
BIOGRAPHICAL SKETCH

NAME	POSITION TITLE
PIETRO GENOVESE, Ph.D.	Project Leader, San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), Milan, Italy

Web page: <http://www.univr.it/ddr-internazionale-in-medicina-molecolare/pietro-genovese/>

EDUCATION/TRAINING

INSTITUTION	DEGREE	YEAR(s)	FIELD OF STUDY
University of Modena and Reggio Emilia	B.Sc. (Summa cum Laude)	2005	Hematopoiesis
“Vita Salute San Raffaele” University	M.Sc. (Summa cum Laude)	2008	Site-Specific Genome editing
“Vita Salute San Raffaele” University	Ph.D.	2013	Gene Editing for Adoptive Immunotherapy

EMPLOYMENT AND EXPERIENCE

- 2004-05 *Lab training* at the laboratory of Molecular Oncology, coordinated by Prof. Bruno Calabretta at University of Modena and Reggio Emilia, Dept. of Biomedical Sciences, Sect. of General Pathology.
- 2005 *Thesis Internship* at the laboratory of Molecular Biology and Gene Therapy, coordinated by Prof. Fulvio Mavilio, at University of Modena and Reggio Emilia, Dept. of Biomedical Sciences, Sect. of Biological Chemistry.
- 2005-08 *Internship* at the laboratory of Molecular and Cell Biology of Gene Transfer, of Prof. Luigi Naldini, at the San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET), Milan.
- 2008-09 *Fellowship* at the laboratory of Molecular and Cell Biology of Gene Transfer, of Prof. Luigi Naldini, at the HSR-TIGET, Milan.
- 2009-13 *Ph.D. student* at the laboratory of Molecular and Cell Biology of Gene Transfer, of Prof. Luigi Naldini, at the HSR-TIGET Milan.
- 2013-16 *Post-doctoral fellow* at the laboratory of Molecular and Cell Biology of Gene Transfer, of Prof. Luigi Naldini, at the SR-TIGET Milan.
- Since 2016 *Project Leader*, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy
Principal Investigator (PI) of the Grant “Giovani Ricercatori 2013” (GR-2013-02358956) and of the SR-Tiget Core Grant 2016-Project E4 (Telethon).
Co-Principal Investigator of the SR-Tiget Core Grant 2016-Project E1 (Telethon).

PROFESSIONAL ACTIVITIES

Scientific Memberships

Associate Member of the American Society of Gene and Cell Therapy (from 2009 to now), of the European Society of Gene and Cell Therapy (from 2009 to now), and of the American Association for Cancer Research (in 2010).

Scientific reviewer

Served as Ad hoc reviewer for international grant applications (DEBRA International, Vienna; French National Research Agency, ANR) and contributed to the revision of scientific manuscripts for Nature journals.

Academic Mentoring

Currently Mentoring 2 Ph.D students (International Graduate School in Molecular Medicine, "Vita Salute San Raffaele" University; Translational and Molecular Medicine Program, "Milano Bicocca" University) and 2 M.Sc. students ("Vita Salute San Raffaele" University).

Mentor of two M.Sc. and a B.Sc. students (Medical Biotechnology, "Vita Salute San Raffaele" University and School of Pharmacy, University of Groningen) to successful completion of their studies and defense of the thesis, all to top grades. Supervised more than 4 undergraduate M.D. and M.Sc. students. Served as external member of the examiners panel for a Ph.D. Thesis defense at the Universidad Autónoma de Madrid (2015).

HONORS

Awards

Recipient of the "2016 Young Investigator Award" from the European Society of Gene and Cell Therapy (ESGCT) for the considerable contribution to the advancement of the field ([link](#)). Lecture of the Award at the ESGCT/ISSCR/ABCD COLLABORATIVE CONGRESS - October 2016, Florence, Italy.

Recipient of the "Nicolò Copernico Award for Biomedical Science" from the Promoting Committee of the Awards «Giulio Natta and Nicolò Copernico» for the Scientific Research and Technology Innovation of Ferrara granted to an Italian young researcher that published an article in a Scientific Journal with an impact factor over 10, December 2014 ([link](#)).

Recipient of the "Cecilia Cioffrese award" from the Fondazione Carlo Erba for the best research followed by Italian young graduates on the field of cancer, December 2014.

Recipient of the "Van Bekkum award" from the European Society of Bone Marrow Transplantation (EBMT) for the best abstract submitted to the physician's program at the EBMT annual congress, April 2012.

