

Daniele Canarutto

Apr 2021 - today **Paediatrician.** *Pediatric Immunohematology Unit and Bone Marrow Transplantation Program, Ospedale S. Raffaele, Milan*

Feb 2023 - today **Research Fellow (Assegnista di ricerca)** *Vita San Raffaele University and SR-TIGET - Novel Gene Therapy Strategies Unit, Milan.* Co-PI and project coordinator of TIGET EDI-T-HIGM1, a phase I/II FIH clinical study to evaluate the use of a CD40LG gene edited T-cells in patients with Hyper IgM 1, a combined primary immunodeficiency.

Previously

Feb 2023 - Oct 2024 Research fellow *Fondazione Telethon*

Education

2023 **PhD in Molecular Medicine**, Vita-Salute S. Raffaele University
2020 **Specialty in Paediatrics** (70/70 cum laude), Vita-Salute S. Raffaele University
2015 **Diploma supplement** (100/100), School of Advanced Studies Sant'Anna, Pisa
2014 **Medical Degree** (110/110 cum laude), University of Pisa

Skills

C2 English 2014 IELTS (8,5/9), 2017 Cambridge Certificate of Proficiency in English (A) **C1 German** 2008 Deutsches Sprachdiplom der Kultusministerkonferenz Zweite Stufe (85/96) **B1 French** B1 2012 DELF (96,5/100)

2023 Grant writing. Communication of scientific research
2022 GCP training
2021 European Paediatric Advanced Life Support

Publications

 0000-0001-6999-8479

- 2025 Molteni et al. “Mechanisms of hematopoietic clonal dominance in VEXAS syndrome” *Nature Medicine* 10.1038/s41591-025-03623-9
- 2024 Ferrari S. and Canarutto D. “Gene Editing Approaches for Haematological Disorders” in Rezaei, N. (Ed.), *Comprehensive Hematology and Stem Cell Research* vol. 5, pp. 372–395. US: Elsevier 10.1016/B978-0-443-15717-2.00053-6
- Calabria A., [...] Canarutto D. et al. “Long-term lineage commitment in hematopoietic stem cell gene therapy” *Nature* 10.1038/s41586-024-08250-x
- Consiglieri G., [...] Canarutto D. et al. “Early skeletal outcomes after hematopoietic stem and progenitor cell gene therapy for Hurler syndrome” *Science Translational Medicine* 10.1126/scitranslmed.adi8214
- Castiello MC., [...] Canarutto D. et al. “Exonic knockout and knockin gene editing in hematopoietic stem and progenitor cells rescues RAG1 immunodeficiency” *Science Translational Medicine* 10.1126/scitranslmed.adh8162
- 2023 Canarutto D, et al. “Unbiased assessment of genome integrity and purging of adverse outcomes at the target locus upon editing of CD4+ T-cells for the treatment of Hyper IgM1” *The EMBO Journal* 10.15252/embj.2023114188
- Asperti C*, Canarutto D*, et al. “Scalable GMP-compliant gene correction of CD4+ T cells with IDLV template functionally validated in vitro and in vivo”, *Molecular Therapy Methods and Clinical Development* 10.1016/j.omtm.2023.08.020
- Canarutto D*, Omer AJ*, et al. “Mobilization-based engraftment of haematopoietic stem cells: a new perspective for chemotherapy-free gene therapy and transplantation”, *British Medical Bulletin* 10.1093/bmb/ldado17
- Canarutto D*, Oltolini C*, et al. “Outcome of BCG Vaccination in ADA-SCID Patients: A 12-Patient Series”, *Biomedicines* 10.3390/biomedicines11071809
- Fiumara F*, Ferrari S*, [...] Canarutto D et al. “Genotoxic effects of base and prime editing in human hematopoietic stem cells”, *Nature Biotechnology* 10.1038/s41587-023-01915-4
- Frontino G*, Di Tonno R*, Sancampiano MR*, Arrigoni F*, [...], Canarutto D. et al. “Paediatric Wolfram syndrome Type 1: should gonadal dysfunction be part of the diagnostic criteria?”, *Frontiers in Endocrinology* 10.3389/fendo.2023.1155644

