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Mucopolysaccharidoses: Diagnosis, Treatment, and Management

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Deadline for manuscript submissions:
closed (30 June 2020)

Message from the Guest Editors

The development of therapeutic options for mucopolysaccharidoses (MPS), including hematopoietic stem cell transplantation (HSCT) and enzyme replacement therapy (ERT), has modified the natural history of many MPS types. In spite of the improvement in some tissues and organs, significant challenges remain unsolved. Newer approaches may provide a better outcome for MPS in the near future.

Therapies should start at a very early stage prior to irreversible bone lesion, and damage due to the severity of CNS involvement and skeletal dysplasia is associated with level of activity during daily life. As early diagnosis and early treatment are imperative to improve therapeutic efficacy, the inclusion of MPS in newborn screening programs should reduce the morbidity associated with MPS diseases. Additionally, we will provide insights into primary storage materials on GAGs ("GAGnomics").

In this Special Issue, we will summarize diagnosis, treatment, and management of MPS and will evaluate available and future treatments, and will describe their advantages and disadvantages. We will also assess the current clinical endpoints and biomarkers used in clinical trials.

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Editor-in-Chief

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Message from the Editor-in-Chief

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