Recipient of the "Jon Van Rood Award" from the European Federation for Immunogenetics (EFI) for Best Abstract submitted to the EFI annual congress, 2012.

Recipient of the "Leslie Fairbairn Runner Up Award" from the PERSIST European Research Consortium, January 2010.

Recipient of four Meritorious Travel Grant Award from the American Society of Gene and Cell Therapy, May 2010, May 2012, May 2013 and May 2014.

Recipient of three Meritorious Travel Grant Award from the European Society of Gene and Cell Therapy, November 2011, October 2013, October 2014.

Invited Speaker or Lecturer

- 2nd Congress of Gene Therapy and Regenerative Medicine – May 2017, Athens, Greece.
- FUTURE MEDICINE / Innovation in Health Sciences – November 2016, Berlin, Germany.
- Young Investigator Award Lecture at the ESGCT/ISSCR/ABCD COLLABORATIVE CONGRESS - October 2016, Florence, Italy.
- 5th International Conference of the Cyprus Society of Human Genetics – October 2016, Cyprus.
- 1st Annual Symposium on Cell and Gene Therapy - Christian Medical College Campus, August 2016, Vellore, India.
- Genome Editing Science Club - Giannina Gaslini Institute, June 2016, Genoa, Italy.
- Annual Meeting of the French society of cellular and gene therapy (SFTCG) - March 2016, Marseilles, France.
- 18th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) - May 2015, New Orleans, USA.
- Genome Editing Symposium - GlaxoSmithKline, January 2015, Stevenage, UK.

Peer-reviewed abstracts

Presentation of 16 proffered abstracts at international meeting including the Annual Meetings of the American Society of Gene and Cell Therapy (ASGCT), the European Society of Gene and Cell Therapy (ESGCT), the CELL-PID & PERSIST, the American Association for Cancer Research (AACR), San Raffaele Scientific Retreat, Telethon Tri-retreat.

Financial Support

Recipient of the 3 year Grant “Giovani Ricercatori 2013” (GR-2013-02358956), sponsored by the Italian Minister of Health. Principal Investigator (PI) of the SR-Tiget Core Grant 2016-Project E4 (Telethon). Co-Principal Investigator of the SR-Tiget Core Grant 2016-Project E1 (Telethon).

PATENTS

Bonini, M.C., Genovese, P., Gregory, P.D., Holmes, M.C., Naldini, L., Paschon, D., Provasi, E., and Zhang, L.. Targeted disruption of T cell receptor genes using engineered zinc finger protein nucleases. [US20110158957 A1](#); Filed in November 2009, granted in June 2011 (Patent # US8956828).

Bonini M.C., Naldini, L., Genovese, P., Mastaglio, S., Magnani, Z., Holmes, M.C., Gregory, P.D., Paschon, D., Zhang, L.. Targeted disruption of T cell receptor genes using engineered zinc finger protein nucleases. [WO2014153470](#); Provisional application filed on March 2013.

Genovese, P., Gregory, P.D., Holmes, M.C., Lombardo, A., Naldini, L.. Delivery methods and compositions for nuclease-mediated genome engineering in hematopoietic stem cells. [US20150174169A1](#) and [WO2015057976](#); Provisional application filed on October 2014.

SCIENTIFIC ACTIVITIES

Dr. Genovese main research interest is to exploit gene-engineering technologies to study biological functions and solve problems with a direct impact on human health. During his academic studies, he matured his scientific interests working on innovative projects aimed to

