Cellular Immunology 342 (2019) 103945

Contents lists available at ScienceDirect

Cellular Immunology

journal homepage: www.elsevier.com/locate/ycimm

Editorial

Balancing immunity and tolerance in gene therapy for inherited and acquired diseases

With several successful gene therapy clinical trials and the first Food and Drug Administration and European Medicine Agency approvals of gene therapy products, the promise of a permanent cure for numerous genetic diseases is becoming a reality. Part of the success of the field of gene therapy is due to several technical advances in both vector design and manufacturing scale, as well as the quality of this class of advanced therapeutics. Yet, like for other complex biological therapeutics, as development of gene-based therapeutic approaches progressed from early proof-of-concept studies to the clinic, the understanding of the interactions between the gene therapy product and the host immune system has been instrumental to enhance safety and efficacy of gene therapy in humans.

This special issue of *Cellular Immunology* is focused on some of the key aspects of the immunology of gene therapy, concerning both the adeno-associated virus (AAV) and lentiviral vector platforms. Both vector- and transgene-directed immune responses are discussed, addressing both complexities related to pre-existing immunity in humans, animal models suitable to perform immune studies, and strategies to overcome immune-mediated toxicities in gene transfer. Importantly, as we gain more confidence on the technical platforms for gene transfer, novel therapeutic horizons will open. These include the ability to exploit liver gene transfer to establish immunological tolerance to tolerize against immunogenic protein therapeutics or to eradicate established autoimmunity.

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https://doi.org/10.1016/j.cellimm.2019.103945

Available online 14 June 2019