Stanford

Maria Grazia Roncarolo

George D. Smith Professor of Stem Cell and Regenerative Medicine and Professor of Medicine (Blood and Marrow Transplantation and Cellular Therapy)

Pediatrics - Stem Cell Transplantation

NIH Biosketch available Online

CONTACT INFORMATION

- **Administrative Assistant**

  Ginger Exley

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Bio

BIO

Maria Grazia Roncarolo, MD is the George D. Smith Professor in Stem Cell and Regenerative Medicine, Professor of Pediatrics and of Medicine, director of the Center for Definitive and Curative Medicine, and co-director of the Institute for Stem Cell Biology and Regenerative Medicine.

Dr. Roncarolo leads efforts to translate scientific discoveries in genetic diseases and regenerative medicine into novel patient therapies, including treatments based on stem cells and gene therapy.

A pediatric immunologist by training, she earned her medical degree at the University of Turin, Italy. She spent her early career in Lyon, France, where she focused on severe inherited metabolic and immune diseases, including severe combined immunodeficiency (SCID), better known as the "bubble boy disease." Dr. Roncarolo was a key member of the team that carried out the first stem cell transplants given before birth to treat these genetic diseases.

While studying inherited immune diseases, Dr. Roncarolo discovered a new class of T cells. These cells, called T regulatory type 1 cells, help maintain immune system homeostasis by preventing autoimmune diseases and assisting the immune system in tolerating transplanted cells and organs. Dr. Roncarolo completed the first clinical trial using T regulatory type 1 cells to prevent severe graft-versus-host disease in leukemia patients receiving blood-forming stem-cell transplants from donors who were not genetic matches.

Dr. Roncarolo worked for several years at DNAX Research Institute for Molecular and Cellular Biology in Palo Alto, where she contributed to the discovery of novel cytokines, cell-signaling molecules that are part of the immune response. She studied the role of cytokines in inducing immunological tolerance and in promoting stem cell growth and differentiation.

Dr. Roncarolo developed new gene-therapy approaches, which she pursued as director of the Telethon Institute for Cell and Gene Therapy at the San Raffaele Scientific Institute in Milan. She was the principal investigator leading the successful gene therapy trial for SCID patients who lack an enzyme critical to DNA synthesis, which is a severe life-threatening disorder. Based on the results of this trial, gene therapy for ADA-SCID has obtained Orphan drug status from both the FDA and EMEA and it

was licensed to Glaxo Smith Klein, which has received European Commission approval to market under the name of Strimvelis. Under her direction, the San Raffaele Scientific Institute has been seminal in showing the efficacy of gene therapy for otherwise untreatable inherited metabolic diseases and primary immunodeficiencies.

Dr. Roncarolo established the Stanford Center for Definitive and Curative Medicine to cure patients with currently incurable diseases through the development of innovative stem cell-and gene-based therapies.

ACADEMIC APPOINTMENTS

- Professor, Pediatrics - Stem Cell Transplantation
- Professor, Medicine - Blood & Marrow Transplantation
- Member, Bio-X
- Member, Institute for Stem Cell Biology and Regenerative Medicine
- Member, Maternal & Child Health Research Institute (MCHRI)
- Member, Stanford Cancer Institute

ADMINISTRATIVE APPOINTMENTS

- Professor, Departments of Pediatrics and Medicine, Stanford University, (2014- present)
- Director, Stanford Center for Definitive and Curative Medicine (CDCM), (2016-2022)
- Co-Director, Institute for Stem Cell Biology and Regenerative Medicine, Stanford School of Medicine, (2014-2022)
- Division Chief, Pediatric Stem Cell Transplantation and Regenerative Medicine, Stanford School of Medicine, (2014-2019)
- Co-Director, Bass Center for Childhood Cancer and Blood Diseases, Lucile Packard Children's Hospital Stanford, (2014-2019)

HONORS AND AWARDS

- Outstanding Achievement Award, American Society of Gene & Cell Therapy (2017)
- Knighthood "Commendatore dell'Ordine Al Merito della Repubblica Italiana", President of Italy (2014)
- "Gold Apple" Prize for outstanding contribution to science, Marisa Bellisario Foundation (2013)
- Elected Member, Austrian Academy of Sciences (2012)
- Eurordis Scientific Award 2012 for outstanding contributions to the cure of genetic diseases, Eurordis (2012)
- Outstanding Achievement Award for career and pioneering contributions to the field, European Society of Gene and Cell Therapy (2010)
- Elected Member, Academia Europaea of Sciences (2005)
- Nominated "Ufficiale dell'Ordine Al Merito della Repubblica Italiana", President of Italy (2000)

