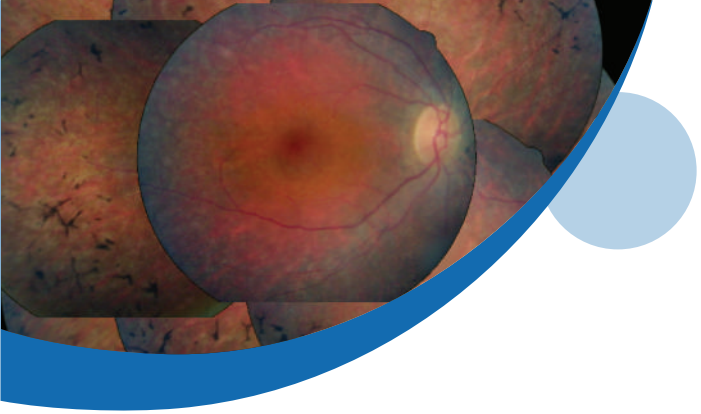
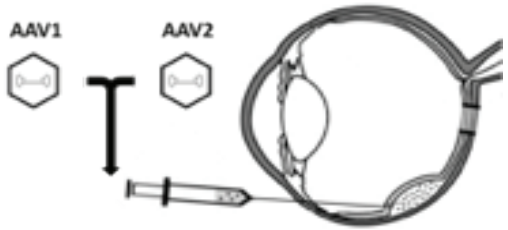


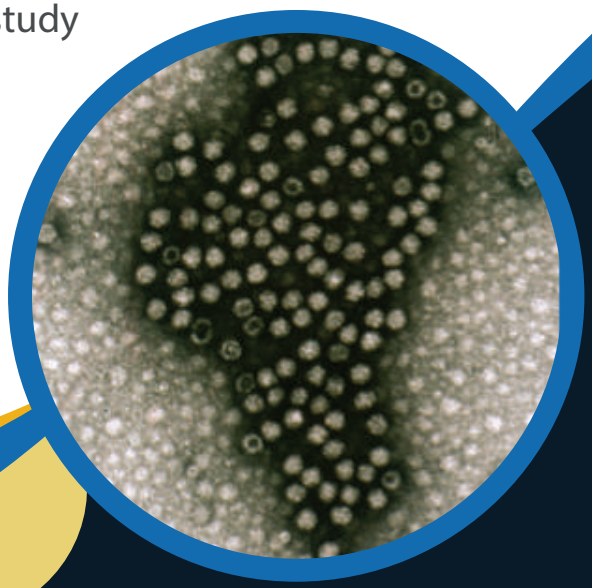
OBJECTIVES

- To produce dual AAV vectors for non-clinical studies and for the clinical trial
- To assess in relevant pre-clinical species the safety, biodistribution and expression of dual AAV vectors manufactured in UshTher
- To perform a clinical observational study on USHIB patients to better define the natural history of USHIB retinitis pigmentosa and establish baseline data on the endpoints that will be monitored in the phase I/II study
- To submit and file the documents required to obtain authorizations to perform a phase I/II clinical trial
- To perform a multicenter phase I/II clinical trial to investigate the safety and efficacy of subretinal administrations of dual AAV8- MYO7A vectors

OUR GOAL

The overarching goal of UshTher is to provide a first-in-human demonstration that gene therapy for USHIB retinitis pigmentosa is both safe and effective using a novel strategy based on dual AAV vectors





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USHTHER CONSORTIUM

USHTHER multidisciplinary consortium includes academic and industrial Partners with internationally-recognized expertise in gene therapy and ophthalmology

USHTHER STAKEHOLDERS

USHTHER project will build a network with key stakeholders: researchers, early-stage career scientists, industries, regulators, patients associations and healthy policy-makers

- Fondazione Telethon, Italy
- Association Genethon, France
- Genosafe, France
- Università degli Studi della Campania Luigi Vanvitelli, Italy
- Instituto Investigation Sanitaria Fundacio Jimenez Diaz, Spain
- Rotterdam Eye Hospital, The Netherlands
- Reithera srl, Italy
- University of Milano-Bicocca, Italy

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USHTHER project has received funding from the European Union's programme Horizon2020
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UshTher
Innovative Gene Therapy for USHIB Retinitis Pigmentosa

Clinical trial of gene therapy with dual AAV vectors for retinitis pigmentosa in patients with Usher syndrome type IB

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