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Special Issue Reprint

Gene Therapy for Rare Diseases

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A rare disease is a condition that affects only a small part of the population. Although each single disease is rare, the total number of patients suffering from a rare disease is high. Importantly, nearly all rare diseases lack an approved treatment, making the development of novel therapies for rare diseases a major strategic research goal. Developing such therapies for rare diseases, however, is challenging. Most rare diseases have a genetic background and gene therapy is the only curative option.

This Special Issue on “Gene Therapy of Rare Disease” collects several reviews and one original article on currently ongoing research trying to make gene therapy a more widely used treatment option in the clinic. In addition to reviews on mechanistic aspects of rare disease treatment by gene replacement and gene correction, the current state of gene therapies for Diamond-Blackfan anemia and lysosomal storage disorders is described.

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