BOARDS, ADVISORY COMMITTEES, PROFESSIONAL ORGANIZATIONS

- Member, Regulatory and Ethics Committee of European Society of Gene and Cell Therapy (ESGCT) (2006 - present)
- Member, Editorial Board for Current Gene Therapy (2006 - present)
- Charter Member, Eureka Institute for Translational Medicine (2008 - present)
- Member, European Group for the Bone and Marrow Transplantation (EBMT) Immunology Working Party (2008 - present)
- Member, Review Editorial Board of Frontiers in Immunological Tolerance (2010 - present)
- Member, Editorial Board for Molecular Therapy: Methods and Clinical Development (2013 - present)
- Member of the Editorial Board, Current Stem Cell Reports (2014 - present)
- Member, Scientific Advisory Board of BC Children's Hospital Research Institute (CHRI) (2014 - present)
- Member, Scientific Advisory Board of Spark Therapeutics (2015 - present)
- Co-Chair, Scientific Advisory Board of Glaxo Smith Kline Cell and Gene Therapy (CGT) (2016 - present)

- Member, Scientific Program Committee of the Federation of Clinical Immunology Societies (FOCIS) Meeting (2016 - present)
- Member, Editorial Board for Science Immunology (2016 - present)
- Member, External Immunology Board of Glaxo Smith Kline Immunology Network (2015 - 2016)
- Member, Scientific Board of the Association “Festival della Scienza” (2013 - 2014)
- Member, Scientific Advisory Board of the French Rare Diseases Foundation (2012 - 2014)
- Member, Scientific Advisory Board of the Global Health Institute (GIH) Lausanne (2011 - 2014)
- Member, Scientific Committee of the European Congress of Immunology (ECI) (2010 - 2012)
- Member, Scientific Committee of the 2nd International Conference on Immune Tolerance (2010 - 2011)
- Member, Organizing Committee of the Federation of Clinical Immunology Societies (FOCIS) Meeting (2010 - 2011)
- Member, Nominating Committee of the American Society of Gene and Cell Therapy (ASGCT) (2010 - 2011)
- Member, American Society of Hematology (ASH) Committee in Immunology and Host Defense (2009 - 2014)
- Member, Scientific Program Committee of the International Congress of Immunology (ICI) (2009 - 2013)
- Member, Organizing and Scientific Committees of the Federation of European Biochemical Societies (FEBS) (2008 - 2011)
- Chair, Immunology of Gene Therapy Committee of the American Society of Gene and Cell Therapy (ASGCT) (2008 - 2009)
- Member, Program Committee of the American Society of Gene and Cell Therapy (ASGCT) (2008 - 2009)
- Member, Editorial Board for Italian Journal of Pediatrics (2007 - 2014)
- Member, Editorial Board for Human Immunology (2007 - 2014)
- Member, External Scientific Advisory Board of the Tumorzentrum L. Heilmeyer Comprehensive Cancer Center Freiburg (CCCF) (2007 - 2012)
- Member, Membership Committee of the American Society of Gene and Cell Therapy (ASGCT) (2005 - 2011)
- Member, Scientific Committee of the European School of Hematology (ESH) (2004 - 2014)
- Member, Immunology of Gene Therapy Committee of the American Society of Gene and Cell Therapy (ASGCT) (2004 - 2010)
- Member, Scientific Advisory Board of the University of Nantes's Institut de transplantation et de recherche en transplantation (ITERT) (2001 - 2009)
- President, Genethon Scientific Advisory Board of the Association Française contre les Myopathies (AFM) (1999 - 2002)
- Member, Scientific Advisory Board of Kinetix Pharmaceutical (1997 - 2000)

PROFESSIONAL EDUCATION

- M.D., University of Turin, Italy , Medicine (1982)
- Natl. Board, University of Turin, Italy , Pediatrics (1986)
- Natl. Board, University of Milan, Italy , Clinical Immunology (1990)

