research



# PhD in BIOINGEGNERIA / BIOENGINEERING - 39th cycle

## PNRR 117 Research Field: DEVELOPMENT OF NEW THERAPEUTIC GENE DELIVERY APPROACHES FOR NEUROMUSCULAR DISEASES

### 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.

Con	text of the research activity
Motivation and objectives of the research in this field	Neuromuscular disorder (NMD) encompasses a heterogeneous group of disorders affecting skeletal muscles due to abnormalities in nerve, neuromuscular junction, ion channels, or metabolic derangements. Unfortunately, the large majority of these diseases are currently incurable.  On the other hand, the COVID-19 pandemic has created several opportunities for continued growth in gene therapy. Gene therapy as a treatment for NMDs is an ever-developing concept based on using nucleic acid as a therapeutic agent. In the search for appropriate strategies, however, many bottleneck exists.  This research project aims to develop innovative nucleic acid delivery tools for new patient-specific therapeutic strategies to tackle neuromuscular diseases. We aim to develop next-generation gene delivery assemblies, much more effective than first-generation ones, and devices to deliver specific kinds of nucleic acids into the target cell population.
Methods and techniques that will be developed and used to carry out the	This project focuses on developing innovative techniques for assembling nucleic acid-vector molecules and cell-stimulating devices to deliver nucleic acids for therapeutic purposes in patient-specific in vitro models (motor

neurons and muscle cells derived from patient-specific

will gain experience in designing cellular stimulation

induced pluripotent stem cells (iPSCs) in neuromuscular disease). As part of this project, the successful candidate

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	will gain experience in designing cellular stimulation devices, developing and characterizing nanocarrier dispersions (VibroFect, DLS, and Z-potentiometer), performing cellular biology techniques such as proliferation and in vitro assays, using molecular biology techniques such as real-time PCR, digital PCR, Taqman Array Cards, and Methyl-Seq, and employing biochemical techniques like multiplex immunoassays, immunofluorescence, and RNAscope.
Educational objectives	The Ph.D. candidate will acquire a comprehensive understanding of various technologies for shaping nanoassemblies and methods for characterizing them. Additionally, they will develop the skills necessary to design and create a device for mechanically stimulating cells and influencing their behavior.  Furthermore, they will gain proficiency in the latest cell biology, molecular biology, and biochemical techniques for evaluating the effectiveness and, consequently, the results of a specific biomedical stimulus.
Job opportunities	R&D in biomedical, pharmaceutical, and biotech companies. Post-DOC scientist in academia and IRCCS hospital institutes.
Composition of the research group	0 Full Professors 1 Associated Professors 2 Assistant Professors 6 PhD Students
Name of the research directors	PROFF. GABRIELE CANDIANI - STEFANIA MARCUZZO

### Contacts

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https://www4.ceda.polimi.it/manifesti/manifesti/controller/ricerche/RicercaPerDocentiPublic.do?EVN\_ELENCO\_DIDATTICA=evento&lang=IT&k\_doc=277651&aa=2023&n\_docente=candiani&tab\_ricerca=2&jaf\_currentWFID=main

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

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Housing - Out-of-town residents (more than 80Km out of Milano)	
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Scholarship Increase for a period abroad		
Amount monthly	700.0 €	
By number of months	6	

National Operational Program for Research and Innovation	
Company where the candidate will attend the stage (name and brief description)	Fondazione IRCCS Istituto Neurologico Carlo Besta (FINCB)
By number of months at the company	6
Institution or company where the candidate will spend the period abroad (name and brief description)	To be defined
By number of months abroad	6

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

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

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

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