BIOGRAPHICAL SKETCH

NAME	POSITION TITLE
NALDINI LUIGI	*Professor* of Cell and Tissue Biology and *Professor* of Gene and Cell Therapy, "Vita Salute San Raffaele" University School of Medicine, Milan, Italy *Director*, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy

EDUCATION/TRAINING

INSTITUTION	DEGREE	YEAR(s)	FIELD OF STUDY
University of Torino Medical School	M.D.	1983	
University of Rome " La Sapienza"	Ph.D.	1983 - 1987	Cell and Developmental Biology
Meloy Laboratories (with Yossi Schlessinger), Rockville, MD	Post-doctoral training	1987 - 1989	Tyrosine Kinases and Signal Transduction

EMPLOYMENT AND EXPERIENCE

1990 - 1996	Assistant Professor of Cell and Tissue Biology, Dept. of Biomedical Sciences and Oncology, University of Torino Medical School (since 1993 with tenure).
1994 - 1996	Visiting Scientist, The Laboratory of Genetics (Director: Inder M. Verma), the Salk Institute for Biological Studies, La Jolla, CA.
1996 - 1998	Senior Scientist and Director, Lentiviral Vector Project, Somatix Therapy Corp. & Cell Genesys, Foster City, CA.
1998 - 2002	Associate Professor of Cell and Tissue Biology, University of Torino Medical School and Head, Laboratory for Gene Transfer and Therapy, Institute for Cancer Research, Candiolo (Torino), Italy.
Since 2002	Full Professor of Cell and Tissue Biology, "Vita Salute San Raffaele" University School of Medicine, Scientific Codirector San Raffaele Telethon Institute for Gene Therapy, Milan, Italy.
Since 2005	Full Professor with tenure
Since 2008	Scientific Director, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy.

PROFESSIONAL ACTIVITIES

- Past President of the European Society of Gene and Cell Therapy (ESGCT; 2012-2014)
- Member of the Board of Directors (2005-2008) and the Advisory Council (2008-2012) of the American Society of Gene and Cell Therapy (ASGCT)
- Ad hoc advisor of EMA and WHO Committees for the evaluation of novel gene transfer medicines
- Past and current member of several Scientific Committees of the ASGCT, ESCGT, American Association of Cancer Research (AACR), International Society for Stem Cell Research (ISSCR), International Society of Cell Therapy (ISCT)
- Associate Editor (until 2012) *Human Gene Therapy*
- Advisory Editorial Board Member, *EMBO Molecular Medicine* and *Cell Stem Cell*

- Appointed member of the Human Gene Editing Study Committee by the National Academies of Sciences and of Medicine of the USA (2015-2017)
- Appointed member of the Advisory Committee to the Italian Ministry of Health (since 2015)
- Appointed member of the Italian National Advisory Committee on Biosafety, Biotechnology and Life Sciences in 2016 (2016-2020, renewed 2021-2024 and 2024-2027)
- President of the Italian Society of Gene and Cell Therapy (SITGEC; since Foundation in 2024)

PATENTS

Inventor of 58 granted international patents and 45 pending. These include a cornerstone patent on lentiviral vector technology owned by the Salk Institute and a family originator patent owned by Cell Genesys. Intellectual property generated at the San Raffaele Institute covers bidirectional vectors for coordinate gene expression, micro-RNA regulated vectors, angiogenic monocytes and macrophage microRNAs, tolerogenic vectors, vector production and engineering, transplantation and gene editing technologies.

