Special Issue

Genetic Rare Diseases in Children

Message from the Guest Editor

We invite submissions to this Special Issue, **'Genetic Rare Diseases in Children'**, aimed at advancing translational research and clinical understanding in this field. Genetic rare disorders, while individually uncommon, collectively pose significant challenges in pediatric healthcare and represent a growing area of scientific and therapeutic interest. This Special Issue seeks to bridge the gap between bench and bedside, encouraging contributions that reflect the interdisciplinary nature of this work. **We welcome original research, reviews, case series, case reports, and short communications** that address (but are not limited to) the following topics:

- Novel gene discovery and disease mechanisms;
- Genotype–phenotype correlations;
- Advances in molecular diagnostics and newborn screening;
- Clinical trial design and therapeutic interventions, including gene- and RNA-based therapies;
- Ethical, psychosocial, and health policy considerations in rare disease care;
- Data sharing and collaborative approaches to improve diagnosis and care.

Guest Editor

Dr. Wenying Zhang
Division of Human Genetics, Cincinnati Children's Hospital Medical Center, Cincinnati, OH, USA

Deadline for manuscript submissions

30 October 2025

Children

an Open Access Journal by MDPI

Impact Factor 2.1
CiteScore 3.8
Indexed in PubMed

mdpi.com/si/239702

Children
Editorial Office
MDPI, Grosspeteranlage 5
4052 Basel, Switzerland
Tel: +41 61 683 77 34
children@mdpi.com

mdpi.com/journal/children

Children

an Open Access Journal by MDPI

Impact Factor 2.1
CiteScore 3.8
Indexed in PubMed

mdpi.com/journal/children

MDPI

About the Journal

Message from the Editor-in-Chief

You are invited to contribute a research article or comprehensive review for consideration and publication in *Children* (ISSN 2227-9067). *Children* is an open access journal—research articles, reviews, and other content are published online immediately after acceptance. The scientific community and the general public have unlimited free access to the content as soon as it is published. The journal focuses on sharing clinical, epidemiological, and translational science relevant to children's health. We would be pleased to welcome you as one of our authors.

Editor-in-Chief

Prof. Dr. Paul R. Carney
Departments of Child Health and Neurology, University of Missouri, 400 Keene Street, Columbia, MO 65211, USA

Author Benefits

Open Access

— free for readers, with article processing charges (APC) paid by authors or their institutions.

High Visibility:

indexed within Scopus, SCIE (Web of Science), PubMed, PMC, Embase, and other databases.

Journal Rank:

JCR - Q2 (Pediatrics) / CiteScore - Q2 (Pediatrics, Perinatology and Child Health)