- 2022 Omer-Javed, A. [...], Canarutto D., and Naldini L. "Mobilization-based chemotherapy-free engraftment of gene-edited human hematopoietic stem cells", *Cell* 10.1016/j.cell.2022.04.039
- Barzaghi F., Cicalese MP, [...] Canarutto D. et al. "Case Report: Consistent disease manifestations with a staggered time course in two identical twins affected by adenosine deaminase 2 deficiency", *Frontiers in Immunology* 10.3389/fimmu.2022.910021
- Ferrari S*, Jacob, A*, Cesana, D*, [...], Canarutto D. et al. "Choice of Template Delivery Mitigates the Genotoxic Risk and Adverse Impact of Editing in Human Hematopoietic Stem Cells", *Cell Stem Cell* 10.1016/j.stem.2022.09.001
- 2021 Canarutto D*, Tucci F*, et al. "Peripheral blood stem and progenitor cell collection in pediatric candidates for ex vivo gene therapy: a 10 year series", *Molecular Therapy - Methods & Clinical Development* 10.1016/j.omtm.2021.05.013
- Ferrari S*, Vavassori V*, Canarutto D* et al. "Gene Editing of Hematopoietic Stem Cells: Hopes and Hurdles Toward Clinical Translation", *Frontiers in Genome Editing* 10.3389/fgeed.2021.618378
- Lu, C*, An, X*, [...] Canarutto D. et al. "Pooled Analysis of Gastric Emptying in Patients With Obesity: Implications for Oral Absorption Projection", *Clinical Therapeutics* 10.1016/j.clinthera.2021.08.006
- Frontino G*, Raouf T*, Canarutto D. et al. "Off-Label Liraglutide Use in Children With Wolfram Syndrome Type 1: Extensive Characterization of Four Patients", *Frontiers in Pediatrics* 10.3389/fped.2021.755365
- 2020 Li G.-F., [...] Canarutto D. et al. "Do proton pump inhibitors influence SARS-CoV-2 related outcomes? A meta-analysis", *Gut* 10.1136/gutjnl-2020-323366
- Canarutto D**. et al. "Prolonged asymptomatic SARS-CoV-2 infection in a child receiving immunosuppressive therapy", *Pediatric Pulmonology* 10.1002/ppul.24983
- Del Barba P., Canarutto D. et al. "COVID-19 cardiac involvement in a 38-day old infant", *Pediatric Pulmonology* 10.1002/ppul.24895
- Canarutto D**. et al. "COVID-19 infection in a paucisymptomatic infant: Raising the index of suspicion in epidemic settings", *Pediatric Pulmonology* 10.1002/ppul.24754
- 2019 Tucci F., [...] Canarutto D. et al. "Bone marrow harvesting from paediatric patients undergoing haematopoietic stem cell gene therapy", *Bone Marrow Transplantation* 10.1038/s41409-019-0573-6

Ferrua F., [...] Canarutto D. et al. "Lentiviral haemopoietic stem/progenitor cell gene therapy for treatment of Wiskott-Aldrich syndrome: interim results of a non-randomised, open-label, phase 1/2 clinical study", *Lancet Haematology* 10.1016/s2352-3026(19)30021-3

2018 Peccatori J., Canarutto D. et al. "Complicanze associate al trapianto: ruolo chiave dell'endotelio?", *Current Therapeutics* 8:6-11

Tucci F., [...] Canarutto D. et al. "Successful treatment with Ledipasvir/Sofosbuvir in an ADA-SCID infant with HCV infection allowed gene therapy with Strimvelis®", *Hepatology* 10.1002/hep.30160

2015 Glauber M, Miceli A., Canarutto D. et al. "Early and long-term outcomes of minimally invasive mitral valve surgery through right minithoracotomy: a 10-year experience in 1604 patients." *Journal of Cardiothoracic Surgery*. 10.1186/s13019-015-0390-y

