TITLE: LENTIVIRAL VECTORS AND THEIR USES

FIELD OF INTEREST

Biotechnology (gene therapy, Diamond-Blackfan anemia).

CLINICAL NEED

Diamond-Blackfan anemia (DBA) is a rare disease with an estimated prevalence of 5-7 cases per million live births. The hallmark of DBA is macrocytic anemia, which usually presents in the first year and often progresses to neutropenia and thrombocytopenia, and in some cases to myelodysplastic syndrome or acute myeloid leukemia. Mutations in 20 DBA genes, plus 3 "DBA-like" genes, account for 70-80% of patients with DBA. RPS19, the gene that codes for the ribosomal protein S19, is the most commonly affected in DBA.

Corticosteroids are the first therapeutic option for patients with ABD. Approximately 80% of patients respond initially to corticosteroids with an improvement or complete remission of their anemia. However, long-term corticosteroid therapy has shown limited efficacy in many patients. To date, allogeneic hematopoietic stem cell transplantation is the only curative treatment available for patients with DBA. There remains a critical need for an effective treatment regimen for DBA.

DESCRIPTION OF THE INVENTION

Researchers propose the use of lentiviral vectors to treat DBA. These lentiviruses are not only capable of restoring the expression of the RPS19 protein, but they are also capable of correcting the alteration in the ribosomal biogenesis process.

TECHNOLOGY KEYWORDS

Gene therapy, DBA, RPS19 protein, lentiviral vectors.

IPR STATUS

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Applicants: FIIS-FJD, CIEMAT, CIBER, Université Libre de Bruxelles and Fundación para la Investigación Biomédica del Hospital Infantil Universitario Niño Jesús

TYPE AND ROLE OF PARTNER

Looking for commercial partners interested in licensing.

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