

# Special Issue

## Orphan Drugs

### Message from the Guest Editor

We are all familiar with the great problem of rare diseases and of their treatment and cure. “Orphan viruses”, for example, which are not linked to a recognized disease, such as hepatitis G, as well as genes, receptors, and enzymes, lead to orphan diseases, which imply two separate but related concepts: (a) diseases that are neglected by doctors and (b) diseases that affect a small number of people. Thus, the term “orphan drugs” describes rare medicinal products intended for diagnosis, prevention or treatment of life-threatening or very serious rare diseases or disorders. This Topical Collection, “Orphan Drugs”, will comprise a selection of research papers and reviews offering knowledge on the development of orphan drugs, rare diseases for which research was successful, rare diseases and their treatment with well-known orphan drugs, new entities inserted in clinical applications, and future perspectives. Moreover, in vitro and in vivo studies will provide a possible high impact in the development of orphan-drug-based strategies and in the identification of the key biological regulation processes that remain uncovered.

### Guest Editor

Prof. Dr. Dimitra Hadjipavlou-Litina

Department of Pharmaceutical Chemistry, School of Pharmacy,  
Aristotle University of Thessaloniki, 54124 Thessaloniki, Greece

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Editorial Office  
MDPI, Grosspeteranlage 5  
4052 Basel, Switzerland  
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The International Journal of Molecular Sciences (*IJMS*, ISSN 1422-0067) is an open access journal, which was established in 2000. The journal aims to provide a forum for scholarly research on a range of topics, including biochemistry, molecular and cell biology, molecular biophysics, molecular medicine, and all aspects of molecular research in chemistry. *IJMS* publishes both original research and review articles, and regularly publishes special issues to highlight advances at the cutting edge of research. We invite you to read recent articles published in *IJMS* and consider publishing your next paper with us.

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### Editor-in-Chief

Prof. Dr. Maurizio Battino

Department of Odontostomatologic and Specialized Clinical Sciences,  
Sez-Biochimica, Faculty of Medicine, Università Politecnica delle  
Marche, Via Ranieri 65, 60100 Ancona, Italy

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