develop a new gene editing technology that allows site-specific manipulation of the human genome. In 2007, within the group of Prof. Luigi Naldini and in collaboration with Dr. Angelo Lombardo, Dr. Genovese contributed to a break-through work in which they demonstrated for the first time the power of the artificial nucleases technology to direct the integration of exogenous DNA sequences into a predetermined genomic locus of several human cell types. During his Ph.D. studies, he extended his knowledge and skills on this technology exploiting it for the development of a new cancer adoptive immunotherapy approach. In collaboration with the group of Prof. Chiara Bonini, they used artificial nucleases to genetically re-write the endogenous antigen specificity of cytotoxic T cells and re-direct them against a tumor-associated antigen. More recently, Dr. Genovese engaged an ambitious study aimed to develop an effective gene targeting strategy for the correction of inherited mutations in human hematopoietic stem cells (HSC). Here he coordinated a work team of 3 scientists with the aim to overcome the biologic barriers that specifically constrain gene targeting in the most primitive, and clinically relevant, subset of hematopoietic progenitors. By tailoring culture conditions and gene delivery vehicles they developed a protocol that allows targeted integration of a transgene expression cassette into a “safe harbor” site or direct correction of the endogenous *IL2RG* gene of HSCs from healthy donors and X-linked severe combined immunodeficiency (SCID-X1) patients. Currently, Dr. Genovese focused his research on the pre-clinical development and proof of feasibility of these advanced genetic engineering strategies on HSC or T lymphocytes with the aim to develop new treatment for some human diseases, chosen as paradigmatic for testing the therapeutic potential of this approach. Through a recently established collaboration with Editas Medicine, Dr. Genovese is also investigating a pipeline of CRISPR/Cas-based nuclease reagents with the final aim to boost the translation of these breakthrough new technologies into innovative applications for the treatment of several additional clinical conditions.

SCIENTIFIC PRODUCTION

Full list of publications available at:

<http://www.ncbi.nlm.nih.gov/pubmed/?term=Pietro+genovese>

Total Impact Factor: 217.21

H index: 8 (<http://www.scopus.com/authid/detail.url?authorId=22957867100>)

Advanced Manuscripts

Towards Clinical Translation of Hematopoietic Stem Cell Gene Editing for the Correction of Inherited Mutations G Schirotti, S Ferrari, A Jacob, A Conway, L Albano, V Capo, MC Castiello, DY Guschin, T Di Tomaso, E Giuliani, C Bovolenta, GJ Cost, A Villa, G Sitia, MC Holmes, A Lombardo, P Genovese* and Luigi Naldini*.

Targeted Gene Addition in CD34+ Cells from Healthy Donors and Fanconi Anemia Patients. Diez B, Genovese P, Schirotti G, Alvarez L, Román-Rodríguez F.J, Gregory P, Holmes M, Lombardo A, Naldini L, Rio P, Bueren J.A. Manuscript in submitted.

A clinically feasible protocol of TCR gene editing to treat Multiple Myeloma without inducing GvHD. S. Mastaglio, P. Genovese, Z. Magnani, B. Camisa, E. Landoni, G. Schirotti, E. Provasi, A. Lombardo, A. Reik, N. Cieri, M. Ponzoni, F. Ciceri, C. Bordignon, M. Holmes, P. Gregory, L. Naldini, C. Bonini. Manuscript under revision.

Targeted Correction and Reprogramming of SCID-X1 Fibroblasts Rescues IL2RG in iPSC-derived T-lymphoid Cells. Firrito C, Genovese P, Schirotti G, Biffi M, Di Tomaso T, Quintana-Bustamante O, Gennery AR, Gregory PD, Segovia JC, Bueren JA, Lombardo A, Naldini L. Manuscript under revision.

Original Research Articles

1. [Targeted genome editing in human repopulating haematopoietic stem cells](#). Genovese P, Schirotti G, Escobar G, Di Tomaso T, Firrito C, Calabria A, Moi D, Mazziere R, Bonini C, Holmes MC, Gregory PD, van der Burg M, Gentner B, Montini E, Lombardo A, Naldini L. **Nature**. 2014 Jun 12;510(7504):235-40. doi: 10.1038/nature13420. Epub 2014 May 28. PMID: 24870228. 2015 Impact Factor: 38.138

Featured in:

News & Views: Fischer A. *Nature*. 2014. 12;510(7504):226-7
Research Highlights: Koch L. *Nature Reviews Genetics*. 2014. 15, 442-442