PATENTS

- Manuela Battaglia, Maria Grazia Roncarolo. "United States Patent 8562974 Method for expanding Cd4+ Cd25+ T regulator cells", Fondazione Telethon, Ospedale San Raffaele S.R.L., Oct 22, 2013
- Manuela Battaglia, Maria-Grazia Roncarolo. "United States Patent 1869163 Method for expanding cd4+ cd25+ t regulatory cells", Fondazione Centro San Raffaele Del Monte Tabor, Fondazione Telethon, Sep 11, 2011
- Frank Kolbinger, Herrera José M. Carballido, András Aszodi, José W. Saldanha, Bruce M. Hall, Silvia Gregori, Maria Grazia Roncarolo, Véronique Loux, Gregorio Aversa, Margit Jeschke. "United States Patent 1664122 Therapeutic humanised antibodies against cd45 isoforms", Novartis AG, Novartis Pharma GmbH, Mar 17, 2010
- Manuela Battaglia, Maria-Grazia Roncarolo. "Australia Patent 2006217546 Method for expanding cd4+ cd25+ t regulatory cells", San Raffaele Centro Fond, Fond Telethon, Manuela Battaglia, Maria Grazia Roncarolo, Oct 29, 2009
- Aszodi Andras, Aversa Gregorio, Carballido Herrera Jose M, Gregori Silvia, Hall Bruce M, Jeschke Margit, Kolbinger Frank, Loux Veronique, Roncarolo Maria Grazia, Saldanha Jose W. "Australia Patent 2004272289 Therapeutic binding molecules", Sep 18, 2008
- Megan K. Levings, Rene De Waal Malefyt, Maria Grazia Roncarolo. "United States Patent 6,746,670 Regulatory T cells; methods", Schering Corporation, Jun 8, 2004

- Maria-Grazia Roncarolo, Rene de Waal Malefyt, Rosa Bacchetta, Herve M. Groux, Jan E. de Vries. "United States Patent 6,277,635 Use of interleukin-10 to produce a population of suppressor cells", Schering Corporation, Aug 21, 2001
- Maria-Grazia Roncarolo. "United States Patent 5,879,937 Cytokine-induced proliferation of amniotic t-cells", Schering Corporation, Mar 9, 1999
- Maria-Grazia Roncarolo. "United States Patent 5,405,751 Prenatal diagnosis by cytokine induced proliferation of fetal T cells", Schering Corporation, Apr 11, 1995

LINKS

- Pediatric Division of Stem Cell Transplantation and Regenerative Medicine: http://med.stanford.edu/ptrm.html
- Roncarolo Lab: http://med.stanford.edu/roncarololab.html
- Center for Definitive and Curative Medicine: https://med.stanford.edu/cdcm.html
- Institute for Stem Cell Biology and Regenerative Medicine: http://med.stanford.edu/stemcell.html

Research & Scholarship

CURRENT RESEARCH AND SCHOLARLY INTERESTS

Research Interests

Immunetolerance: Mechanisms underlying T-cell tolerance, induction of T-cell anergy and regulatory T cells; Immunomodulation: mAbs, proteins and low molecular weight compounds which can modulate T-cell activation; Primary immunodeficiencies: Characterization of molecular and immunological defects; Gene therapy: Gene transduction of hematopoietic cells for gene therapy in primary immunodeficiencies and metabolic diseases; Hematopoiesis: Mechanisms underlying growth and differentiation of hematopoietic stem cells; Transplantation: Immune reconstitution and T-cell tolerance after allogenic stem cell transplantation; Cytokines/Cytokine receptors: Role in regulation of immune and inflammatory responses

Clinical Interests

Primary Immunodeficiencies

Monogenic Autoimmune Disorders

Allogenic Bone Marrow Transplantation

Gene Therapy Clinical Trials

Cell Therapy Clinical Trials

Clinical Trials in Autoimmune Diseases and Organ Transplantation

Clinical Trials in Hemoglobinopathies

CLINICAL TRIALS

- CD4^LVFOXP3 in Participants With IPEX, Recruiting
- Stem Cell Transplant From Donors After Alpha Beta Cell Depletion in Children and Adults With T-allo10 Cells Addback, Recruiting

Teaching

STANFORD ADVISEES

Postdoctoral Faculty Sponsor

Allison Boss, Steven Strubbe

Doctoral Dissertation Co-Advisor (AC)

