Development of a life-long anticoagulant therapy by once-and-done gene editing of *F11*

A multidisciplinary consortium from CIBER, UMU, FFIS, CIEMAT and IIS-FJD have developed a new disruptive anticoagulant treatment using gene therapy methods sustained by data of patients with a rare disease.

The Need

Thrombosis is the most common underlying cause of cardiovascular diseases. The contribution of thrombosis to the global burden of disease is expected to increase in the coming years. Unfortunately, the **anticoagulant armamentarium is limited** and has limitations, mainly **bleeding risk**. This and the huge number of patients on anticoagulation (> 100 million/year) have strongly encouraged the **development of new anticoagulant treatments. Anti-FXI** agents are emerging anticoagulants with antithrombotic efficiency and minor bleeding risk, which still have important limitations.

The Solution

The invention consists of ***F11* gene editing** by *in vivo* **delivery of lipid nanoparticles** encapsulating the CRISPR/Cas9 system as a **permanent treatment for the prevention of venous thrombosis** in patients at high risk for thrombotic events that will require life-long anticoagulation.

Innovative Aspects of the New Treatment

- **Single administration** to achieve **life-long anticoagulation**.
- **No adherence or compliance issues**.
- **Stable pharmacodynamic effect**, without significant fluctuations.
- **No** known **interference** from pharmacogenetic factors or concomitant medications.
- **No** routine therapeutic **monitoring** required and **lower** overall treatment **costs**.
- **Favorable benefit–risk profile**: anti-FXI strategies provide comparable or superior anticoagulant efficacy with a lower bleeding risk.
- Potential for **combination** therapy **with other anticoagulan**t strategies without substantially increasing bleeding risk.
- **Reversibility**, with FXI concentrates available as a reversal strategy.

Stage of Development: in mouse and human cell lines)
Pre-clinical assays have been performed *in vitro (*and *in vivo* (mice models). Efficacy, safety and specificity of the administration procedure, showing promising results to accomplish clinical trials.

Intellectual Property:

- Very positive patentability report
- European patent application in 2026

Developmental stage of the project

Aims

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

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