SELECTED OPPORTUNITIES IN GENE THERAPY

Neuroglobin gene therapy for use in the treatment or prevention of a mitochondrial ophthalmic disease associated with respiratory chain complex (BIO14389)

NEUROGLOBIN GENE THERAPY FOR USE IN THE TREATMENT OR PREVENTION OF A MITOCHONDRIAL OPHTHALMIC DISEASE ASSOCIATED WITH RESPIRATORY CHAIN COMPLEX (BIO14389)

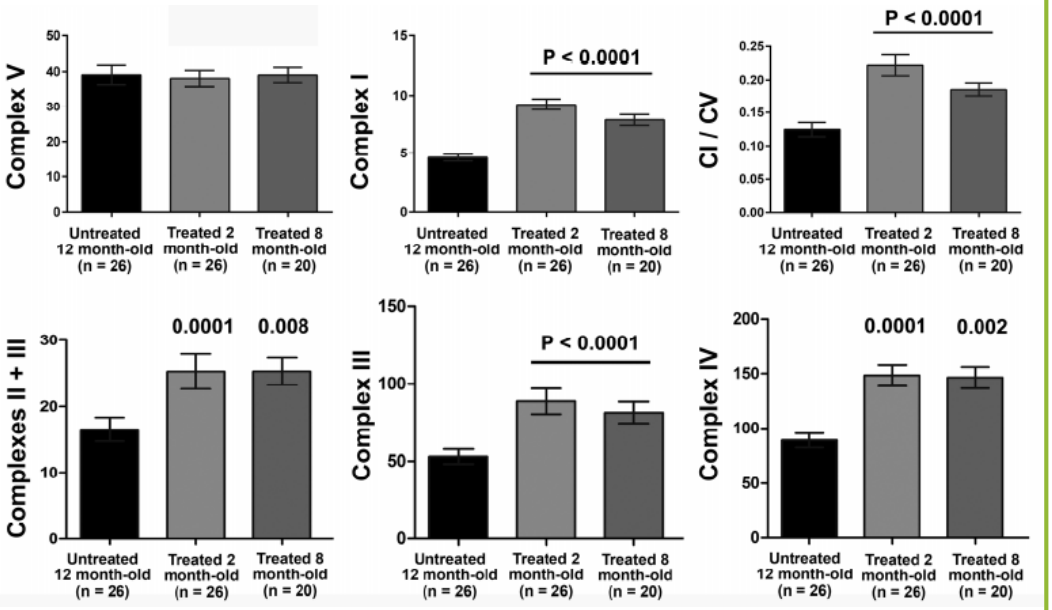
Product factsheet

- **Product:** Neuroglobin agonist (typically gene therapy vector)
- **Mechanism:** NGB expression is decreased in the retina due to a reduction in both the number of NGB-positive cells and the overall NGB expression both at the mRNA and the protein levels in rat models of RCCI deficiency
- **Phase of development: in vitro and in vivo POC**
  - NGB expression knockdown provokes rat retinal ganglion cell (RGC) degeneration and Respiratory Chain Complex RCCI and RCCIII defects in rat optic nerves that engender visual function impairment
  - NGB overexpression was efficient in changing RGC functional fate, via the increased activity of complex I in their axons, which lead to visual function preservation despite the reduced number of nerve fibers
- **Potential applications:** Glaucoma, optic atrophy …
- **Patents:** PCT/EP2014/070991
- **Publications** : Lechauve et al. Mol Ther. 2014

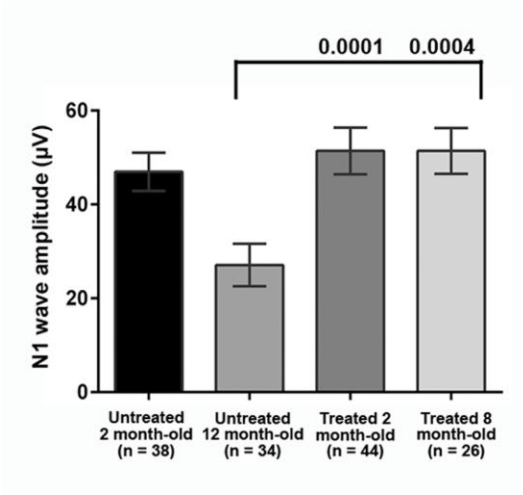
NGB GENE THERAPY TO TREAT OPHTHALMIC MITOCHONDRIAL DISEASES (BIO14389)

