Technology Offer

GENE EDITING STRATEGY FOR THE TREATMENT OF DYSTROPHIC EPIDERMOLYSIS BULLOSA

A research group from UC3M, CIEMAT, FJD, and CIBER has used a gene editing strategy to treat a mutation that causes Recessive Dystrophic Epidermolysis Bullosa

The Need

Recessive Dystrophic Epidermolysis Bullosa (RDEB) is a rare and severe inherited skin disorder caused by mutations in the COL7A1 gene, responsible for producing type VII collagen. This leads to extreme skin fragility, blistering, scarring, fibrosis, and a high risk of developing skin cancers. Other organs and tissues can also be involved.

c.6527insC mutation is particularly common in RDEB patients from Spain and Latin America, with an estimated 200 or more individuals currently affected.

Despite growing efforts, there is still no curative treatment available. Existing experimental gene editing approaches have shown limited safety and effectiveness, highlighting the urgent need for a more precise and less toxic therapeutic strategy.

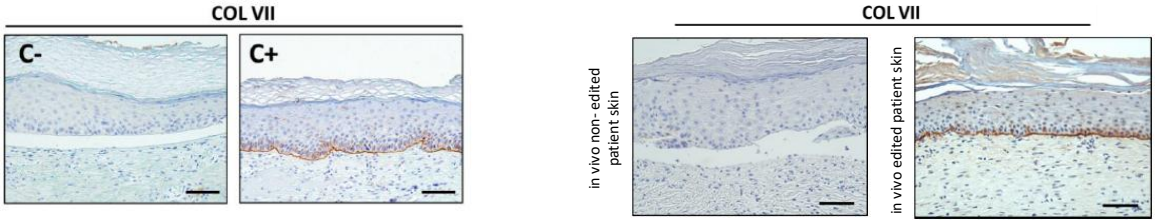
The Solution

This project proposes a new gene editing approach based on prime editing, a recent strategy that allows highly accurate genetic corrections in the COL7A1 gene without causing toxic effects.

Correction efficiencies above 80% have been observed in cell models, representing one of highest percentages reported so far. The method can be applied both ex vivo and in vivo, making it a versatile for treating RDEB. Unlike other strategies, it does not rely on high-cost viral vectors.

Innovative Aspects

- Most advanced and precise gene editing strategy proposed so far for RDEB.
- Safer alternative to traditional methods, minimizing genotoxic risks while maintaining high therapeutic efficiency.
- No gene editing products on the market for RDEB, and other existing experimental alternatives still on development.
- Well-defined target population and relatively large (<200) for a rare disease).
- No need for expensive manufacturing processes, such as GMP-grade viral vectors.



In vivo skin regeneration after transplantation of dermo-epidermal equivalents containing cells corrected by the prime editing strategy with the patented RNAs into immunodeficient mice.

Stage of Development:

Early-stage preclinical development (validated results with patient-derived cells).

Intellectual Property:

- Priority European patent application filed.
- Suitable for international extension (PCT application)

Aims

Looking for a partner interested in a license and/or a collaboration agreement to develop and exploit this asset.

Contact details

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