

## Special Issue

# Drug Design and Development for Rare Hematologic Diseases

### Message from the Guest Editors

The last decade has seen an exponential increase in therapeutic options for rare hematologic diseases. The latter encompass benign conditions, including congenital anemias, autoimmune cytopenias, bone marrow failure syndromes, and rare hemostatic disorders, as well as neoplastic ones. Moreover, rare metabolic disorders such as Gaucher disease may involve bone marrow and result in cytopenic phenotype and have gained clinical attention since novel substitutive therapies have been developed. Novel treatment strategies include oral small molecules, intravenous or subcutaneous monoclonal antibodies, small interfering RNAs, gene therapy, and many others. All of these therapies are at different phases of development and will likely be positioned at different disease stages or in specific settings. In this Special Issue of *Pharmaceuticals*, we aim to collect all types of articles dealing with novel drugs under development for rare hematologic conditions. Manuscript focusing on any aspects of efficacy, safety, and management of novel targeted therapies, or on predictors of response, will be welcomed.

---

### Guest Editors

Dr. Bruno Fattizzo

Dr. Irene Motta

Dr. Marco Capecchi

---

### Deadline for manuscript submissions

closed (25 March 2022)



## Pharmaceuticals

---

an Open Access Journal  
by MDPI

---

Impact Factor 4.8  
CiteScore 7.7  
Indexed in PubMed



[mdpi.com/si/93871](https://mdpi.com/si/93871)

*Pharmaceuticals*  
Editorial Office  
MDPI, Grosspeteranlage 5  
4052 Basel, Switzerland  
Tel: +41 61 683 77 34  
[pharmaceuticals@mdpi.com](mailto:pharmaceuticals@mdpi.com)

[mdpi.com/journal/  
pharmaceuticals](https://mdpi.com/journal/pharmaceuticals)





# Pharmaceuticals

---

an Open Access Journal  
by MDPI

---

Impact Factor 4.8  
CiteScore 7.7  
Indexed in PubMed



[mdpi.com/journal/  
pharmaceuticals](https://mdpi.com/journal/pharmaceuticals)



## About the Journal

### Message from the Editor-in-Chief

Because of your expertise in the field of drug sciences, I kindly invite you to consider publishing your current work, in the form of a research article or a review, in the open access electronic journal *Pharmaceuticals*. *Pharmaceuticals* is characterized by an active editorial board and a dynamic editorial staff. Manuscripts are peer-reviewed and a final decision is provided to authors within 4–6 weeks after submission. Papers are published on the web immediately after acceptance. For details on the submission process or any other matter, please do not hesitate to contact us. We hope to handle your contribution to *Pharmaceuticals* soon.

---

### Editor-in-Chief

Prof. Dr. Amélia Pilar Rauter

Departamento de Química e Bioquímica (DQB) e Centro de Química Estrutural (CQE), Institute of Molecular Sciences, Faculdade de Ciências, Universidade de Lisboa, Lisboa, Portugal

---

### 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, CAPlus / SciFinder, and other databases.

#### Journal Rank:

JCR - Q1 (Pharmacology and Pharmacy) / CiteScore - Q1 (Pharmaceutical Science)