Proof of concept

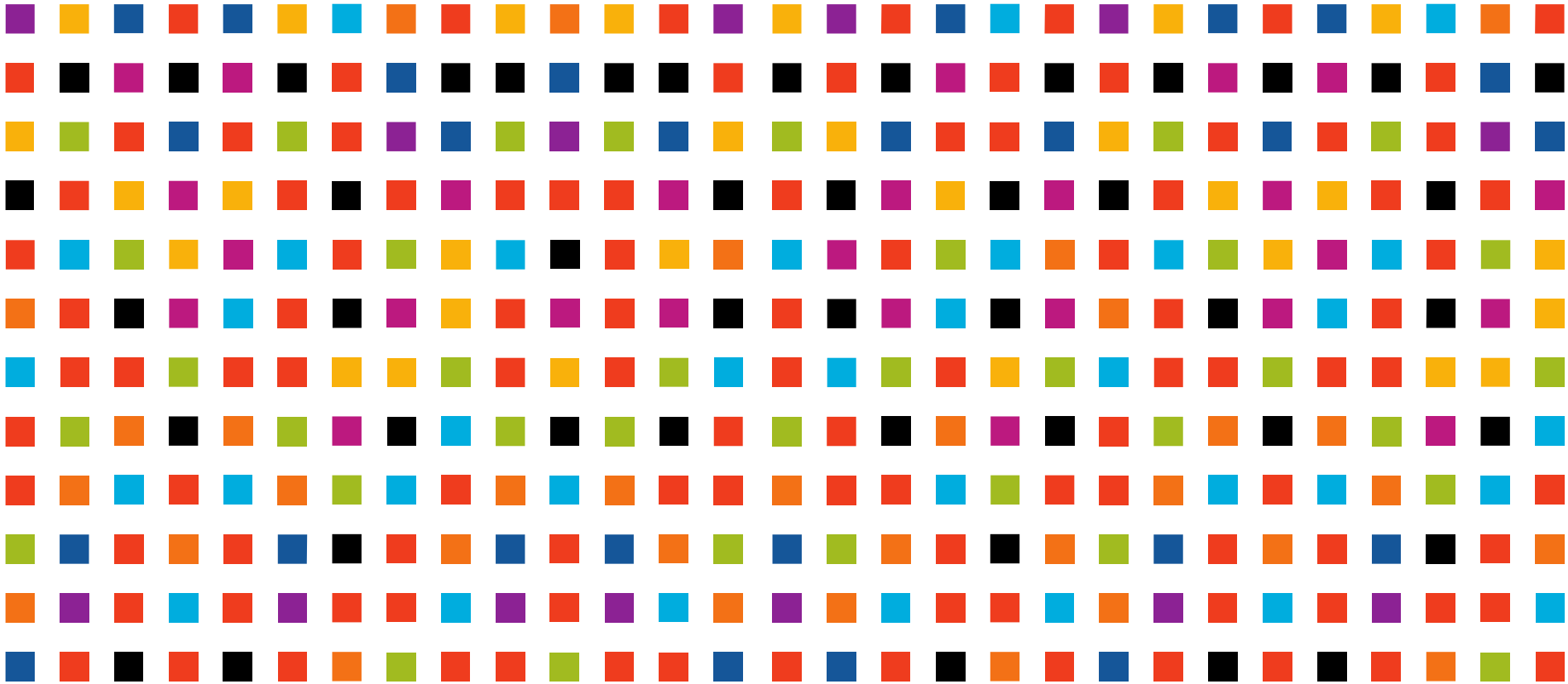
Respiratory chain activities were protected in optic nerves independently of when the AAV2/2-NGB vector was administrated



Preservation of eye to brain communication in AAV2/2-NGB treated eyes protects DBA/2J mouse vision



improved response in the visual cortex upon light stimulation



ANNE.COCHI@INSERM-TRANSFERT.FR

Inserm Transfert - Paris Biopark
7 Rue Watt - 75013 Paris
Tel: +33 1 55 03 01 00 / Fax: +33 55 03 01 60
www.inserm-transfert.fr