HONORS

- Elected Member of *EMBO*, the *European Molecular Biology Organization*, in 2008.
- Awarded the European Research Council (ERC) *Advanced Investigator grant*, which recognizes top EU scientists with an outstanding achievement track record in 2009.
- Premio Sapio of the Italian Research (Health Area) in 2012.
- Outstanding Achievement Award from the American Society of Gene and Cell Therapy in 2014.
- Human Gene Therapy "Pioneer Award" 2014.
- Premio Gili Agostinelli for Biological and Medical Sciences from the Accademia delle Scienze di Torino in 2014.
- Honorary doctorate from the Vrije University, Brussel, in 2015.
- Outstanding Achievement Award from the European Society of Gene and Cell Therapy in 2015.
- The Jimenez Diaz Prize, Conchita Rábago de Jiménez Díaz Foundation, Madrid, in 2016.
- Premio Capitani 2016 , Milan, Italy
- The Beutler Prize from the American Society of Hematology (ASH), USA, in 2017
- The Louis-Jeantet Prize for Medicine, Lausanne, in 2019
- The Global Health Pioneer Award, Dubai, in 2019
- Nominated "Grande Ufficiale" dell'Ordine "Al Merito della Repubblica Italiana", one of the highest ranking honor in Italy, from the President of the Republic and the Prime Minister of Italy , on December 27th 2019
- Elected "Socio Corrispondente - Classe di Scienze Fisiche, Matematiche e Naturali" at the "Accademia Nazionale dei Lincei", the oldest Scientific Academy in the world, on July 26th, 2022
- Phacilitate Advanced Therapies Lifetime Achievement Award, Miami, USA, in 2024

SCIENTIFIC ACTIVITY

Luigi Naldini has published 305 papers in international peer-reviewed scientific journals.
Full list available at: https://www.ncbi.nlm.nih.gov/pubmed/?term=Naldini+Luigi
Total Impact Factor (I.F.) 2,089 based on Journals I.F. 2015, with average I.F. 10.93 per paper.
As of march 2024, his papers have been cited **49,650** times since 1996. **Scopus *h*-index: 108** as available at https://www.scopus.com/authid/detail.uri?authorId=7005494915
ORCID ID: https://orcid.org/0000-0002-7835-527X

Invited speaker or lecturer to more than 200 International Meetings, Workshops or Universities in the last 10 years. Keynote speaker or main lecture in the Presidential Symposium at several venues, including the Annual Meetings of the American Society of Hematology, European Society of Hematology, American

Society for Blood and Marrow Transplantation, American Society of Gene and Cell Therapy, European Society of Gene and Cell Therapy, EMBO Meetings, Keystone and FASEB Conferences.

In his early career, L. Naldini identified the ligand for the Met receptor with Hepatocyte Growth Factor (HGF), proved its identity with Scatter Factor and elucidated its mechanism of regulation and function in triggering motility and invasion of epithelial cells. *MET* has since been one of the most investigated oncogene in epithelial cancer and metastasis.

During his stay within Inder Verma and Didier Trono laboratories at the Salk Institute for Biological Studies, La Jolla (1994-96), he first described the use of HIV-derived hybrid lentiviral vectors for gene transfer into non-dividing cells. The original paper reporting this work is one of top-cited articles in the journal *Science* (>4,130 citations). He then developed the technology for safe and efficient use working as a senior scientist at Cell Genesys, Foster City, CA. He discussed with the RAC, FDA and EMA the requirements and implications of lentiviral vector administration to humans. Overall, this work laid the foundation for the currently broad use of lentiviral vectors; what was initially received as an elegant proof-of-principle of an unlikely and fearsome technology, has become one of the most widely used tool in biomedical research.

At the end of 1998, L. Naldini returned to academia as professor at the University of Torino and in 2003 moved to the San Raffaele Telethon Institute for Gene Therapy (SR-Tiget) in Milan, initially as co-director with Maria Grazia Roncarolo and since 2008 as director of the Institute. Throughout this time, he has continued to investigate new strategies to overcome the major hurdles to safe and effective gene transfer, bringing about innovative solutions that not only were translated into new therapeutic strategies for genetic disease and cancer but also allowed gaining novel insights into fundamental biological processes such as hematopoietic stem cell function, induction of immunological tolerance and tumor angiogenesis.

Concerning vector development, L. Naldini's work led to improved gene transfer into relevant cell types such as hematopoietic stem cells (HSC). By reaching exhaustive cell marking with minimal interference with cell function, individual HSC activity can now be monitored *in vivo* to unprecedented levels. A boost towards the broad use of lentiviral vectors came from studies primarily conducted in Naldini's laboratory showing that the advanced design of lentiviral vectors is associated with much lower genotoxicity than conventional gamma-retroviral vectors, thus providing for a safer gene transfer platform despite the original concerns raised by the nature of the parental virus. The demonstration of high gene transfer efficiency coupled with improved safety provided by these pre-clinical studies was crucial for moving lentiviral vectors to clinical testing. Gratifyingly, these predictions have now been verified in a growing number of clinical studies, as mentioned below.

