PATENT

Method to produce enzymes

Market sector: nanomedicine, rare diseases

Type of opportunity: licensing and/ or co-development

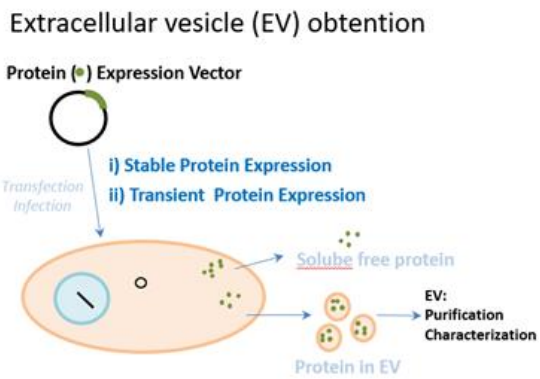
Scope of the problem

The biomedical use of proteins (i.e., enzymes, growth factors, hormones) has been widely explored by the biotechnological industry including enzyme replacement therapy (ERT).
One of the major bottlenecks regarding therapeutic protein treatments, such as those used in ERT to treat lysosomal storage diseases (LSD), it is to achieve a proper biodistribution and biological availability of the protein at the right tissues and organs. Conventional approaches tend to administer high doses of the protein product to obtain the desired therapeutic activity, which often leads to deleterious side effects.
The use of nanotechnology to deliver active proteins might overcome this particular concern by increasing protein stability, reducing clearance and directing the protein to specific cells. Particularly, the use of extracellular vesicles (EVs), as vehicles for biologically active proteins. EVs have been shown to be biocompatible and fully tunable, allowing the production and encapsulation of recombinant proteins inside in a single step.
We have generated specific IPR on the use of EV as protein delivery vehicles and tested several examples such as EVs loaded with the lysosomal enzyme alpha Galactosidase (GLA). EVs-GLA exhibited significantly higher enzymatic activity than the reference enzymatic treatment in vitro and in vivo and proved to be an ideal system for ERT in lysosomal storage diseases.

Patient need addressed: Lysosomal storage diseases (Fabry disease), cancer, infections, immune disorders

Our innovation:

- Innovative method for the synthesis an encapsulation in an **all-in-one process** of a functional version of enzymes with high biological (i.e. enzymatic) activity, vehiculized in EVs for protein based therapies
- The nanodelivery system and the therapeutic compound are produced and isolated at the same time in **a simpler process** than conventional protein purification processes
- The method produces a mixture comprising **proteins with a therapeutic interest and EVs** or fragments of lipid bilayer of these EVs, both EVs and proteins obtained from the same cell.
- In the case of lysosomal enzymes our system **increases the catalytic activity of the enzyme** with respect to the naked enzyme produced in the same cell, fact that translates into a much higher biological activity in preclinical disease models.



Competitive advantages: Increase of the treatment efficacy while reducing the production cost. Potential transference of this system to other LSD such as Sanfilippo, Gaucher, Pompe, Hurler or Hunter, and other protein based therapies. Application to other diseases currently treated with recombinant proteins.

Market size/ opportunity: Global nanomedicine market expected to reach $261,063 Million by 2023 (Allied Market Research, Nov. 2, 2017). Global Protein Therapeutics Devices Market is poised to grow at a CAGR of around 8.6% over the next decade to reach approximately $315.90 billion by 2025 (Protein Therapeutics Market Analysis and Trends - Therapeutic Proteins, Application, Function - Forecast to 2025).

Intellectual property

Priority European patent application filed (January 24, 2019)
Suitable for international extension (PCT application)

Contact: techoffer@ciber-bbn.es www.ciber-bbn.es