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Balancing On Target and Off Target Delivery during Systemic Gene Therapy

Guest Editor:

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Deadline for manuscript submissions:
closed (15 August 2020)

Message from the Guest Editor

Dear Colleagues,

Some genetic diseases affect only certain tissues, whereas others affect every cell in the body. For many of these diseases, intravenous delivery is favored to reach distant diseased tissues. While systemic delivery has some ability to do this, it also exposes many more off-target cells and tissues to the therapy. This increases the risk of toxic and immunologic side effects. Efficacy is frequently examined in gene therapy. Side-effects and toxicity are frequently ignored.

This Issue focuses on strategies to target vectors to the cells in need of therapy while also detargeting them from off-target sites. These strategies may include screening different vectors or capsids, modifying vectors with cell-targeting ligands, and post-entry strategies to control expression of transgenes in on-target tissues. Articles are encouraged that successfully track gene delivery by measuring transgene expression, but that also track where all of the failed vectors go by tracking vector genomes. Articles that examine vector efficacy in combination with measurements of gene therapy side effects are most encouraged.

Prof. Michael A. Barry
Guest Editor

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Message from the Editor-in-Chief

Genes are central to our understanding of biology, and modern advances such as genomics and genome editing have maintained genetics as a vibrant, diverse and fastmoving field. There is a need for good quality, open access journals in this area, and the *Genes* team aims to provide expert manuscript handling, serious peer review, and rapid publication across the whole discipline of genetics. Starting in 2010, the journal is now well established and recognised.

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