Jason Nideffer

Publications

PUBLICATIONS

- **Identification of unstable regulatory and autoreactive effector T cells that are expanded in patients with FOXP3 mutations.** *Science translational medicine*
  Borna, Š., Lee, E., Nideffer, J., Ramachandran, A., Wang, B., Baker, J., Mavers, M., Lakshmanan, U., Narula, M., Garrett, A. K., Schulze, J., Olek, S., Marois, et al
  2023; 15 (727): eadg6822

- **A novel FOXP3 knockout-humanized mouse model for pre-clinical safety and efficacy evaluation of Treg-like cell products.** *Molecular therapy. Methods & clinical development*
  Sato, Y., Nathan, A., Shipp, S., Wright, J. F., Tate, K. M., Wani, P., Roncarolo, M. G., Bacchetta, R.
  2023; 31: 101150

- **IPEX Syndrome from diagnosis to cure, learning along the way.** *The Journal of allergy and clinical immunology*
  Bacchetta, R., Roncarolo, M. G.
  2023

- **Radiation and Busulfan-Free Hematopoietic Stem Cell Transplantation Using Briquilimab (JSP191) Anti-CD117 Antibody-Conditioning, Transient Immunosuppression and TCR # # + T-Cell/CD19+B-Cell Depleted Haploidentical Grafts in Patients with Fanconi Anemia**
  Agarwal, R., Bertaina, A., Soco, C., Saini, G., Kunte, N., Hiroshima, L., Chan, Y., Willner, H., Krampf, M. L., Nofal, R., Barbarito, G., Sen, S., Felber, et al
  AMER SOC HEMATOLOGY.2023

- **LENTIVIRAL-MEDIATED GENE THERAPY FOR PATIENTS WITH FANCONI ANEMIA [GROUP A]: UPDATED RESULTS FROM GLOBAL RP-L102 CLINICAL TRIALS**
  Sevilla, J., Booth, C., Czechowicz, A., Agarwal, R., Zubicaray, J., Rio, P., Chetty, K., O'Toole, G., Xu-Bayford, J., Ancliff, P., Sebastian, E., Choi, G., Zeini, et al
  SPRINGERNATURE.2023: 276-277

- **T-ALLO10 INFUSION AFTER A.DEPLETED-HSCT IN CHILDREN AND YOUNG ADULTS WITH HEMATOLOGIC MALIGNANCIES: IMPROVED IMMUNE RECONSTITUTION IN THE ABSENCE OF SEVERE GVHD**
  Bertaina, A., Bacchetta, R., Shyr, D., Saini, G., Lee, J., Kristovich, K., Agarwal-Hashmi, R., Klein, O., Melsop, K., Tate, K., Barbarito, G., Oppizzi, L., Chen, et al
  SPRINGERNATURE.2023: 232-234

- **LENTIVIRAL-MEDIATED GENE THERAPY FOR SEVERE PYRUVATE KINASE DEFICIENCY: RESULTS FROM AN ONGOING GLOBAL PHASE 1 STUDY**
  Lopez Lorenzo, J., Shah, A., Sevilla, J., Navarro, S., Llanos, L., de Camino Gaisse, B., Sanchez, S., Zubicaray, J., Glader, B., Chien, M., Quintana Bustamante, O., Zeini, M., Choi, et al
  SPRINGERNATURE.2023: 275-276

- **LENTIVIRAL-MEDIATED GENE THERAPY FOR FANCONI ANEMIA [GROUP A]: RESULTS FROM RP-L102 CLINICAL TRIALS**
  Czechowicz, A., Sevilla, J., Booth, C., Agarwal, R., Zubicaray, J., Rio, P., Navarro, S., Chetty, K., O'Toole, G., Xu-Bayford, J., Ancliff, P., Sebastian, E., Choi, et al
  WILEY.2023: S136-S137

- **LENTIVIRAL-MEDIATED GENE THERAPY FOR SEVERE PYRUVATE KINASE DEFICIENCY: GLOBAL PHASE 1 STUDY RESULTS**
  Shah, A., Lorenzo, J., Sevilla, J., Navarro, S., Llanos, L., Gaisse, B., Sanchez, S., Zubicaray, J., Glader, B., Chien, M., Bustamante, O. Q., Zeini, M., Choi, et al
  WILEY.2023: S133-S134

