PERSONAL INFORMATION

Michela Milani

San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget)
Via Olgettina 58, Milan (20132) - Italy

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Sex Female | *Date of birth* 14/07/1990 | *Nationality* Italian (MLNMHL90L54F205V)

Enterprise	University	EPR
☐ Management Level	☐ Full professor	☐ Research Director and 1st level Technologist / First Researcher and 2nd level Technologist / Principal Investigator
☐ Mid-Management Level	☐ Associate Professor	☒ Level III Researcher and Technologist
☐ Employee / worker level	☒ Researcher and Technologist of IV, V, VI and VII level / Technical collaborator	☐ Researcher and Technologist of IV, V, VI and VII level / Technical collaborator

WORK EXPERIENCE

Since 2024

Project Leader

San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget)

- *In vivo* gene transfer into hematopoietic stem and progenitor cells (visiting scientist at the CIEMAT institute, Madrid, Spain – Paula Rio lab)
- Generation of universal re-targeted lentiviral vectors, tailored for *in vivo* use

Biotechnology, Gene Therapy

Since 2022

Contract Professor

“Vita-Salute San Raffaele” University

- Histology: Teacher, assistant of practicals in microscopic morphology

Histology

2019-2024

Post-doctoral researcher

San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget)

- Pre-clinical and clinical development of hemophilia A and B gene therapy
- *In vivo* gene transfer into hematopoietic stem and progenitor cells (visiting scientist at the CIEMAT institute, Madrid, Spain – Paula Rio lab)
- Identification and characterization of facultative liver stem cells
- Lentiviral vector engineering and development of strategies to improve manufacturing

Biotechnology, Gene Therapy

2015

Research fellow

San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget)

- Pre-clinical development of hemophilia A and B gene therapy
- Lentiviral vector engineering and development of strategies to improve manufacturing
- Evaluation of lentiviral vector safety *in vitro*

Biotechnology, Gene Therapy

EDUCATION AND TRAINING

2015-2019

Ph.D. in Molecular Medicine

“Vita Salute San Raffaele” University, Milan, IT

Advancing lentiviral vectors for liver-directed gene therapy - Prof. Luigi Naldini

- Pre-clinical development of hemophilia A and B gene therapy
- Lentiviral vector engineering and development of strategies to improve manufacturing

- Identification and characterization of facultative liver stem cells (visiting scientist at the Gurdon Institute, Cambridge, UK - Meritxell Huch lab)

2012-2014 **Master of Science in Medical, molecular and cellular biotechnology**

"Vita Salute San Raffaele" University, Milan, IT

Targeted genome editing of cell lines for improved and scalable production of lentiviral vectors for human gene therapy - Prof. Luigi Naldini - 110/110 *cum laude*

- Lentiviral vector engineering and development of strategies to improve manufacturing

2009-2012 **Bachelor of Science in Medical and pharmaceutical biotechnology**

"Vita Salute San Raffaele" University, Milan, IT

Prions: different strains, one genetic information - Prof. Roberto Burioni - 110/110

WORK ACTIVITIES

Awards Meritorious Abstract Travel Award (ASGCT 2017, 2018, 2021, 2022, 2023; ISSCR 2019, 2020, 2021, 2024, ESGCT 2024), selected for Roche Continents (2018), FCSR Fellowship program (2018), Student and Fellow Awards for Excellence in Research Award (ASGCT, 2018), Under40 in Hematology (2022), Nature photo contest #ScientistsAtWork (2022).

Grants Fondo Italiano per la Scienza, FIS2 (Italian Ministry of University and Research, 2024)

Editorial activity Reviewer of manuscripts submitted to Gene Therapy, Frontiers in Bioengineering and Biotechnology, Viruses, Biomolecules and Molecular Therapy Methods and Clinical Development. Poster reviewer for ESGCT and ASGCT.

