TITLE: TREATMENT OF EPIDERMOLYSIS BULLOSA

FIELD OF INTEREST

Biotechnology (Gene Editing, Epidermolysis bullosa)

CLINICAL NEED

Epidermolysis Bullosa refers to a group of diseases characterized by strong skin fragility. The recessive dystrophic subtype (RDEB) is the most severe phenotype of the disease.

Many mutations have been described within genes that are implicated in the codification of the protein called type VII collagen, making this type of genes the main target of gene therapy strategies RDEB.

Recently, benefits have been shown in an ex vivo phase I clinical trial in a patient transplanted with skin equivalents, but new gene editing tools open the door to new advanced therapies.

The present invention discloses the most efficient tool for genome editing in primary keratinocytes (a cell type considered hard-to-transfect).

DESCRIPTION OF THE INVENTION

For the first time, a large collection of AAV serotypes have been tested in order to find the highest transduction efficiency in primary keratinocytes. Also, it is possible to develop a large collection of AAVs containing different regions of the genes that are implicates in the collagen synthesis, correcting any mutation along these genes.

Furthermore, the present technology is capable of achieve close to 40% of precise gene correction in primary keratinocytes, surpassing previous HR-bases gene correction ratios.

TECHNOLOGY KEYWORDS

Gene editing, Epidermolysis bullosa, mutations, collagen proteins.

IPR STATUS

Patent application number: EP20382027.9.

Applicants: UC3M, CIEMAT, CIBER, FIIS-FJD.

TYPE AND ROLE OF PARTNER

Looking for commercial partners interested in licensing.

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