UNIVERSITÀ DI TRENTO
Dipartimento di Biologia Cellulare, Computazionale e Integrata - CIBIO

30 MARCH

2 P.M.
ROOM A109 - POVO1

IVANA TRAPANI

THE TELETHON INSTITUTE OF GENETICS AND MEDICINE

EXTERNAL SEMINAR

AAV-BASED GENE THERAPIES FOR INHERITED RETINAL DISORDERS DUE TO MUTATIONS IN LARGE GENES

Adeno-associated viral (AAV) vectors are widely used for **in vivo gene therapy**; however, their limited packaging capacity poses a **major challenge** for treating inherited retinal disorders caused by **large genes**. To address this, we have developed different strategies based on **dual AAV platforms and AAV-mediated genome editing approaches**. In the **dual AAV strategy**, large therapeutic proteins are split into smaller fragments, delivered via separate vectors, and reconstituted in target cells. In parallel, we are developing **genome editing approaches** to enable the **integration of large DNA templates into diseased genes**, allowing **correction of multiple mutations** with a single therapeutic construct. These methodologies aim to **overcome current delivery constraints** and enable broader, **mutation-independent therapeutic applications**. In this seminar, I will present the conceptual framework of these approaches, summarize **preclinical data** obtained in relevant models of **inherited retinal diseases**, and discuss their potential to expand the **therapeutic landscape**.