://doi.org/10.5920/bjpharm.614

British Journal of Pharmacy

www.bjpharm.hud.ac.uk

Proceedings of the APS@FIP Conference 2018

EPTRI – European Paediatric Translational Research Infrastructure. Bridging the gaps of the paediatric excellence medicine

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ARTICLE INFO

Received: 25/05/2018
Accepted: 18/06/2018
Published: 17/04/2019

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KEYWORDS: Research Infrastructure; Translational Research; Age-related Science; Paediatric Pharmacology

SUMMARY

Development of age appropriate medicines for children is one of the major challenges of our century. Historically, research of new paediatric drugs has been neglected due to poor industrial interest and limited public and private investments. The ID-EPTRI project is aimed to bridge the existing gaps in paediatric medicine that stop the progress from early stage drug development phases to be translated into paediatric use of medicines, through a new paediatric Research Infrastructure. To reach this goal, EPTRI has developed and disseminated a survey in order to identify the gaps and map the competences of the excellence of the paediatric research in pan-European countries that will be the potential service providers of the new Research Infrastructure. EPTRI will network all the available competences and technologies useful to the paediatric research, creating an open science space allowing top-level researchers to work together.

INTRODUCTION

Most of the drugs derived by the advancements of the life sciences have been discovered and developed with adult patients in mind. Historically, research of new paediatric drugs has been neglected due to poor industrial interest and limited public and private investments. This has led to off-label and unlicensed use of adult medicines. Nevertheless, it has been well established that children are not small adults, rather they represent a very heterogeneous group due to their continuous growth, maturation of body composition and physiologic and cognitive changes. (Breitkreutz J et al., 2007; Moore, 1998). The development of age appropriate medicines for children requires not only an understanding of their preferences for different formulations, flavours and textures of products but also an understanding of the physical and biochemical differences between children and adults such as pharmacokinetics and pharmacodynamics, potential routes of administration and medicine-related toxicity. (Breitkreutz J et al., 2007; Moore P., 1998; Batchelor et al., 2013). Although there has been significant innovation in life sciences, including in pharmacogenetics, cell therapies, taste masking technologies, etc, these innovative medicines have been developed using adult models (both preclinical and clinical studies) and at the end of the development process only partially have been made available to use in children. So children remain orphans of many therapeutics because of the challenges faced to appropriately study and tailor medicinal and other products to them. EPTRI, building up the new paediatric Research Infrastructure, will create the bridge between the expertise in paediatric research

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and the necessity of the patients in order to fill up these gaps.

MATERIALS AND METHODS

The project ID-EPTRI aims to design the framework of the new Research Infrastructure (RI), by preparing the Conceptual Design Report (CDR) of the future RI, to promote and lead the paediatric–related medicines, expediting the creation/use of innovative technologies for better and safer drugs for children from drug discovery and early development phases to the formulation. To prepare the Conceptual Design Report (CDR), the project will encompass three phases:

- *Context Analysis phase* to estimate the possible services providers and the existing gaps
- *Operational phase* to plan the different components of the new RI, including governance model, strategies for interaction with national Authorities and the existing RIs, the IT-architecture model, services to be provided and a business plan.
- *Feasibility phase* to develop virtual exercises simulating the operations of the RI to work as a "one-stop-shop" for advice in paediatric drug development.

To this aim, in this first phase of the context analysis, a survey has been set up to map the competences and expertise of the excellence of the paediatric research in pan-European and identify the possible gaps in the available paediatric research services and facilities.

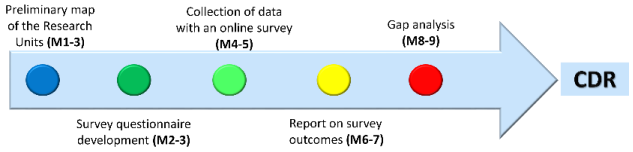

Fig. 1. *Step to create the Conceptual Design Report*

RESULTS AND DISCUSSION

The survey is focused on five main areas: (1) Human development and paediatric basic science. (2) Early translational discovery and paediatric drug development (IT technologies, 3D cell culture, pluripotent stem cell models, etc). (3) Paediatric biomarkers and biosamples (including pharmacogenomics, Biobank organisation, etc). (4) Paediatric pharmacology (including tools/ methodologies evaluating the specific impact of growth and maturation). (5) Paediatric medicines formulation (including formulation design and taste masking technologies). The questionnaire was delivered to about 900 paediatric leading experts in the fields of drug discovery and early development phases (70), paediatric pharmacology (70), biomarkers (100), paediatric formulations (50), paediatric studies (80), etc., identified through a preliminary map of European research units as bibliographic research, colleagues, etc.

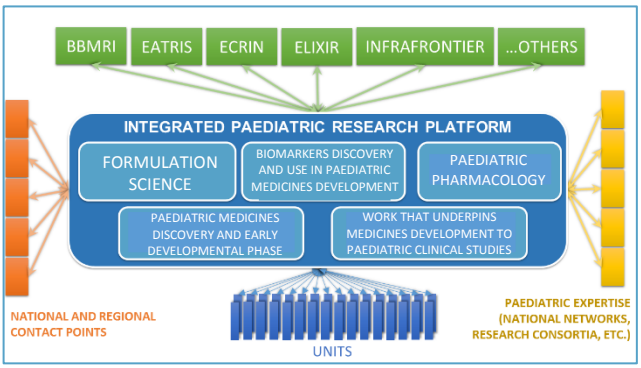

Fig. 2. *EPTRI: five main areas of interest in an integrated Research Infrastructure*

This survey will allow the identification of potential service providers to be included in the future Research Infrastructure and high-end apparatus (equipment, resources, research facilities, platforms, methodologies and experimental settings) which, even if not specifically marked as used in "paediatric research", can offer considerable expertise to a paediatric context.

CONCLUSIONS

EPTRI will built up a new European Research Infrastructure by bridging all the available competences and technologies useful to the paediatric research, creating an open science space, which allows top-level researchers to work together in order to cover the gap in development of key technologies drugs for the paediatric population.

ACKNOWLEDGEMENTS

This Project has received funding from the European Union's Horizon 2020 programme under Grant Agreement No. 777554

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