Invited presentations Invited speaker at the Stem Cell Clonality & Genome Stability Retreat (Sevilla, Spain; 2025), WFH New Technologies and Gene Therapy Workshop (Montreal, Canada; 2024), Fanconi Cancer Foundation Scientific symposium (Charlotte, USA; 2024), ASGCT (Baltimore, USA; 2024 – New Orleans, USA; 2025), Frontiers Health (Milan, Italy; 2022) and at the "Festival della Scienza" (Genova, Italy; 2020)

Mentoring activity Supervised four Master students (Biotechnology and Medical Biology – "Vita-Salute San Raffaele" University, Milan, Italy; Medical and Pharmaceutical Biotechnology – "Università degli Studi di Trieste", Italy); invited to be part of a PhD thesis dissertation board (Universidad Autonoma de Madrid, Spain, 2024).

Teaching activity Contract professor of the "Histology" course for students in the medical degree at "Vita-Salute San Raffaele" University (Milan, Italy) since 2022.

Patents Inventor of 5 granted and pending patent applications:

- "Vector Production" – Priority date May 16th 2018 – UK patent application number: 1807945.9
- "Use of lentiviral vectors expressing factor FVIII" – Priority date January 31st 2019 – USA patent application number: PCT/US2019/016122
- "Vector Production" – Priority date April 28th 2023 – EU patent application number: EP23170841.3
- "Novel Lentiviral Vector Particles" – Priority date September 05th 2023 – EU patent application number: EP23195542
- "Combinations, Inhibitors, Viral Vectors, Methods, and Uses for *In Vivo* Gene Therapy and Gene Editing" – Priority date April 19th 2024 – EU patent application number: EP24171391.6

PERSONAL SKILLS

Mother tongue Italian

Other languages

	UNDERSTANDING		SPEAKING		WRITING
	Listening	Reading	Spoken interaction	Spoken production	
English	C1	C1	C1	C1	C1
	First Certificate (Grade A) – Cambridge English				
Spanish	B1	B1	A2	A2	A2

Levels: A1/A2: Basic user - B1/B2: Independent user - C1/C2 Proficient user
Common European Framework of Reference for Languages

Communication skills

Excellent communication skills, acquired during my university studies and my volunteering activity as a team leader of an educational and personal growth path for 15-19 years old people. During my Ph.D. I also undertook a number of activities aimed at communicating science to the general public. Among these, the most important one has been the "#365GiorniDaRicercatore" ("365 days as a researcher") project, during which for one year I posted every day on my personal Facebook account facts related to the life of a researcher, ethics and science.

Organisational skills

Great organizational skills matured during numerous team work experiences both in the laboratory and outside in the band that I founded in 2013 and in which I sing.

Job-related skills

Ability in the usage of different laboratory techniques: cell line and primary cell culture, ELISA assays, protein and nucleic acid electrophoresis, western blot, PCR, DNA cloning and purification techniques, lentiviral vector production and characterization, liver organoid generation and culturing, mice handling, flow cytometry, imaging and single cell/spatial transcriptomic techniques.

Digital competence

SELF-ASSESSMENT				
Information processing	Communication	Content creation	Safety	Problem solving
Proficient user	Proficient user	Independent user	Independent user	Proficient user

Levels: Basic user - Independent user - Proficient user
Digital competences - Self-assessment grid

European Computer Driving License

- Excellent command of Microsoft OfficeTM tools
- Excellent command of photo editing software gained as an amateur photographer
- Excellent command of GraphPad, FCSExpress, QuantaSoft

Driving licence B

ADDITIONAL INFORMATION

Publications under review

Milani M*, Starinieri F*, Beretta S, Canepari C, Monti M, Zambrano S, Mazza D, Fabiano A, Simoni C, Cammarota E, Volpin M, Bortolussi G, Russo F, Biffi M, Genua M, Ostuni R, Muro AF, Montini E, Moalli F, Iannacone M, Merelli I, Cantore A. Spatiotemporal liver dynamics shapes hepatocellular heterogeneity and impacts in vivo gene engineering. Under review. *Equal contribution.

