

SUCCESS STORY

AAVEYE/Gene Therapy for Inherited Severe

Photoreceptor Diseases

Research area: FP7 – Cooperation / HEALTH – Gene therapy tools targeting the central nervous system

Number of partners: 4 among which the Foundation Asylum for the Blind

Start date – End date: 2008-11-01 to 2011-10-31

Duration: 36 months

Funding: € 2 971 000 / FAA: € 776 400

Type of contract: Small or medium-scale focused research project



YVAN ARSENIJEVIC



“If we save just one square millimetre of cell, we can change patients’ lives.”

RENDERING VISUALLY IMPAIRED PEOPLE THEIR VISION

Yvan Arsenijevic, professor at the Faculty of Biology and Medicine of the University of Lausanne and head of the Gene Therapy Unit of the Foundation Asylum for the Blind (FAA), obtained in 2008 a European subsidy for his project AAVEYE (HEALTH field FP7) aimed at proposing a gene therapy to treat certain diseases of vision.

What is the main goal of your research?

I am trying to understand the mechanisms of degeneration, how to slow them down or how to render vision, either through cellular transplant or by gene transfer. Every day our path crosses that of children in the educational centre for visually impaired pupils, therefore we feel very involved in these projects. Our work may not profit their generation, but it will benefit the following.

What was the scope of project AAVEYE?

The name retinitis pigmentosa encompasses more than 200 diseases. Project AAVEYE proposed a gene-therapy approach to treat two recessive, precocious and severe diseases that particularly affect children. This is notably the case of Leber’s congenital amaurosis which could represent 5% of retinal dystrophies. The retina is no longer functional but with some patients it remains in good condition long enough to consider a gene transfer. The study has singled out a new therapeutic vector, efficient when there’s a mutation on genes AILP1

and PDE6b. The tests conducted on animal models have been encouraging.

How did you proceed?

Approximately 200 patients were genotyped, which contributed to enrich the register of diseases of Retina Suisse, and to inform people suffering from rare diseases that different treatments are being evaluated in certain countries to cure retinal dystrophy. The disability of blindness really touches me. Many people daren’t go out of their homes anymore. To recover their eyesight many patients are ready to take the same therapeutic risks as patients in a terminal state. Some perceive blindness as equally serious as a fatal disease.

ABOUT THE PROJECT

Yvan Arsenijevic is involved in two studies financed by the European Union, including project AAVEYE (HEALTH field FP7), terminated in October 2011. Three academic partners – University College London, Naples University and of course the Foundation Asylum for the Blind (FAA) / UNIL – formed a tightly-knit consortium. It was joined by an Estonian small-to-medium company specialised in high output genotyping. Project AAVEYE will have a major impact: the English group

has obtained private financing for doing the first clinical tests. Moreover, Professor Arsenijevic notes that the AAVEYE study has consolidated the relationships between the Foundation Asylum for the Blind, the Institute for research in Ophthalmology and Retina Suisse, and also with the European groups working in this field.