

Special Issue on Innovative Paediatric Research to Cover the Unmet Therapeutic Needs

CALL FOR PAPERS

Due to the paediatric specific physiology, pharmacotoxicology, and clinical developmental characteristics, medicines for children need to be evaluated through ad hoc paediatric investigational programs, including ad hoc pharmacokinetic/pharmacodynamic (PK/PD), efficacy-safety, postmarketing pharmacovigilance, and observational cost-efficacy/HTA studies.

However, the current methodological approaches to conduct clinical trials result difficult to apply in this population. In particular, large randomized controlled trials commonly required establishing the efficacy and safety of new and old drugs and their pharmaceutical formulations are often unfeasible in children due to the small number of patients to be recruited, and appropriate tools (validated endpoints, appropriate comparators, noninvasive procedures, ad hoc formulations, etc.) are needed.

These difficulties can be overcome by adopting alternative, innovative approaches, for example, Modelling&Simulation (M&S) and extrapolation, as well as by exploring novel methods currently under study in adult trials (adaptive designs and enriched enrolment). These new research methodologies reduce the number of studies requested for approving a paediatric indication and limit the number of children to be enrolled in clinical studies. To date, they represent an interesting tool and the most advanced frontier of the paediatric clinical research.

In this special journal issue, we seek high quality, original research articles and reviews focused on the state-of-the-art employed approaches and the major achievements in the field of drugs and related formulations for children as well as paediatric clinical research methodology. Besides the theoretical investigations, examples of real applications of innovative approaches in developing drugs for children and paediatric clinical studies for specific drugs and age ranges are particularly welcome.

Potential topics include but are not limited to the following:

- ▶ Ethics and regulation in paediatric research
- ▶ Off-label uses in paediatrics to cover therapeutic needs
- ▶ Paediatric pharmacokinetic/pharmacodynamics studies
- ▶ Innovative study designs and statistical approaches (M&S, extrapolation, Bayesian approach, adaptive design, enriched enrolment, etc.)
- ▶ Choice of comparators in paediatric trials
- ▶ Biochemical and genomic biomarkers
- ▶ Impact of innovative methodologies on trial management and regulatory procedures
- ▶ Challenges and progresses in paediatric pharmaceutical formulations
- ▶ Use of innovative pharmaceutical technologies in adults to develop new paediatric drug formulations
- ▶ Experience of paediatric investigation plans including innovative approaches
- ▶ Patients empowerment in the decision-making process of paediatric clinical trials

Authors can submit their manuscripts through the Manuscript Tracking System at <http://mts.hindawi.com/submit/journals/bmri/pediatrics/iprc/>.

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First Round of Reviews

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