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 THERAPUETICS AND GENE THERAPY

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):

University of Padova

Department of Biomedical Sciences

TUTOR: Prof. Rosario Rizzuto

PROPOSED RESEARCH ACTIVITIES (max 300 words):

The research activities will focus on developing RNA-based therapies for metabolic and genetic diseases. On the one hand, metabolic, cardiovascular, and skeletal muscle diseases majorly impact global health; on the other hand, therapies for severe monogenic disorders are needed.

Thanks to the previous studies, several targets will be investigated to develop RNA-based drugs to counteract skeletal muscle atrophy during adulthood and aging. In detail, a subset of genes has been already identified; the inhibition of which results in muscle mass and mitochondrial function recovery in different catabolic conditions such as loss of innervation, aging, and metabolic disorders. The project aims to first use a local RNA-based therapy on isolated muscles, then expand the study to whole-body muscles to reach a systemic effect by enhancing muscle mass and metabolism. A

best RNA cocktail (targeting a selection of genes involved in protein degradation, anabolism, and mitochondrial function) will be selected to recover muscle function in the different catabolic conditions.

In addition, these research activities will aim to treat a selected group of uncurable genetic disorders. Thus, cutting-edge viral vector-based or genome and base editing technology for gene therapy will be developed.