Miceli A., Murzi M., Canarutto D. et al. "Minimally invasive mitral valve repair through right minithoracotomy in the setting of degenerative mitral regurgitation: early outcomes and long-term follow-up." *Annals of Cardiothoracic Surgery*. 10.3978/j.issn.2225-319X.2015.04.10

2014 Lio A., Miceli A., Varone E., Canarutto D., et al. "Mitral valve repair versus replacement in patients with ischaemic mitral regurgitation and depressed ejection fraction: risk factors for early and mid-term mortality", *Interactive CardioVascular and Toracic Surgery* 10.1093/icvts/ivuo66

Oral contributions

INVITED SPEAKER

2024 "Clinical trial of gene edited T cells for CD40L", *Inborn Errors Working Party Annual Conference*, 28-9-2024, Helsinki

"Il ruolo del gene editing e della terapia genica come contributo terapeutico per le malattie genetiche rare", *European Biotech Week*, 24-9-2024, Milano

2023 "Validation of high coverage OGM sensitivity for the detection of genomic rearrangements following HDR gene editing", *Institut Curie Optical Genome Mapping (OGM) Meeting*, 31-5-2023, Paris

2021 “Gene editing for HIGM1”, *Inborn Errors Working Party Annual Conference*, 25-9-2021, Milano

SELECTED ABSTRACT

2025 “Highly efficient and seamless selection of long-range gene-edited HSPCs by targeting haploinsufficient genes”, *ASGCT 28th Congress*, 14-05-2025, New Orleans

“HDR gene correction of CD4+ T cells approaching the clinic for the treatment of Hyper IgM1”, *ASGCT 28th Congress*, 14-05-2025, New Orleans

2024 “Efficient long range gene editing and enrichment of lymphocytes and hematopoietic stem and progenitor cells with the desired editing outcome”, *ESGCT 31st Congress*, 24-10-2024, Rome

“Long-range gene editing of primary cells results in frequent and heterogeneous on-target genomic rearrangements”, *FASEB The Genome Engineering Conference*, 18-6-2024, Rome

“Heterogeneous on-target genomic rearrangements occur upon long-range gene editing in T-cells assessed by PCR-free technologies”, *ASGCT 27th Annual Meeting 2024*, 10-5-2024, Baltimore 10.1016/j.ymthe.2024.04.020

2021 Vavassori V., [...], Canarutto D. et al “Towards Clinical Translation of Hematopoietic Cell Gene Editing for Treating Hyper-IgM Type 1”, *ASH Annual Meeting 2021* 10.1182/blood-2021-148572

Posters and abstracts

2025 Canarutto et al “Proportional but not equal relation between busulfan dose adjustments and resulting exposure variations”, 51st EMBT Congress, 1-4-2025, Florence

2024 Weber A., Canarutto D. et al. “Highly efficient and seamless in vivo selection of long-range gene-edited HSPCs by targeting haploinsufficient genes”, 31st ESGCT Congress, 24-10-2024, Rome

Campochiaro C. [...], Canarutto D. et al. “Unraveling pathophysiology and hematopoiesis of VEXAS syndrome by multi-omics analysis and targeted gene editing”, *Annals of the Rheumatic Diseases* 2024;83:5-6. *EULAR 2024 Congress*, 12/15-6-2024, Vienna 10.1136/annrheumdis-2024-eular.3532

Ferrari S. [...], Canarutto D. et al. "Efficient Ex-Vivo Selection of Gene Edited Hematopoietic Stem Cells by Leveraging on Artificial Transactivators and Lipid Nanoparticle-Mediated Delivery", *ASGCT 27th Annual Meeting 2024*, 10-5-2024, Baltimore.
10.1016/j.ymthe.2024.04.020

Campochiaro C. [...], Canarutto D. et al. "Pervasive Inflammation Poisons Hematopoiesis and Drives Clonal Dominance in VEXAS Syndrome", *American College of Rheumatology Convergence 2024* 17-11-2024, Washington, DC. 10.1002/art.42992

