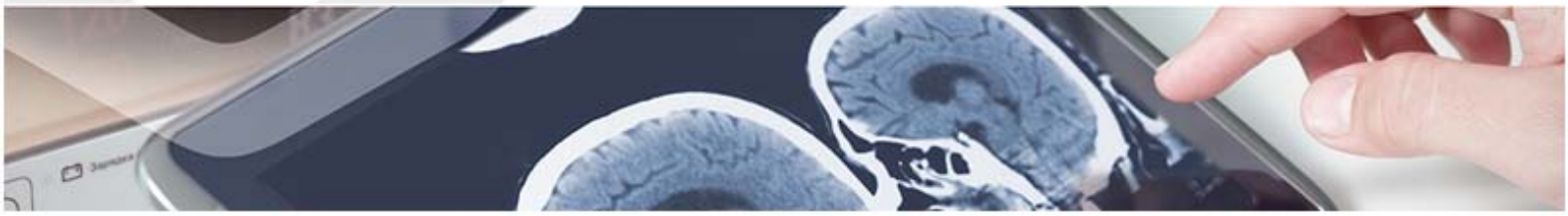
Biotech – Pharma (Therapy)

Gene therapy for treatment of the retinosis pigmentosa

A research group from APHS in collaboration with researchers of SCU and from CSIC has developed a non-viral transfection system for the treatment of Retinosis Pigmentosa.

TECHNOLOGY TRANSFER OFFICE
Andalusian Public Health System



Description

Retinitis pigmentosa (RP) is the most common retinal dystrophy. It is a hereditary disease of the retina characterized by a progressive degeneration of photoreceptors resulting in a visual impairment or complete blindness.

Up to date, 57 genes associated with the RP have been identified. Some of these genes are non-retina-specific and their mutations are responsible for 12% of cases of autosomal dominant retinitis pigmentosa (adRP).

There are currently no effective treatments to prevent or reverse vision loss. However, many advances have been made to slow down the degeneration of photoreceptors and improving their function using neuroprotective factors, antioxidants, drugs, stem cells or neural progenitors.

In recent years, gene therapy has become into a promising therapeutic alternative. Thus, it has been recently used to restore vision in Leber Congenital Amaurosis patients, through the replacement of defect genes by functional genes using viral vectors. This new strategy opens up the possibility of restoring vision in other patients, where photoreceptors degenerate due to the mutation of specific genes.

A research group from Andalusian Public Health System (APHS) in collaboration with researches of University of Santiago de Compostela and from Consejo Superior de Investigaciones Científicas (CSIC) has shown by *in vivo* testing a statistically significant improvement and a therapeutic effect on the sharpness and spatial vision, as well as the thickness, of the retina of RP animal models where the causative gene for adRP is administered into the sub-retinal space.

For the administration of this gene, the research group has developed a non-viral transfection system based on span that has shown a high transfection level. The treatment of the animal models with the nanoparticles functionalized with the mentioned gene plasmid allows to avoid the retina degeneration and to improve the visual function and retina thickness after one month of the treatment.

Therefore, these tests constitute an *in vivo* proof-of-concept about the clinical efficacy of the developed nanomedicines for RP treatment.

Advantanges

The non-viral transfection system developed has the following advantages:

- A very simple one-step, mild, cheap, fast and easy scalable technique which allows to modulate the physic-chemical characteristics of the developed nanoparticles by incorporating additional components which therefore render them a high versatility.
- High transfection level.
- The developed nanoparticles showed as main features a high stability and an appropriate toxicity profile in terms of cell viability, as well as in terms of biocompatibility and absence of deleterious effects at retinal level.

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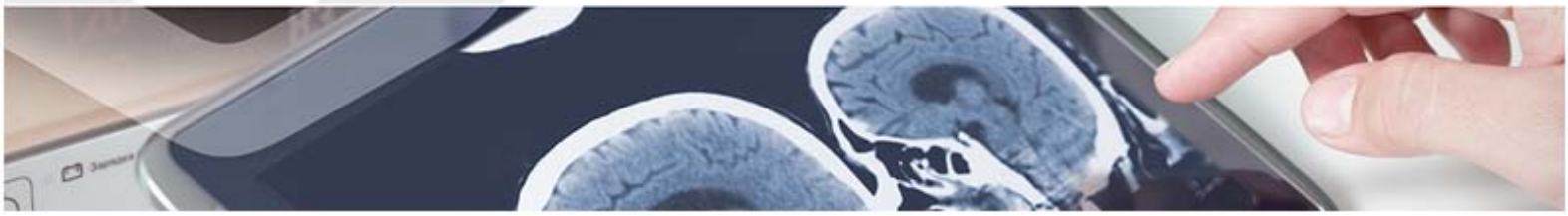

Intellectual Property

The technology is protected by a PCT patent application with the possibility of international extension.

Aims

The research group is looking for a collaboration agreement for further development or a licence agreement.

Classification

Area: Biotech - Pharma
Technology: Gene Therapy
Pathology: Ophthalmology and optometry

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