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# **RNA Splicing in Health and Disease**

Guest Editor:

#### Dr. Olatz Villate

Pediatric Oncology Group, Biocruces Bizkaia Health Research Institute, 48903 Barakaldo, Spain

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## Message from the Guest Editor

Large numbers of splicing-related diseases have been documented; however, this number is likely to be substantially underestimated because the effects of mutations on splicing are often not pursued as a primary cause of disease

In fact, hundreds of thousands of DNA variants are detected in massive sequencing projects of genetic disorders, and interestingly, recent estimations have shown that an unexpectedly large fraction of genetic diseases are caused by variants that disrupt the splicing process. Although the classification of variants located within many canonical splice sites is often straightforward due to clear biological consequences, it is important to identify and functionally study the impact on splicing of sequence variants outside the canonical splice sites.

Moreover, the modulation of splicing provides a potent therapeutic approach. In recent years, there has been emergence in RNA therapeutics to directly target specific RNA molecules, but there are still many challenges that arise. This Special Issue focuses on the impact of RNA splicing in health and disease and the new therapies that are emerging to modulate it.













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### Prof. Dr. Selvarangan Ponnazhagan

Department of Pathology, The University of Alabama at Birmingham, 1825 University Blvd, SHEL 814, Birmingham, AL 35294-2182, USA

## Message from the Editor-in-Chief

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