2023 Canarutto D. et al "In-depth genome integrity evaluation and GMP compliant manufacturing of HDR gene edited CD4+ T cells for the treatment of Hyper IgM 1", *EHA 2023 Congress*, 9-6-2023, Frankfurt

Molteni R. [...], Canarutto D. et al. "Unraveling Pathophysiology and Hematopoiesis of Vexas Syndrome By Multi-Omics Analyses and Targeted Gene Editing", *65th Annual Meeting of the American-Society-of-Hematology (ASH)*, 10.1182/blood-2023-179749

2022 Canarutto D. et al. "Safety assessment of gene edited CD4+ T-cells for the treatment of Hyper-IgM1", *ESGCT Congress 2022*, 11-14-10-2022, Edinburgh

Canarutto D. et al. "Tiling the target site with ddPCR probes to quickly assess genome integrity at an edited genomic locus", *FASEB - The genome engineering conference: cutting-edge research and applications*, 26-30-6-2022, Lisbon

Calabria A. [...], Canarutto D. et al. "Adaptive Routes of Hematopoietic Stem Cell Differentiation to Disease Conditions and Age in Gene Therapy Patients", *64th ASH Annual Meeting*, 1-12-2022, New Orleans 10.1182/blood-2022-162195 140 Supplement 1: pag. 1904-1905

Fiumara M.*, Ferrari S.*, Canarutto D.*, et al. "Efficient ex-vivo selection of gene edited human hematopoietic stem/progenitor cells", *FASEB - The genome engineering conference: cutting-edge research and applications*, 26-30-6-2022, Lisbon

Omer-Javed A. [...], Canarutto D. and Naldini L. "Chemotherapy-Free Engraftment of Gene Edited Human Hematopoietic Stem Cells Leveraged on Mobilization and mRNA-Based Engineering", *ASGCT 25th Annual Meeting*, 16-19-5-2022, Washington DC
10.1016/j.ymthe.2022.04.017 pag. 231-232

Jacob A.*, Ferrari S.*, Cesana D.*, [...], Canarutto D. et al. "The Choice of Template Delivery Mitigates the Genotoxic Risk and Adverse Impact of Editing in Human Hematopoietic Stem Cells", *ASGCT 25th Annual Meeting*, 16-19-5-2022, Washington DC
10.1016/j.ymthe.2022.04.017 pag. 5-6

Ferrua F., [...], Canarutto D. et al. "Up to 10.5 Years of Follow-Up in 17 Subjects Treated with Hematopoietic Stem and Progenitor Cell Lentiviral Gene Therapy for Wiskott-Aldrich Syndrome", *ASGCT 25th Annual Meeting*, 16-19-5-2022, Washington DC
10.1016/j.ymthe.2022.04.017 pag. 27-28

2021

Canarutto D. et al "Mobilization and apheresis of haematopoietic stem and progenitor cells in pediatric candidates for gene therapy: a 10-year, 45 patient series", *48° Congress of the Italian Society of Hematology, Milano, Italy, October 24-27, 2021*
10.3324/haematol.2021.s3 pag. 175

Canarutto D. et al. "A 10 year retrospective series of peripheral blood stem and progenitor cell collection in pediatric candidates for autologous ex-vivo gene therapy", *EHA 2021*
10.1097/HS9.0000000000000566 pag. 339

Oltolini C., Canarutto D. et al. "A single-centre experience on Mycobacterium tuberculosis complex infection in children with primary immunodeficiency", *21st ICHS Symposium on Infections in the Immunocompromised Host*

Pellizzoni F. A., Canarutto D. et al. "Se le dimensioni contano: un caso di macrotrombocitopenia", *XXVII Congresso Nazionale Società Italiana di Neonatologia Volume Atti SIN 2021*, pag 179

