

# Tentative Outline

## Special Semi-Thematic Issue for Current Gene Therapy Issue (CGT)

**Title of the Mini-Thematic Issue:** “Gene Therapy for rare genetic diseases”

**Guest Editor:** Fawzy Ali Saad

**Scope of the Mini-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 shed 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  $\beta$ -Thalassemia

**Keywords:** Rare Genetic diseases, Gene Therapy, Cargo delivery, Duchenne Muscular Dystrophy,  $\beta$ -Thalassemia.

### Subtopics

The sub-topics to be covered within the issue:

- Vehicles for delivery of gene therapy cargos
- Rare genetic diseases gene therapy

### Tentative titles of the articles

Tentative titles of the articles and list of contributors with their names, designations, addresses and email addresses.

1. Editorial: New horizons for rare genetic diseases gene therapy.
2. Viral Vector Delivery of Gene Therapy Cargos.
3. Gene therapy for Duchenne Muscular Dystrophy
4. Precision gene therapy of beta-Thalassemia: correcting the beta-globin gene mutations by
5. CRISPR/Cas9 based gene editing.

### Schedule

Manuscript submission deadline: 04/30/2022

Peer Review Due: 05/30/2022

Revision Due: 06/30/2022

Announcement of acceptance by the Guest Editors: 07/20/2022

Final manuscripts due: 08/20/2022

### Contact

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