Sant'Anna
Scuola Universitaria Superiore Pisa

28 April 2026

Scuola Superiore Sant'Anna
Aula Magna Storica

6:00 - 7:00 pm

PhD in Translational Medicine - Seminars

Enhancing ASOs Activity through Genetic Modulation and Small-Molecule Adjuvants in Myotonic Dystrophy Type 1

Abstract

This PhD project explores how to improve the effectiveness of antisense oligonucleotides (ASOs) for treating Myotonic Dystrophy type 1 (DM1), focusing on overcoming limitations in intracellular delivery and endosomal entrapment. Using a HeLa reporter system, the research combines chemical screening and genetic approaches to identify compounds and cellular pathways that enhance ASO uptake and activity. Promising candidates will be validated in patient-derived cells and animal models, with the goal of developing more effective therapies for DM1 and related neuromuscular disorders.

Short Bio

Yasmine Ferchichi, born in Italy and raised in Tunisia, developed an early interest in rare diseases during her studies in Biological Sciences at the University of Tuscia. She later specialized in Applied Biology at the University of Pisa, where she researched spinocerebellar ataxia SCA27B in collaboration with leading neurogenetics experts.

She gained research experience at the University of Pisa and the Sant'Anna School of Advanced Studies, contributing to a European project on biomedical micro-robotics. She is currently a Marie Skłodowska-Curie PhD candidate at the Universitat de València, where her work focuses on improving the intracellular delivery and therapeutic effectiveness of antisense oligonucleotides (ASOs) for neuromuscular diseases such as Myotonic Dystrophy type 1.

ENTRY DM

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