A Universal Approach to Target Various HBB Gene Mutations in Hematopoietic Stem/Progenitor Cells for Beta-Thalassemia Gene Therapy by CRISPR/Cas9 and the rAAV6 Vector

Research

Keywords:

Posted Date: December 23rd, 2020

DOI: <https://doi.org/10.21203/rs.3.rs-56828/v2>

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Abstract

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