Vavassori V., [...] Canarutto D. et al. "Towards Clinical Translation of Hematopoietic Cell Gene Editing for Treating Hyper-IgM Type 1", *ASGCT 2021*
10.1016/j.ymthe.2021.04.019 n 38, pag. 19

2019

Ferrua F., [...] Canarutto D. et al. "Lentiviral Hematopoietic Stem and Progenitor Cell Gene Therapy for Wiskott-Aldrich Syndrome (WAS): Up to 8 Years of Follow up in 17 Subjects Treated Since 2010", *American Society of Hematology Annual Meeting*
10.1182/blood-2019-124665

Gallo D., Canarutto D. et al. "Importazioni pericolose", *XXV Congresso Nazionale Società Italiana di Neonatologia P 069 Volume Atti SIN 2019*, pag 74

2018

Canarutto D. et al. "Gestione delle complicanze da vaccino con BCG in pazienti con ADA-SCID e risultati dopo terapia genica", *Congresso AIEOP 2018* 10.4081/hr.2018.7767 Po61 pag. 36

Recupero S., Tucci F, Canarutto D. et al. "Un caso di sclerosi multipla in paziente con piastrinopenia immune cronica", *Congresso AIEOP 2018* 10.4081/hr.2018.7767 Po58 pag. 35

Tucci F., et al. "Espianto di midollo osseo in pazienti pediatrici sottoposti a terapia genica

con cellule staminali ematopoietiche autologhe”, *Congresso AIEOP 2018* 10.4081/hr.2018.7767
Po65 pag. 37

Patents

- 2024 Canarutto D., Ferrari S., Weber A., Naldini L. Ep 24171984.8 Means and methods for safe and efficient gene editing in cells (submitted)
- 2023 Villa A., Naldini L., Ferrari S., Castiello MC., Porcellini S., Canarutto D., WO2023062030A1 Polynucleotides useful for correcting mutations in the Rag1 gene
- Vavassori V., Naldini L., Ferrari S., Canarutto D., Asperti C., Radrizzani M., WO2024252342A1 Genetic modification protocols in immune cells

Grants

- 2023 HyperIgM Foundation Grant
- Responsible for U.O.3 grant GR-2016-02364847 “Exploiting cutting edge technologies to hunt T cell receptors for TCR gene editing of acute myeloid leukemia”

Awards

- 2025 National Scientific qualification (ASN – Abilitazione Scientifica Nazionale) as associate professor for the disciplinary field 06/A1 - Medical genetics
- ASGCT Travel Grant
- EHA congress VIP invitation
- 2024 National Scientific qualification (ASN – Abilitazione Scientifica Nazionale) as associate professor for the disciplinary field 06/G1 - Pediatrics
- Fellowship, Vita-Salute San Raffaele University
- ESGCT Travel Grant
- FASEB Early Career Award
- ASGCT Meritorious abstract travel award
- 2023 EHA Travel Grant & VIP invitation
- 2021 EHA Participation grant & VIP invitation

Memberships

- 2025-today European Bone Marrow Transplant society (EBMT)
- 2024-today American Society for Gene and Cell Therapy (ASGCT)

2022-today	Italian Society for Gene and Cell Therapy (SITGEC)
	European Society for Gene and Cell Therapy (ESGCT)
2021-today	European Hematology Association (EHA)
	Italian Society of pediatrics (SIP)

Communication

2024	Scientific and clinical scripting advisory to televised episode of scientific TV programme “V Dimensione” on CRISPR editing for HIGM1, televised on Rai3 on Jan 4th 2025.
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Peer review

2025	Molecular therapy
2020	BMJ Pediatrics open
	BMC Pediatrics

** co-first ** corresponding author. Last updated June 1, 2025*

Autorizzo il trattamento dei dati personali contenuti nel mio curriculum vitae in base al D. Lgs. 196/2003, coordinato con il D. Lgs. 101/2018, e al Regolamento UE 2016/679. I give consent to process my data in accordance to the Regulation of the European Parliament 679/2016 and D. Lgs. 196/2003