By tracking the hematopoietic cell contribution to angiogenesis, Naldini's work established a novel paradigm in which the bone marrow contributes essential paracrine regulators to the newly formed vessels. These studies helped define a subset of proangiogenic monocytes, which selectively engage in tissue remodeling and regeneration and are recruited and contribute to tumor growth. Naldini and his collaborators are now exploiting these findings to develop a new therapeutic strategy by which the monocyte progeny of transplanted hematopoietic progenitors is engineered to selectively target immune stimulatory cytokines, such as interferon-alpha by gene therapy to tumors, thus enhancing therapeutic efficacy and avoiding systemic toxicity. A clinical trial for the first-in-human testing of this strategy opened at the beginning of 2019 for the treatment of glioblastoma multiforme sponsored by a biotech company spin-off co-founded by Luigi Naldini and the San Raffaele Scientific Institute. Current findings from this phase I/II dose-escalating study show tolerability and safety of the strategy, with dose-dependent engraftment of gene-marked cells without dose-limiting toxicity up to the highest dose tested, evidence of targeted interferon activity in the tumor microenvironment and preliminary indication of efficacy.

In another development, Naldini's research applied microRNA regulation to vector design and provided the prototype for making transgenes and medically used viruses stringently responsive to cell type- and differentiation-specific cues. By using this innovative approach, Naldini's team could overcome the immunological barrier to stable gene transfer, one of the major hurdles to successful gene therapy, establish long-term correction of hemophilia in small and large animal models and induce active tolerance to the transgene. Follow-up work performed in collaboration with industry is progressing these studies towards clinical translation of a new gene therapy treatment for hemophilia. The strategy of microRNA regulation is now widely exploited to develop safer vectors, oncolytic viruses and viral vaccines.

In collaboration with John Dick's group, L. Naldini identified microRNAs with specific activity in HSC, showed that miR-126 sets a threshold for HSC activation and governs HSC pool size, and contribute to key pathogenic features of leukemia initiating cells when aberrantly expressed. The expression pattern of miR-126 was then exploited to design vectors transcriptionally silent in HSC but active in their mature progeny.

Naldini's laboratory also pioneered the use of engineered Zinc-finger nucleases to edit the human genome in relevant cells for therapeutic applications. These studies opened the way to *correct*, rather than replace genes, a potentially revolutionary approach that may substantially expand the scope and power of genetic manipulation. Together with Chiara Bonini's group, L. Naldini provided the first proof-of-principle of T-cell receptor genetic editing as a novel means of T-cell therapy, in which a new biological function is instructed to an immune effector cell by genetically re-writing its endogenous antigen specificity. L. Naldini's group also reported the first evidence of successful targeted genome editing in human HSC and its application to correct mutations causing some primary immunodeficiencies in patients' cells and in mouse disease models. Recently, optimization of the editing procedure, also using CRISPR/Cas technology, has allowed achieving substantial levels of targeted gene editing in human long-term repopulating HSC to support further development towards clinical testing. Because of his contribution to this rapidly growing field of studies, L. Naldini was appointed member of several international study committees on Human Gene Editing, which have issued widely received recommendations for the development of this technology in view of its scientific potential medical and ethical implications.

