TECH PROFILE

Fundación Instituto de Investigación Sanitaria de Santiago de Compostela

Lysoslert

Business area
Discovery

Market sector
Enzyme Replacement Therapies (ERTs), orphan drugs or rare diseases

Medical Indication
Lysosomal Storage Diseases (LSDs)

Research goal

Specialized administration system for the lysosomal release of enzymes used in the enzymatic replacement treatment of lysosomal storage diseases.

Problem to solve

There are only parenteral enzyme solutions to treat a small number of EDLs through ERT. There are still a high number of EDLs without enzymatic treatment. Nowadays of the approximately 70 EDLs that are known, there are only 15 treatments commercialized or in studies clinical The biggest problem associated with these treatments is that due to the high doses and frequency of administration, patients develop antibodies and may suffer anaphylactic reactions that require stop it without other possibilities of enzymatic treatment and therefore producing worse important in their pathologies. In addition, enzyme solutions hardly reach some tissues affected like bone or central nervous system. Any improvement in the access of the enzyme to the tissues without producing anaphylactic reactions or generate antibodies will be a breakthrough for treatment and therefore a great success from the point of commercial view. The availability of a system that improves the distribution to the SNC without having to use the intrathecal or intra-ventricle-cerebral route, it will be a commercial success and will produce evident savings in the treatment by avoiding operations for the placement of intrathecal reservoirs

Innovation

The invention is based on a specialized administration system for the lysosomal release of enzymes used in the enzymatic replacement treatment of lysosomal storage diseases. End users are patients who suffer EDLs diseases of which ERT therapy exists, as well as the national health systems that finance these treatments. The treatment of these diseases is throughout all of life since at the moment there is no curative therapy.

Market opportunity

LYSOSLERT is aimed at the pharmaceutical market for orphan or rare diseases and specifically at Lysosomal storage diseases. The average cost of orphan drugs in Spain is € 32,342.00 per patient (€ 1,251.00-407,631.00 / patient). The average cost of treatment in patients with EDL it is estimated between € 40,000.00 and € 800,000.00 per year. Attending patients with morquio the average costa of treatment it is estimated between 250.000,00 and 650.000,00€ per patient. We also have to mention the Gaucher diseases (ERT with inmiglucerase), Pompe (alglucosidase), Hurler (laronidase), Hunter (Idursulfasa), Fabry (algasidase) and Maroteaux-Lamy with an average cost per patient per year of between € 40,000.00 for this last pathology and € 400,000.00 from the Pompe treatment.

Research team

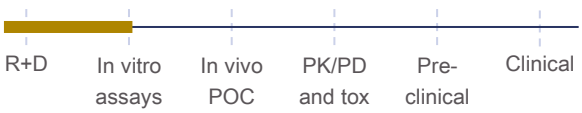
Metabolic Disorders Research Group of the Health Research Institute of Santiago de Compostela; Department of Pharmacy and Pharmaceutical Technology of University of Santiago de Compostela

- **Jose Víctor Álvarez González.**
- **Francisco J. Otero Espinar.**

Intellectual property

Patent Request.
EP18382513 "Nanostructure lipid system"

Development stage:



Available for: *Licensing, co-development*