- **Discovery of Key Transcriptional Regulators of Alloantigen-Inducible Tregs Used for Cell Therapy**
  Cepika, A., Amaya, L., Waichler, C., Narula, M., Thomas, B. C., Chen, P. P., Mantilla, M. M., Pavel-Dinu, M., Freeborn, R., Porteus, M. H., Bacchetta, R., Mueller, F., Greenleaf, et al
  CELL PRESS.2023: 370-371

- **Global Phase 1 Study Results of Lentiviral Mediated Gene Therapy for Severe Pyruvate Kinase Deficiency**
  Shah, A. J., Lopez Lorenzo, J., Sevilla, J., Navarro, S., Llanos, L., de Camino Gaisse, B., Sanchez, S., Zubicaray, J., Glader, B., Chien, M., Quintana Bustamante, O., Zeini, M., Choi, et al
  CELL PRESS.2023: 118-119

- **Lentiviral-Mediated Gene Therapy for Fanconi Anemia [Group A]: Results from Global RP-L102 Clinical Trials**
  Czechowicz, A., Sevilla, J., Booth, C., Navarro, S., Agarwal, R., Zubicaray, J., Rio, P., Chetty, K., O'Toole, G., Xu-Bayford, J., Ancliff, P., Sebastian, E., Choi, et al
  CELL PRESS.2023: 118

- **SATB1 chromatin loops regulate Megakaryocyte/Erythroid Progenitor Expansion by facilitating HSP70 and GATA1 induction.** *Stem cells (Dayton, Ohio)*
  Wilkes, M. C., Chae, H. D., Scanlon, V., Cepika, A. M., Wentworth, E. P., Saxena, M., Eskin, A., Chen, Z., Glader, B., Roncarolo, M. G., Nelson, S. F., Sakamoto, K. M.
  2023

- **Hematopoietic and Immunological Assessment in Fanconi Anemia after Ex Vivo Lentiviral FANCA Gene Therapy with RP-L102**
  Nofal, R., Chan, Y., Sen, S., Figueroa, U., Willner, H., Felber, M., Krampf, M., Thongthip, S., Choi, G., Nicoletti, E., Schwartz, J. D., Weinberg, K., Rodriguez, et al
  AMER SOC HEMATOLOGY.2022: 7772-7773

- **Lentiviral-mediated Gene Therapy for Patients with Fanconi Anemia [Group A]: Updated Results from Global RP-L102 Clinical Trials**
  Czechowicz, A., Sevilla, J., Booth, C., Agarwal, R., Zubicaray, J., Rio, P., Navarro, S., Chetty, K., O'Toole, G., Xu-Bayford, J., Ancliff, P., Sebastian, E., Choi, et al
  AMER SOC HEMATOLOGY.2022: 10646-10647

- **Epigenetic and Immunological Indicators of IPEX Disease in subjects with FOXP3 gene mutation.** *The Journal of allergy and clinical immunology*
  Narula, M., Lakshmanan, U., Borna, S., Schulze, J. J., Holmes, T. H., Harre, N., Kirkey, M., Ramachandran, A., Tagi, V. M., Barzaghi, F., Grunebaum, E., Upton, J. E., Hong-Diep Kim, et al
  2022

- **Downregulation of SATB1 by miRNAs Reduces Megakaryocyte/Erythroid Progenitor Expansion in pre-clinical models of Diamond Blackfan Anemia.** *Experimental hematology*
  Wilkes, M. C., Scanlon, V., Shibuya, A., Celika, A. M., Eskin, A., Chen, Z., Narla, A., Glader, B., Roncarolo, M. G., Nelson, S. F., Sakamoto, K. M.
  2022

- **Unraveling Transcriptomic Profiles of Pediatric Acute Myeloid Leukemia Cells Sensitive or Resistant to Cytotoxic Killing by Engineered TR1-like Cells**
  Sayitoglu, E., Luca, B., Thomas, B., Cieniewicz, B., Uyeda, M., Chen, P., Cepika, A., Gentles, A., Roncarolo, M.
  CELL PRESS.2022: 153

- **Two is Better Than One: CRISPR/Cas9 Based Gene Editing with FOXP3 Isoforms for IPEX Therapy**
  Lee, E., Borna, S., Sato, Y., Bacchetta, R., Roncarolo, M., Porteus, M.
  CELL PRESS.2022: 34