Publications

total number of publications in peer-review journals: 7 (8)
total number of citations: 193; H-index: 5

Milani M, Annoni A, Bartolaccini S, Biffi M, Russo F, Di Tomaso T, Raimondi A, Lengler J, Holmes MC, Scheiflinger

F, Lombardo A, Cantore A, Naldini L. Genome editing for scalable production of alloantigen-free lentiviral vectors for in vivo gene therapy. EMBO Mol Med. 2017 Nov;9(11):1558-1573. doi: 10.15252/emmm.201708148.

Milani M*, Annoni A*, Moalli F, Liu T, Cesana D, Calabria A, Bartolaccini S, Biffi M, Russo F, Visigalli I, Raimondi A, Patarroyo-White S, Drager D, Cristofori P, Ayuso E, Montini E, Peters R, Iannacone M, Cantore A, Naldini L. Phagocytosis-Shielded Lentiviral Vectors Improve Liver Gene Therapy in Non Human Primates. Sci Transl Med. 2019 May 22;11(493). pii: eaav7325. doi: 10.1126/scitranslmed.aav7325. *Equal contribution

Milani M, Canepari C, Liu L, Biffi M, Russo F, Plati T, Curto R, Patarroyo-White S, Drager D, Visigalli I, Brombin C, Albertini P, Follenzi A, Ayuso E, Mueller C, Annoni A, Naldini L, Cantore A. Liver-directed lentiviral gene therapy corrects hemophilia A mice and achieves normal-range factor VIII activity in non-human primates. Nat Commun. 2022 May 4;13(1):2454. doi: 10.1038/s41467-022-30102-3.

Ho N, Agarwal S, **Milani M**, Cantore A, Buchholz CJ, Thalheimer FB. In vivo generation of CAR T cells in the presence of human myeloid cells. Mol Ther Methods Clin Dev. 2022 Jun 9;26:144-156. doi:10.1016/j.omtm.2022.06.004. eCollection 2022 Sep 8.

Milani M, Canepari C, Assanelli S, Merlin S, Borroni E, Starinieri F, Biffi M, Russo F, Fabiano A, Zambroni D, Annoni A, Naldini L, Follenzi A, Cantore A. Lentiviral vectors bearing the baculovirus GP64 surface protein efficiently transduce liver endothelial cells in vivo and correct mouse hemophilia A. EMBO Mol Med. 2024.

Milani M, Starinieri F*, Fabiano A*, Beretta S, Plati T, Canepari C, Biffi M, RussoF, Norata R, Sanvito F, Merelli M, Aloia L, Huch M, Naldini L, Cantore A. Identification of hepatocyte-primed biliary epithelial cells in the homeostatic liver by in vivo lentiviral gene transfer to mice and non-human primates. EMBO Mol Med. 2024 Jun;16(6):1427-1450. doi: 10.1038/s44321-024-00072-8. Epub 2024 Apr 29. *Equal contribution.

Milani M[§], Fabiano A, Perez-Rodriguez M, Hernandez RJ, Zecchillo A, Zonari E, Ottonello S, Basso-Ricci L, Volpin M, Iannello V, Capo V, Quaranta P, Seffin L, Russo F, Biffi M, Ormoli L, Brombin C, Carlucci F, Forlino A, Filibian M, Montini E, Scala S, Villa A, Bueren JA, Rio P, Aiuti A, Cantore A*, Naldini L[*,§]. In vivo hemopoietic stem cell gene therapy enabled by postnatal trafficking. *Nature – Accepted in principle*. 2025. *Equal contribution. [§]Corresponding authors.

Solforosi L*, **Milani M***, Mancini N, Clementi M, Burioni R. A closer look at prion strains: characterization and important implications. **Prion.** 2013 Mar-Apr;7(2):99-108. doi: 10.4161/pri.23490. Epub 2013 Jan 28. *Equal contribution. Review

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