Perspectives on rational drug design and therapy for pediatric precision medicine

Aims & Scope:
The pharmacological therapy of children diseases is changing very quickly in the last decades. In this respect, chronic autoimmune and inflammatory diseases are becoming a major issue for the pediatricians. In spite of the availability of new biological drugs, which has allowed to assist to a great improvement in the prognosis and quality of life of many patients, new therapeutic tools are urgently needed. This special issue will discuss innovative approaches for rational drug design and therapy personalization in the pediatric population, potentially leading to precision medicine improving efficacy and reducing adverse effects of therapy, that are particularly significant in chronic diseases. The repositioning of old drugs to treat rare pediatric immune diseases will be discussed, such as lapaquistat for Mevalonate Kinase Disease and antimalarials in interferonopathies. Also severe oncological pediatric diseases could benefit from precision drugs such as kinase inhibitors targeting specific genetic alterations; this approach will be considered. Innovative disease models are needed to lead to improved drug design and innovative therapies, and the promising application of pluripotent induced stem cells will be presented. Finally, pharmacokinetics and pharmacogenomics based on innovative molecular markers such as transcriptomics, and their application to therapy personalization and drug discovery for pediatric inflammatory bowel disease, will be also discussed.

Keywords:
Rational drug design, antimalarials, interferonopathies, TAK-475, leukemia, inflammatory bowel disease, biomarkers.

Subtopics:
- Repositioning drugs for rare immune diseases: hopes and challenges for a precision medicine.
- Repositioning of TAK-475 in Mevalonate Kinase Disease: translating theory into practice.
- Reappraisal of antimalarials in interferonopathies: new perspectives for old drugs.
- Targeting kinase-activating lesions to improve therapy of pediatric acute lymphoblastic leukemia.
- Induced pluripotent stem cells as a model for drug design and therapy personalization of pediatric patients: disease modeling and drug adverse effects prevention.
- Personalization of immunomodulatory and biologic therapies through the monitoring of drug levels in children with inflammatory bowel disease.
- Pharmacotranscriptomic biomarkers in glucocorticoid treatment of pediatric inflammatory bowel disease.
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