BIOGRAPHICAL SKETCH

NAME: Angela Gritti

POSITION TITLE: **Associate Professor of Histology** | Vita-Salute San Raffaele University; **Group Leader** | Gene and neural stem cell therapy for LSD | San Raffaele Telethon Institute for Gene Therapy (SR-Tiget) - San Raffaele Scientific Institute, Via Olgettina 60, 20132 Milano – Italy

EDUCATION/TRAINING

University of Milan, Italy	Master's degree in Biological Sciences (summa cum laude)	1990	Biological Sciences, Pharmacology
Italy	Qualification to practice as a biologist	1991	
University of Milan, Italy	Specialty in Toxicology (70/70)	1996	Toxicology
University of Turin, Italy	PhD in Basic Sciences and Veterinary Biotechnology (XVIII cycle)	2006	Neuroscience, comparative neurogenesis, neural stem cells
Italy	National Academic Qualification as Associate Professor	2018	05/H2 - Histology

PERSONAL STATEMENT

As a Principal Investigator at Ospedale San Raffaele (OSR), San Raffaele Telethon Institute for Gene Therapy (SR-Tiget, Milan), I lead a team focusing on rare genetic diseases affecting the CNS, particularly Lysosomal Storage Disorders (LSDs) such as Metachromatic Leukodystrophy (MLD), Globoid Cell Leukodystrophy (GLD), Alexander's Disease (AxD), Sandhoff Disease (SD), and Tay-Sachs Disease (TSD). Our research spans basic to translational approaches, utilizing murine and human models for in vivo and ex vivo gene therapy, cell therapy, gene addition, and genome- and epigenome editing. We study early pathogenic events and therapeutic mechanisms to refine gene therapy strategies, employing patient-specific iPSC-derived neural populations for in vitro modeling.

As an assistant professor of Human Histology at the Univ. Vita-Salute San Raffaele, Milan, Master's degree in Medicine and Surgery and International MD program, I combine frontal and practical lessons and promote the active participation of students through interactive group discussions. My goal is to provide a comprehensive overview of the primary tissue types and subtypes, their developmental origin, function, maintenance, and contribution to organs and systems, addressing from a scientifically informed standpoint the recent advances in stem cell manipulation, tissue replacement, and regeneration while highlighting their current limitations and significant gaps in our knowledge.

EMPLOYMENT AND EXPERIENCE

2006-present	Group Leader, Unit of Gene/neural stem cell therapy for lysosomal storage diseases, IRCCS Ospedale San Raffaele (OSR), San Raffaele Telethon Institute for Gene Therapy (SR-Tiget).
2016-present	Adjunct professor, course "Human Histology" (course coordinator from a.a. 2019/2020), School of Medicine, Master's degree in Medicine and Surgery and International MD program, Univ. Vita-Salute San Raffaele, Milan, Italy.

PUBLICATIONS

Scopus Author ID: 7004129522; ORCID ID: 0000-0002-9845-0370; Researcher ID: K-2729-2016

Complete list of publications at: https://www.scopus.com/authid/detail.uri?authorId=7004129522

5 most relevant publications within the last ten years:

- Sala D, Ornaghi F, Morena F, Argentati C, Valsecchi M, Alberizzi V, Di Guardo R, Bolino A, Aureli M, Martino S**, Gritti A.** Therapeutic advantages of combined gene/cell therapy strategies in a murine model of GM2 gangliosidosis. *Mol Ther Methods Clin Dev. 2022* 25:170-189. doi: 10.1016/j.omtm.2022.03.011.
- Mangiameli E, Cecchele A, Morena F, Sanvito F, Matafora V, Cattaneo A, Della Volpe L, Gnani D, Paulis M, Susani L, Martino S, Di Micco R, Bachi A, **Gritti A.** Human iPSC-based neurodevelopmental models of globoid cell leukodystrophy uncover patient- and cell type-specific disease phenotypes. *Stem Cell Reports. 2021* 16(6):1478-1495. doi: 10.1016/j.stemcr.2021.04.011.
- Frati G, Luciani M, Meneghini V, De Cicco S, Ståhlman M, Blomqvist M, Grossi S, Filocamo M, Morena F, Menegon A, Martino S, **Gritti A.** Human iPSC-based models highlight defective glial and neuronal differentiation from neural progenitor cells in metachromatic leukodystrophy. *Cell Death Dis. 2018* 9(6):698. doi: 10.1038/s41419-018-0737-0.
- Meneghini V, Frati G, Sala D, De Cicco S, Luciani M, Cavazzin C, Paulis M, Mentzen W, Morena F, Giannelli S, Sanvito F, Villa A, Bulfone A, Broccoli V, Martino S, **Gritti A.** Generation of Human Induced Pluripotent Stem Cell-Derived Bona Fide Neural Stem Cells for Ex Vivo Gene Therapy of Metachromatic Leukodystrophy. *Stem Cells Transl Med. 2017* 6(2):352-368. doi: 10.5966/sctm.2015-0414.
- Meneghini V, Lattanzi A, Tiradani L, Bravo G, Morena F, Sanvito F, Calabria A, Bringas J, Fisher-Perkins JM, Dufour JP, Baker KC, Doglioni C, Montini E, Bunnell BA, Bankiewicz K, Martino S, Naldini L, **Gritti A.** Pervasive supply of therapeutic lysosomal enzymes in the CNS of normal and Krabbe-affected non-human primates by intracerebral lentiviral gene therapy. *EMBO Mol Med. 2016* 8(5):489-510. doi: 10.15252/emmm.201505850.

RESEARCH FUNDING

In the last 10 years Angela Gritti has been the principal or co-principal investigator of 10 grants (**active grants in bold**): two from the Italian Telethon Fundation **(TTAGD0222TT**; TGT16D02), one from the Italian Ministry of Health (RF-2016-02362404), four from the European Leukodystrophy Association (**ELA; ELA 2022-009C2; ELA 2022-006C2**; ELA 2019-015I2; ELA 2020-011I2), one from the patient's association Vaincre les Maladies Lysosomales (VML), one from the European Joint Programme on Rare Diseases (EJP RD; project NG4leuko), one from the Bespoke Gene Therapy Consortium (BGTC; **FNIH RFP NUMBER: 2022-BGTC003**).