Throughout the years, L. Naldini's efforts towards improving gene therapy have always been pursued with the clear goal in mind of therapeutic translation. Work from his laboratory showed that the post-transplant recruitment of hematopoietic cells to the resident microglia pool could be exploited to deliver gene therapy to the central and peripheral nervous system and treat leukodystrophies in the mouse model. Successful clinical testing of lentiviral vectors in HSC gene therapy was first reported in 2009 by a French team led by Patrick Aubourg to treat adrenoleukodystrophy (ALD), using the vector platform previously developed by Naldini and collaborators. Shortly thereafter, a lentiviral vector-based HSC gene therapy trial was launched at SR-Tiget for metachromatic leukodystrophy (MLD), which is invariably lethal and without any effective conventional treatment. Children treated before or early after symptoms onset are reported at the latest follow-up, reaching up to 13 years, in good conditions and leading a normal or near normal life, whereas they would have already succumbed to the disease if left untreated. Application of lentiviral vector HSC gene therapy continues to expand, at SR-Tiget and elsewhere in the world, to treat patients with immunodeficiencies, storage diseases and hemoglobinopathies like thalassemia, again showing excellent safety and clinical improvements reaching up to full transfusion independence. Overall, >140 patients have been treated at SR-Tiget in Milan and >450 worldwide, with nearly all studies reporting excellent safety and efficacy. Molecular monitoring of the patients in these trials show extensive and stable genetic engineering of human hematopoiesis, with highly polyclonal reconstitution and none or only sporadic indication of potential vector genotoxicity, consistently with the advanced engineering of the vector design and the preclinical predictions from experimental models. Moreover, these studies allow unprecedented insights into the clonal dynamics of human hematopoiesis, providing the first glimpses of HSC activity in living humans.

Based on the pioneering work of SR-Tiget in the clinical development of early generation HSC gene therapy for Adenosine Deaminase Deficiency (ADA-SCID) and the leadership provided in pursuing a new generation of vectors based on lentiviruses, SR-Tiget entered in 2010 in a strategic alliance with GlaxoSmithKline (later transferred to Orchard Therapeutics) to support further clinical development and market access of these therapies and make HSC gene therapy a clinical reality. This first-of-its-kind agreement between a major pharmaceutical company and an academic center engaged in gene therapy highlighted a road map for many more such alliances to come in recent years and was credited in 2016 by the successful registration in EU of the first ex vivo gene therapy product worldwide, Strimvelis, and in 2020 of the lentiviral gene therapy for MLD in EU (Libmeldy) and in USA (Lenmeldy, 2024).

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Original Research Articles

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List of researchers who trained under Luigi Naldini

Ph.D. students	*Years*
Michele De Palma	2000-2004
Mary A. Venneri	2001-2005
Francesca R. Santoni de Sio	2003-2006
Mario Amendola	2003-2007
Angelo L. Lombardo	2004-2011
Ilaria Visigalli	2005-2009
Daniela Cesana	2006-2010
Ferdinando Pucci	2007-2011
Marco Ranzani	2008-2012
Erika Zonari	2008-2012
Alessio Cantore	2008-2012
Pietro Genovese	2009-2013
Francesco Boccalatte	2009-2014
Alice Giustacchini	2010-2013
Claudia Firrito	2012-2016
Giulia Escobar	2012-2016
Giulia Schiroli	2013-2017
Michela Milani	2015-2019
Samuele Ferrari	2016-2020
Aurelien Jacob	2016-2020
Valentina Vavassori	2017-2020
Maura Manzi	2017-2020
Filippo Birocchi	2017-2020
Thomas Kerzel	2018-2022
Daniele Canarutto	2019-2023
Federico Rossari	2019-2023
Martina Fiumara	2020-2023
Gabriele Pedrazzani	2020-2023
Marco Notaro	2020-2024
Chiara Bresestri	2020-2024
Alessandra Weber	2022-

Post-Doctoral Fellows	*Years*	*Current Position*
Laurie Ailles, Ph.D	1998-2001	Research Scientist, Weissman's Lab, Stanford University, CA; (since 2008) Scientist, Ontario Cancer Institute, Toronto, CA
Antonia Follenzi, M.D.	1998-2003	ESCGT Young Investigator Award 2008; Scientist, Gupta's Lab, Albert Einstein College of Medicine, NY; Full Professor, University of Piemonte Orientale, Novara, IT
Elisa Vigna, Ph.D.	1998-2003	Scientist, Institute for Cancer Research and Cure (IRCC), Candiolo, Torino, IT

