ITCC-P4 International Workshop

IMPROVING PEDIATRIC ONCOLOGY
DRUG DEVELOPMENT THROUGH
PRECLINICAL RESEARCH

September 27\textsuperscript{th} and 28\textsuperscript{th}, 2018 // Amsterdam, NL

By invitation

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under Grant Agreement No 116064. This Joint Undertaking receives support from the European Union’s Horizon 2020 research and innovation programme and the European Federation of Pharmaceutical Industries and Associations. http://www.imi.europa.eu - http://www.itccp4.eu
OVERVIEW

Cancer remains the leading cause of disease-related death in children. For the ~25% of children who experience relapses of their malignant tumors, usually after very intensive first-line therapy, curative treatment options are scarce.

Over the last few years, regulatory initiatives in the US and Europe have significantly changed the landscape of new oncology drug development for children. However, there is a major need to improve the field to bring effective new therapies to patients more rapidly and better prioritise drugs to be developed in the pediatric population among the numerous anticancer drugs currently developed in adults as well as drugs specifically developed for children.

Preclinical drug testing can contribute to identify promising treatment options that match the molecular and immunology makeup of the patient’s tumor and should be more systematically part of science-driven drug development plans based on the compounds molecular mechanism of action and the biology of pediatric malignancies.

Two preclinical projects have been recently launched to better address preclinical drug testing: the NCI Pediatric Preclinical Testing Consortium (PPTC) in the US and the IMI2 ITCC Paediatric Preclinical Proof of Concept Platform (ITCC-P4) in Europe.

There is a need to define an international scientific consensus on preclinical evaluation to facilitate and improve new anticancer drug development for children and adolescents in the currently changing international regulatory environment.
**GOAL OF THE WORKSHOP**

To make recommendations on the role and place of preclinical evaluation on pediatric tumor models to improve prioritization and effectiveness of drug development for children and adolescents with cancer.

The overarching goal is to streamline and improve the development of new oncology drugs for children and adolescents within the European Pediatric Medicine and the US Race for Children regulations. This workshop will be held as part of the IMI2 ITCC-P4 project.

**THE PRIMARY OBJECTIVE** is to reach an international scientific consensus that will be published in a peer-reviewed journal and will serve as a basis for a guidance to be submitted to competent authorities for qualification.

**REASONS TO PARTICIPATE**

- To update knowledge on ongoing preclinical research initiatives in the field of pediatric oncology
- To discuss improving predictability of preclinical programs
- To contribute to an international consensus on preclinical evaluation in order to streamline new drug development for children and adolescents with cancer

**WHO WILL PARTICIPATE?**

International academic experts and experts from pharmaceutical companies in science, models, preclinical and clinical development as well as regulatory science; members from the regulatory bodies; representatives of parent organisations and advocacy groups.

**HOW TO PARTICIPATE?**

An online questionnaire is available in order to pre-register for the Workshop:

PRELIMINARY PROGRAMME

Day 1: Thursday, 27th September 2018

12:30-1:00 pm Registration
1:00-1:30 pm WELCOME
1:30-6:30 pm SESSION 1—Sharing Science and Data:
  • Introduction and goals
  • Lessons learned from the Pediatric Preclinical Testing Program (PPTP)
  • Lessons learned from Pharma preclinical programs
  • Improving the predictability of preclinical models including:
    • Novel models (organoids)
    • Novel models for Immuno Oncology compounds
    • Novel imaging approaches
    • Molecular characterization
    • Improving methodology
  • Novel approaches and challenges (including the BBB issue) to evaluating compound efficacy in preclinical brain tumor models
  • Summary of Pediatric Preclinical Testing Consortium (PPTC)
  • Summary of ITCC-P4 project
  • Proposal: Target actionability in pediatric cancers

Day 2: Friday, 28th September 2018

8:30-12:30 am SESSION 2—Break out sessions:
  • 4 break out sessions on specific questions and controversial topics
12:30-13:30 pm LUNCH
1:30-4:00 pm SESSION 3—General discussion and consensus
4:00-4:30 pm WRAP UP
4:30 PM END OF THE WORKSHOP

Innovative Therapies for Children with Cancer Paediatric Preclinical Proof-of-concept Platform (ITCC-P4) is a newly formed public-private partnership supported by the European consortium ‘Innovative Medicines Initiative’ (IMI). The consortium with currently 21 partners from 8 countries aims to establish 400 new patient-derived preclinical models of high-risk pediatric solid tumors which will be fully characterized (molecularly, immunologically, pharmacologically and clinically well-annotated) and build a sustainable comprehensive platform to use these models for drug testing. It brings together many of Europe’s most distinguished academic and clinical research institutions, well established Small-to-Medium sized Enterprises (SMEs), members of the European Bio-pharmaceutical Enterprises (EBE) and members of the European Federation of Pharmaceutical Industries and Associations (EFPIA), thus providing a unique setting to improve patient outcomes by introducing new and effective medicines in standard of care of young people still dying of these rare cancers.

www.itccp4.eu
It is possible to book accommodation in Amsterdam during the Workshop. There are two hotels near the Conference Center (KIT Tropical Royal Institute):

- The Amsterdam Tropen Hotel, 4 minutes by foot from the Conference Center, 150€ per night per person ([http://www.amsterdamtropenhotel.com/en](http://www.amsterdamtropenhotel.com/en));
- The Hotel Arena, 6 minutes by foot from the Conference Center, 150€ per night per person ([https://www.hotelarena.nl/en](https://www.hotelarena.nl/en)).

If you want to book a room in a hotel in Amsterdam, please contact the receptions by yourself and indicate you will participate to the ITCC-P4 Workshop.