

# RESEARCH ACTIVITY SHEET

#### 2025 PhD selections

### YOUR DETAILS

\* Name & Surname

Alberto Auricchio, Nicola Brunetti Pierri, Alessia Indrieri, Nunzia Pastore, Pasquale Piccolo, Ivana Trapani

\* Affiliation - TIGEM, Telethon Institute of Genetics and Medicine

## PHD PROJECT DETAILS

#### Molecular therapy

\* Title of the proposed project

The mission of TIGEM's Molecular Therapy (MT) research plan is to develop novel strategies for the prevention and treatment of disease. One of our major research strategies is in vivo vector-mediated gene therapy. The general objective is to develop new vector systems using adeno-associated vectors (AAV) and helper dependent adenoviral vector (HDad) delivery methods, test them in the appropriate animal models, and translate the most promising approaches to the clinic.

Here at TIGEM, the AAV vector system has been particularly successful when used in several pre-clinical studies on lysosomal storage disorders (LSDs) and in a vast series of animal models of retinal degenerations. We also utilize small molecules such as specific chaperones for the treatment of LSDs.

- \* Short description of the project (up to 300 words)
- \* Indicate the main research area for the project described above -

If needed indicate a second research area for the project described above -

- \* Provide up to 3 key words for project:
- 1. Molecular Biology
- 2. Genetic disorders
- 3. Therapeutic tools

# YOUR LABORATORY ACTIVITIES DETAILS

Alberto Auricchio: https://www.tigem.it/research/research-faculty/auricchio

Nicola Brunetti Pierri: https://www.tigem.it/research/research-faculty/brunetti

Alessia Indrieri: https://www.tigem.it/research/research-faculty/indrieri

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