Brian D. Brown, Ph.D.	2003-2007	Full Professor, Mount Sinai School of Medicine, New York, USA (2018); Assoc. Director of Mount Sinai's Precision Immunology Institute and acting Director of the Icahn Genomics Institute (IGI)
Michele De Palma, Ph.D.	2005-2006	ESCGT Young Investigator Award 2007 Group Leader, San Raffaele Scientific Institute, Milan, IT; Associate Professor, School of Life Sciences and Executive Director, Agora Cancer Research Center, Swiss Federal Institute of Technology Lausanne (EPFL), CH
Alessandra Biffi, M.D.	2003-2006	ASGCT Outstanding New Investigator Award 2010; Director, Gene Therapy Program, Associate Professor, Harvard Medical School, Boston, MA, and Full Professor, University of Padova, IT
Eugenio Montini, Ph.D.	2003-2007	ESCGT Young Investigator Award 2008 ASGCT Outstanding New Investigator Award 2015; Group Leader, San Raffaele Scientific Institute, Milan, IT
Mary Anna Venneri, Ph.D.	2005-2009	Full Professor, University of Rome La Sapienza
Roberta Mazzieri, Ph.D.	2006-2012	Senior Research Fellow, University of Queensland, Diamantina Research Institute, Brisbane, AU
Mario Amendola	2007-2010	Post-doctoral fellow, Netherlands Cancer Institute, Amsterdam NL; Group Leader, INSERM, Genethon Institute, Paris, FR and Associate Professor, University of Foggia, IT
Francesca Santoni de Sio	2003-2007	Scientist, San Raffaele Scientific Inst., Milan, IT
Angelo L. Lombardo	2011-2013	ESCGT Young Investigator Award 2011; (since 2013) Group Leader, SR-Tiget, Milan, IT and Associate Professor, San Raffaele University (2021)
Bernhard Gentner, M.D.	2006-2015	ESCGT Young Investigator Award 2011; (since 2015) Haematologist and Group Leader, SR-Tiget, Milan, IT; (since 2022) Assoc. Professor and Group leader, Oncology Dept., University of Lausanne and Ludwig Institute for Cancer Research, Lausanne, CH, Lausanne
Alice Giustacchini	2014-2015	Post-doctoral fellow, Oxford University, UK and (since 2019) Lecturer, University College London, UK; (since 2023) Group Leader Human Technopole, Milan, IT

Alessio Cantore	2013-2015	ESCGT Young Investigator Award 2018; Assistant Professor, San Raffaele University and (since 2020) Group Leader, SR-Tiget, Milan, IT
Pietro Genovese	2014-2015	ESCGT Young Investigator Award 2016; (since 2019) Assistant Professor, Harvard Medical School, Boston, MA; ASGCT Outstanding New Investigator Award 2024
Francesco Boccalatte	2014-2015	Post-doctoral fellow, New York University, NY; (since 2024) Group Leader, IRCC, Candiolo, IT
Giulia Escobar	2016-2017	Post-doctoral fellow, Brigham and Women's Hospital; (since 2023) Director of Preclinical Research, Massachusetts General Hospital and Instructor, Harvard Medical School, MA
Giulia Schiroli	2017-2018	Post-doctoral fellow, Harvard University, MA; (since 2024) Assoc. Director, Tessera Therapeutics, Boston, MA
Michela Milani	2019 -	Post-doctoral fellow, SR-Tiget, Milan, IT
Attya Omer	2019 -	Post-doctoral fellow, SR-Tiget, Milan, IT
Samuele Ferrari	2020 -	Post-doctoral fellow, SR-Tiget, Milan, IT; ESGCT Young Investigator Award 2023; (since 2024) Project Leader, SR-Tiget
Filippo Birocchi	2020 – 2022	Post-doctoral fellow, SR-Tiget, Milan, IT; (since 2022) Post-doctoral fellow, Massachusetts General Hospital, Boston, MA
Valentina Vavassori	2020 - 2023	Post-doctoral fellow, SR-Tiget, Milan, IT
Aurelien Jacob	2020 - 2023	Group Leader of Innovation Viral Vectors Systems, Genethon, FR