



Orphan Drugs

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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.





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Message from the Editor-in-Chief

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