

PhD in BIOINGEGNERIA / BIOENGINEERING - 41st cycle

THEMATIC Research Field: SPINAL MUSCULAR ATROPHY TRANSLATING CUSTOMIZED HEALTH INNOVATIONS TO NEWBORN AND GROWN-UP PATIENTS

Monthly net income of PhDscholarship (max 36 months)

1400.0

In case of a change of the welfare rates during the three-year period, the amount could be modified.

Context of the research activity	
Motivation and objectives of the research in this field	Spinal muscular atrophy (SMA) natural history is changing with the advent of disease-modifying drugs (DMDs). However, SMA patients' response to DMDs is partial and highly variable, with marked benefits in the precocious treatments. This project aims to disclose, by high-throughput multi- omics profiling in a patient-derived skeletal-muscle in vitro model, new druggable muscle intrinsic pathogenic processes leading to atrophy and develop and assess the efficacy of novel nanobiotechnology-based treatments targeting molecular SMA-associated pathways.
Methods and techniques that will be developed and used to carry out the research	This project focuses on developing innovative technologies, based on gene/non-coding RNA delivery via nanobiotechnologies, to counteract progressive muscle atrophy in a new patient-specific in vitro model of SMA (skeletal muscle cells derived from patient-specific induced pluripotent stem cells (iPSCs)). As part of this project, the successful candidate will gain experience in developing (synthesis and/or purification, and formulation with nucleic acids) and characterizing nanoassembly dispersions (dynamic light scattering (DLS), Z-potentiometer, gel retardation assay (GRA)). Furthermore, they will perform cellular biology techniques such as proliferation in vitro assays, cutting-edge molecular biology techniques such as real-time PCR, digital PCR, Taqman, and biochemical techniques like multiplex immunoassays, immunofluorescence, and



	multiplex immunoassays, immunofluorescence, and RNAscope. The candidate will have also the opportunity to collaborate in the execution of high-throughput RNA- sequencing and proteomic profiling of iPSC-derived skeletal muscle cells.
Educational objectives	The PhD candidate will gain a thorough understanding of different technologies used for creating and characterizing nanoassemblies. Furthermore, the candidate will become proficient in the latest techniques in cell and molecular biology, along with biochemical methods, to identify new personalized druggable targets and assess the effectiveness of their modulation.
Job opportunities	R&D in biomedical, pharmaceutical, and biotech companies. Post-DOC scientist in academia, IRCCS hospital institutes, and research centres (e.g., CNR, IIT).
Composition of the research group	1 Full Professors 0 Associated Professors 4 Assistant Professors 5 PhD Students
Name of the research directors	G. Candiani, N. Bono, S. Bonanno, S. Marcuzzo

Contacts

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Additional support - Financial aid per PhD student per year (gross amount)	
Housing - Foreign Students	
Housing - Out-of-town residents	

POLITECNICO DI MILANO



Amount monthly	700.0 €
By number of months	6

Additional information: educational activity, teaching assistantship, computer availability, desk availability, any other information

Prof. Gabriele Candiani,

Dr. Nina Bono,

Dr. Silvia Bonanno,

Dr. Stefania Marcuzzo

Educational activity: The student will be encouraged to attend to courses at POLIMI or abroad 2 / 3 in International Schools.

Teaching assistantship: There are various forms of financial aid for activities of support to the teaching practice. The PhD student is encouraged to take part in these activities, within the limits allowed by the regulations.

Computer and desk availability: the student will be allowed to access facilities of the DEIB.