

Special Issue

New Frontiers in Molecular Hematology

Message from the Guest Editor

The severe complications that still exist on hematopoietic stem cell transplants in the absence of a compatible donor led us, 20 years ago, to explore another therapeutic strategy for genetic diseases of the hematopoietic system. The key idea behind the treatment of these inherited diseases is simple: The diseased bone marrow is replaced with autologous gene-modified cells, in order to establish a stable expression of the missing protein and thus restore its function. Several inherited immune deficiencies and blood disorders can be cured with the use of a lentiviral vector for people affected by immune deficiencies worldwide. Our aim is to bring this powerful innovation to all affected patients.

Guest Editor

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Deadline for manuscript submissions

closed (15 December 2020)



Journal of Clinical Medicine

an Open Access Journal
by MDPI

Impact Factor 2.9
CiteScore 5.2
Indexed in PubMed



mdpi.com/si/38386

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