

EPTRI - European Paediatric Translational Research Infrastructure: accelerating the future of the paediatric research

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on behalf of European Paediatric Translational Research Infrastructure (EPTRI) project

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Introduction

EPTRI arises from the need to find answers to the serious lack of medicines for children in EU and worldwide. It is coordinated by **Consorzio per Valutazioni Biologiche e Farmacologiche (CVBF)** and is aimed to **design the framework** for a **European Paediatric Translational Research Infrastructure** that will cover the technological and scientific gaps in paediatric preclinical and translational research.

The EPTRI interest in rare diseases (RD) is very high, since RD **affect mainly children** and genetic RD start early in the **prenatal/childhood life**. Nevertheless, children are often **orphan of appropriate and innovative treatments** and many advanced tools, such as pharmacogenomic, pharmacogenetics, cell and animal models, are used mainly for innovative medicines in adults' settings.

Method

With the aim to accelerate the paediatric drugs development process, EPTRI performed a context analysis to scan competencies and available services in the following research areas: **paediatric medicines discovery, biomarkers identification, developmental pharmacology and age tailored formulations**. In addition, an online survey has been conducted with the aim to identify the nature and extent of the needs of the users for services in paediatric drug development. Finally, feasibility studies (FS) were performed as **virtual exercises simulating the future EPTRI operations**.

Conclusions

EPTRI could be able to **foster and direct** the cooperative efforts and the excellence in the development of **innovative treatments** for rare diseases which affect the paediatric population, promoting the adoption of methods, and technologies **tailored on children diseases**. In addition by accelerating discovery of new drugs and valid preclinical research, EPTRI will also **accelerate the clinical phase and the paediatric medicinal use**.

Results

The EPTRI survey has identified more than 300 paediatric research units in Europe, which have developed **sets of organized research facilities and services** in specific research fields. Of these, around **70%** are involved in the field of rare diseases especially in the field of drug discovery and biomarkers identification. Noticeably **3 on 10 FSs** were dealing with Rare Diseases (**Duchenne muscular dystrophy, beta thalassaemia, congenital lactic acidosis**) demonstrating that EPTRI would be able to work as a **"one-stop-shop"** to **accelerate drug development** in case of paediatric Rare Diseases.

