TITLE: IN VIVO LENTIVIRAL GENE THERAPY FOR THE TREATMENT OF PRIMARY HYPEROXALURIA TYPE 1

FIELD OF INTEREST

Biotechnology (lentiviral gene therapy, Primary Hyperoxaluria type 1).

CLINICAL NEED

Primary Hyperoxaluria Type 1 (PH1) is an inherited rare autosomal recessive metabolic disease. It is caused by a deficiency in the alanine:glyoxylate aminotransferase enzyme (AGT). This enzyme is only active intracellularly and is not secreted into the extracellular space. PH1 is characterized by an overproduction of oxalate in the liver. When produced in large amounts, the kidney is the first organ affected due to the aggregates of calcium oxalate (CaOx) that are formed in the urinary space (urolithiasis) and renal tissue (nephrocalcinosis), causing interstitial fibrosis and renal failure. As a result of renal damage, the glomerular filtration rate (GFR) decreases and chronic kidney disease rises. Gene therapy has the potential to be curative with one-time dosing, by restoring AGT activity through delivery of a functional copy of the AGXT gene. AAV vectors are widely adopted for in vivo liver-directed gene therapy, given their remarkable safety and efficacy profile shown in pre-clinical models and clinical trials.

DESCRIPTION OF THE INVENTION

Researchers propose the use of LV based on the vector used for the treatment of Haemophilia B for the treatment of primary hyperoxaluria type 1. These LV are designed to target transgene expression to hepatocytes by a combination of transcriptional and post-transcriptional control. In addition, the proposed LV includes an enhanced version of AGXT that confers a higher stability to the protein.

TECHNOLOGY KEYWORDS

Lentiviral gene therapy, PH1, AGT, hepatocytes.

IPR STATUS

Patent application number: EP21382363.6.

Applicants: FIIS-FJD, CIEMAT and CIBER.

TYPE AND ROLE OF PARTNER

Looking for commercial partners interested in licensing.

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