# **Special Issue**

# New Advances in Rare Genetic Disorder

## Message from the Guest Editor

In this issue dedicated to advances in rare diseases, we would like to focus on those that have moved towards translational research. All of us who work in this field know that most rare diseases do not have an effective therapy, and in many of them, only palliative therapies can be applied once symptoms have developed. In this issue, we want to highlight the research carried out in the search for new therapeutic agents, drug replacement, identification of therapeutic targets, gene therapy, cell therapy, and even the development of new diagnostic strategies towards the development of personalized medicine. We hope that this Special Issue will be of interest to you.

#### **Guest Editor**

Dr. Victoria Campuzano

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# Deadline for manuscript submissions

closed (25 September 2023)



# International Journal of Molecular Sciences

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# Message from the Editor-in-Chief

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### **Editor-in-Chief**

#### Prof. Dr. Maurizio Battino

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