- **The Women of FOCIS: Promoting Equality and Inclusiveness in a Professional Federation of Clinical Immunology Societies.** *Frontiers in immunology*
  Reed, E. F., Chong, A. S., Levings, M. K., Mutrie, C., Laufer, T. M., Roncarolo, M. G., Sykes, M.
  2022; 13: 816535

- **Type 1 regulatory T cell-mediated tolerance in health and disease.** *Frontiers in immunology*
  Freeborn, R. A., Strubbe, S., Roncarolo, M. G.
  2022; 13: 1032575

- **Downregulation of SATB1 by miRNAs Reduces Megakaryocyte/Erythroid Progenitor Expansion in pre-clinical models of Diamond Blackfan Anemia** *Experimental Hematology*
  Wilkes, M. C., Scanlon, V., Shibuya, A., Cepika, A., Eskin, A., Chen, Z., Narla, A., Glader, B., Roncarolo, M., Nelson, S. F., Sakamoto, K. M.
  2022

- **Functional Immune Tolerance Induced By Sequential Hematopoietic Stem Cell-Solid Organ Transplantation**
  Bertaina, A., Barbarito, G., Ramachandran, V. V., Kristovich, K., Lippner, E., Fathallah-Shaykh, S., Al-Uzri, A., Shah, A. J., Aubert, G., Slepicka, P., Oppizzi, L., Agarwal, R., Roncarolo, et al
  AMER SOC HEMATOLOGY.2021: 1818-+

- **JSP191 As a Single-Agent Conditioning Regimen Results in Successful Engraftment, Donor Myeloid Chimerism, and Production of Donor Derived Naive Lymphocytes in Patients with Severe Combined Immunodeficiency (SCID)**
  Agarwa, R., Dvorak, C. C., Prockop, S., Kwon, H., Long-Boyle, J. R., Le, A., Brown, J. W., Merkel, E., Truong, K., Velasco, B., Arulprakasam, K., Harada, N., Dougall, et al
  AMER SOC HEMATOLOGY.2021

- **Engineering Human Invariant Natural Killer T (iNKT) Cells to Overexpress Immunomodulatory Cytokines**
  Mavers, M., Hollingsworth, D., Boonchalermvichian, C., Liu, J., Baker, J., Ramos, T., Lohmeyer, J. K., Lin, P., Roncarolo, M., Negrin, R. S.
  AMER SOC HEMATOLOGY.2021

- **Gene Therapy for Fanconi Anemia [Group A]: Interim Results of RP-L102 Clinical Trials**

Czechowicz, A., Sevilla, J., Agarwal, R., Booth, C., Zubicaray, J., Rio, P., Navarro, S., Ancliff, P., Sebastian, E., Beard, B. C., Law, K. M., Choi, G., Zeini, et al
AMER SOC HEMATOLOGY.2021

- **Alloantigen-specific type 1 regulatory T cells suppress through CTLA-4 and PD-1 pathways and persist long-term in patients.** *Science translational medicine*
  Chen, P. P., Cepika, A., Agarwal-Hashmi, R., Saini, G., Uyeda, M. J., Louis, D. M., Cieniewicz, B., Narula, M., Amaya Hernandez, L. C., Harre, N., Xu, L., Thomas, B. C., Ji, et al
  2021; 13 (617): eabf5264

- **Development of beta-globin gene correction in human hematopoietic stem cells as a potential durable treatment for sickle cell disease.** *Science translational medicine*
  Lattanzi, A., Camarena, J., Lahiri, P., Segal, H., Srifa, W., Vakulskas, C. A., Frock, R. L., Kenrick, J., Lee, C., Talbott, N., Skowronski, J., Cromer, M. K., Charlesworth, et al
  2021; 13 (598)

- **Gene Therapy for Fanconi Anemia [Group A]: Preliminary Results of Ongoing RP-L102 Clinical Trials**
  Czechowicz, A., Sevilla, J., Booth, C., Agarwal, R., Zubicaray, J., Rio, P., Navarro, S., Ancliff, P. J., Beard, B. C., Law, K. M., Choi, G., Zeini, M., Duran-Persson, et al
  CELL PRESS.2021: 339

- **LV.InsB9-23-Based Therapy to Arrest T1D and Suppress Its Recurrency Post Allo-Islets Transplant**
  Russo, F., Citro, A., Sanvito, F., Monti, P., Gregori, S., Roncarolo, M., Annoni, A.
  CELL PRESS.2021: 358-359

