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Development of AAV-Based Gene Therapies: Unmet Needs and Solutions

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Message from the Guest Editors

Until recently, there were no treatment options for inherited diseases. This has changed with the availability of effective and safe vectors for gene delivery, making gene therapy a valid option for the treatment of inherited diseases. Some of the most promising treatment approaches are gene therapies based on recombinant adeno-associated viruses (AAV). AAV vectors can efficiently deliver genes into somatic cells and lead to the long-term expression of the gene of interest. In addition, they have a favorable safety profile with only low to moderate immunogenicity that is clearly dose-dependent. However, AAV vectors have some limitations that restrict their broad applicability. Due to limited tropism, not all tissues and cells can be reached with existing AAV variants. In addition, there are some diseases caused by mutations in large genes or genes with autosomal dominant inheritance patterns. Further limitations arise from the limited availability of suitable preclinical models that would allow rapid and reliable testing of gene therapy candidates.

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Message from the Editor-in-Chief

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