

PNRR Missione 4, Componente 2, Investimento 1.4 “Potenziamento strutture di ricerca e creazione di "campioni nazionali di R&S" su alcune Key Enabling Technologies”
Iniziativa finanziata dall'Unione europea — NextGenerationEU.

National Center for Gene Therapy and Drugs based on RNA Technology
Sviluppo di terapia genica e farmaci con tecnologia a RNA

Codice progetto MUR: **CN00000041** – CUP UNINA: **E63C22000940007**

Doctorate of National Interest
RNA THERAPEUTICS AND GENE THERAPY

TITLE OF THE RESEARCH PROJECT

Characterization and Therapeutic Validation of Spinal Cord Neural Circuit Alterations in Neuromuscular Diseases

SELECT ONE OF THE FOLLOWING RESEARCH AREA:

- ☐ Mechanisms of Diseases and Drug Target Identification
- ☒ Design and Delivery of New Gene Therapy and RNA-Based Medicines
- ☐ Validation and Safety In Preclinical and Clinical Studies

LOCATION OF THE RESEARCH ACTIVITY (INSTITUTION/DEPARTMENT):

Dipartimento di Fisiopatologia Medico-Chirurgica e dei Trapianti
Padiglione ex convitto suore, piano meno 1, Fondazione IRCCS Ca' Granda Ospedale Maggiore
Policlinico di Milano

TUTOR:

Yvan Torrente

PROPOSED RESEARCH ACTIVITIES (max 300 words):

Characterization of possible alterations in the neural circuit of the spinal cord in murine models of neuromuscular pathology, characterization of alterations in the NMJ, and validation of new gene and molecular therapy approaches aimed at restoring and improving the functional muscle unit.