PROTOCOLLO DEL PROGETTO DI RICERCA

VERSOSUDICLINICIDITERAPIAGENICADELL'OCCCHIOEDELPEGATO
TRAMITE AAV

Dettagli progetto

TNumber: TGM11MT6 Durata: 1 Data inizio: 23/09/2011 Importo Totale: 100.000 €

Ricercatore titolare

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Malattie

Iperosshalluria primaria Mucopolisaccaridosi tipo VI Amaurosi Congenita di Leber, tipo IV

Summary

Gene therapy of inherited human diseases is obtaining its first clinical successes. At TIGEM we have focused on gene therapy for inherited retinal diseases and inborn errors of metabolism selected based on the absence of a therapeutic option and on the favorable characteristics of these diseases and their target organs for gene transfer. The overall objective of this program is to rapidly move towards clinical investigations gene therapy using adeno-associated viral vectors (AAV) in patients affected with a severe form of childhood blindness and with two inborn errors of metabolism in which the liver is the target organ. All three diseases have been selected based on: i. their recessive pattern of inheritance; ii. severity and favorable risk-benefit ratio; iii. preclinical data supporting the efficacy of AAV2/8 vectors; iv. availability of patients. We propose to produce the pre-clinical evidence of efficacy to further test the AAV-based approaches in patients.

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