Tentative Outline

Special/Thematic Issue for the journal "Current Gene Therapy - CGT"

Tentative Title: Gene Therapy for rare genetic diseases

Guest Editor: Fawzy Ali Saad

Scope of the Thematic Issue:

Presently, there are 25 registered clinical trials using CRISPR/Cas gene editing technology for gene therapy, only 4 of them targeting rare diseases. Therefore, the time has come to spot the light on the horizon of gene therapy for rare genetic diseases. The editorial article highlights the new horizon of rare genetic diseases therapy and its future after the invention of CRISPR prime gene editing - search and replace, which corrects about 89% of genetic diseases causing mutations. The other three articles provide insights into cargo delivery vectors for gene therapy, gene therapy for Duchenne muscular dystrophy, and β-Thalassemia

Keywords: Rare Genetic diseases, Gene Therapy, Cargo delivery, Duchenne Muscular Dystrophy, β-Thalassemia.

Sub-topics:

The sub-topics to be covered within the issue should be provided:

1. Vehicles for delivery of gene therapy cargos

2. Rare genetic diseases gene therapy

Schedule

Complete Thematic issue submission deadline: **December 20, 2022**

Details of Guest Editors:

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