Special Issue

CRISPR-Engineered Mouse Models for Unraveling Rare Diseases

Message from the Guest Editor

The study of rare human diseases, often caused by subtle single-gene mutations, presents significant challenges due to low patient prevalence and complex, poorly understood pathophysiology. Genetically engineered mouse models, particularly those generated using the CRISPR/Cas9 system, have become an indispensable and powerful tool for mimicking the genetic and clinical hallmarks of these conditions. The precision of CRISPR/Cas9 enables the faithful recapitulation of specific patient mutations (e.g., knock-in point mutations) and the rapid generation of relevant mouse models called "avatars".

This Special Issue invites contributions that showcase the cutting-edge application of CRISPR/Cas9-derived mouse models to provide deep mechanistic insights into human rare diseases. The primary focus is on how these sophisticated models are accelerating understanding of disease mechanisms, identifying novel therapeutic targets (e.g., gene therapy and pharmacological interventions), and performing rigorous preclinical testing.

Both original research articles and reviews are welcome, and all rare human diseases may be included.

Guest Editor

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