2. [Cytokine-Induced Killer \(CIK\) cells engineered with exogenous T cell receptors directed against melanoma antigens: enhanced efficacy of effector cells endowed with a double mechanism of tumor recognition](#). Elia A, Circosta P, Sangiolo D, Bonini C, Gammaitoni L, Mastaglio S, Genovese P, Geuna M, Avolio F, Inghirami G, Tarella C, Cignetti A. **Hum Gene Therapy**. 2015 Mar 10. [Epub ahead of print] PMID: 25758764. 2015 Impact Factor: 4.062
3. [Targeted gene therapy and cell reprogramming in Fanconi anemia](#). Rio P, Baños R, Lombardo A, Quintana-Bustamante O, Alvarez L, Garate Z, Genovese P, Almarza E, Valeri A, Díez B, Navarro S, Torres Y, Trujillo JP, Murillas R, Segovia JC, Samper E, Surrallés J, Gregory PD, Holmes MC, Naldini L, Bueren JA. **EMBO Molecular Medicine**, 2014 May 23;6(6):835-48. doi: 10.15252/emmm.201303374. PMID: 24859981. 2015 Impact Factor: 9.547
4. [CD44v6-targeted T cells mediate potent antitumor effects against acute myeloid leukemia and multiple myeloma](#). Casucci M, Nicolis di Robilant B, Falcone L, Camisa B, Norelli M, Genovese P, Gentner B, Gullotta F, Ponzoni M, Bernardi M, Marcatti M, Saudemont A, Bordignon C, Savoldo B, Ciceri F, Naldini L, Dotti G, Bonini C, Bondanza A. **Blood**. 2013 Nov 14;122(20):3461-72. doi: 10.1182/blood-2013-04-493361. Epub 2013 Sep 9. PMID: 24016461. 2015 Impact Factor: 11.841
5. [Editing T cell specificity towards leukemia by zinc finger nucleases and lentiviral gene transfer](#). Provasi E*, Genovese P*, Lombardo A, Magnani Z, Liu PQ, Reik A, Chu V, Paschon DE, Zhang L, Kuball J, Camisa B, Bondanza A, Casorati G, Ponzoni M, Ciceri F, Bordignon C, Greenberg PD, Holmes MC, Gregory PD, Naldini L, Bonini C. *Equal contribution. **Nature Medicine**, 2012 May;18(5):807-15. doi: 10.1038/nm.2700. PMID: 22466705. 2015 Impact Factor: 30.357

Featured in:

Research Highlights: Kreisberg J. *Nature Biotechnology*. 2012 May; 30(5):411

6. [Site-specific integration and tailoring of cassette design for sustainable gene transfer](#). Lombardo A, Cesana D*, Genovese P*, Di Stefano B*, Provasi E*, Colombo DF*, Neri M, Magnani Z, Cantore A, Lo Riso P, Damo M, Pello OM, Holmes MC, Gregory PD, Gritti A, Broccoli V, Bonini C, Naldini L. *Equal contribution. **Nature Methods**, 2011 Aug 21;8(10):861-9. doi: 10.1038/nmeth.1674. PMID: 21857672. 2014 Impact Factor: 25.328

Featured in:

Perspective: Sadelain M, Papapetrou EP, Bushman FD. *Nature Reviews in Cancer*. 2011. Dec 1;12:51-8

7. [An unbiased genome-wide analysis of zinc-finger nuclease specificity](#). Gabriel R, Lombardo A, Arens A, Miller JC, Genovese P, Kaeppl C, Nowrouzi A, Bartholomae CC, Wang J, Friedman G, Holmes MC, Gregory PD, Glimm H, Schmidt M, Naldini L, von Kalle C. **Nature Biotechnology**, 2011 Aug 7;29(9):816-23. doi: 10.1038/nbt.1948. PMID:21822255. 2014 Impact Factor: 43.113
8. [Hepatocyte-targeted expression by integrase-defective lentiviral vectors induces antigen-specific tolerance in mice with low genotoxic risk](#). Mátrai J, Cantore A, Bartholomae CC, Annoni A, Wang W, Acosta-Sanchez A, Samara-Kuko E, De Waele L, Ma L, Genovese P, Damo M, Arens A, Goudy K, Nichols TC, von Kalle C, L Chuah MK, Roncarolo MG, Schmidt M, Vandendriessche T, Naldini L. **Hepatology**. 2011 May;53(5):1696-707. doi: 10.1002/hep.24230. PMID: 21520180. 2014 Impact Factor: 11.711
9. [Gene editing in human stem cells using zinc finger nucleases and integrase-defective lentiviral vector delivery](#). Lombardo A, Genovese P, Beausejour CM, Colleoni S, Lee YL, Kim KA, Ando D, Urnov FD, Galli C, Gregory PD, Holmes MC, Naldini L. **Nature Biotechnology**, 2007 Nov;25(11):1298-306. Epub 2007 Oct 28. PMID: 17965707. 2014 Impact Factor: 43.113

Featured in:

Preview: Ptaszek L.M., Cowan C.A. *Cell Stem Cell*. 1 (6), pp. 600-602; 2007
Research Highlight: Flintoft L. *Nature Reviews in Genetics*. 2007 Dec 8, 908-909