- **Preclinical Safety and Efficacy Validation of CD4(LVFOXP3) Cells as an Innovative Cell-Based Gene Therapy Approach for IPEX Syndrome**
  Sato, Y., Nathan, A., Wright, J., Tate, K., Wani, P., Fazeli, F., Timnak, A., Bhatia, N., Agarwal-Hashmi, R., Bertaina, A., Roncarolo, M., Bacchetta, R.
  CELL PRESS.2021: 340

- **Engineered Type 1 Regulatory T Cells Have a Cytotoxic Profile and Kill Pediatric Acute Myeloid Leukemia Cells**
  Sayitoglu, E., Uyeda, M., Liu, J. M., Cieniewicz, B., Chen, P., Lacayo, N., Cepika, A., Roncarolo, M.
  CELL PRESS.2021: 317

- **Lentiviral Mediated Gene Therapy for Pyruvate Kinase Deficiency: Updated Results of a Global Phase 1 Study for Adult and Pediatric Patients**
  Lopez Lorenzo, J., Shah, A. J., Navarro, S., Sevilla, J., Llanos, L., Perez Camino de Gaisse, B., Sanchez, S., Glader, B., Chien, M., Quintana-Bustamante, O., Beard, B. C., Law, K. M., Zeini, et al
  CELL PRESS.2021: 42-43

- **Adoptively Transferred, In Vitro-Generated Alloantigen-Specific Type 1 Regulatory T (Tr1) Cells Persist Long-Term In Vivo**
  Cepika, A., Chen, P. P., Agarwal, R., Saini, G., Louis, D. M., Amaya-Hernandez, L. C., Xu, L., Shiraz, P., Tate, K. M., Margittai, D., Bhatia, N., Meyer, E., Bertaina, et al
  CELL PRESS.2021: 73

- **Hergen Spits-A legend at the top of his career.** *Allergy*
  Mjosberg, J., Roncarolo, M. G., Blom, B.
  2021

- **BHLHE40 Regulates IL-10 and IFN-# Production in T Cells but Does Not Interfere With Human Type 1 Regulatory T Cell Differentiation** *Frontiers in Immunology*
  Uyeda, M. J., Freeborn, R. A., Cieniewicz, B., Romano, R., Chen, P. P., Liu, J. M., Thomas, B., Lee, E., Cepika, A., Bacchetta, R., Roncarolo, M.
  2021

- **Co-Expression of FOXP3FL and FOXP3#2 Isoforms Is Required for Optimal Treg-Like Cell Phenotypes and Suppressive Function.** *Frontiers in immunology*
  Sato, Y., Liu, J., Lee, E., Perriman, R., Roncarolo, M. G., Bacchetta, R.
  2021; 12: 752394

- **The Yin and Yang of Type 1 Regulatory T Cells: From Discovery to Clinical Application.** *Frontiers in immunology*
  Sayitoglu, E. C., Freeborn, R. A., Roncarolo, M. G.
  2021; 12: 693105

- **Pre-clinical development and molecular characterization of an engineered type 1 regulatory T-cell product suitable for immunotherapy.** *Cytotherapy*

Liu, J. M., Chen, P., Uyeda, M. J., Cieniewicz, B., Sayitoglu, E. C., Thomas, B. C., Sato, Y., Bacchetta, R., Cepika, A. M., Roncarolo, M. G.
2021

- **InsB9-23 Gene Transfer To Hepatocytes-Based Combined Therapy Abrogates Recurrence of Type-1 Diabetes After Islet Transplantation.** *Diabetes*
  Russo, F., Citro, A., Squeri, G., Sanvito, F., Monti, P., Gregori, S., Roncarolo, M. G., Annoni, A.
  2020

- **Celebrating 20 years of FOCIS.** *Science immunology*
  Roncarolo, M. G., Anderson, M. S.
  2020; 5 (52)

- **Gene Therapy for Wiskott-Aldrich Syndrome: History, New Vectors, Future Directions.** *The Journal of allergy and clinical immunology*
  Ferrua, F., Marangoni, F., Aiuti, A., Roncarolo, M. G.
  2020

- **Engineered Type-1 Regulatory T Cells as Cellular Therapy for Treatment of Immune Mediated Diseases**
  Liu, J. M., Chen, P., Cieniewicz, B., Cepika, A., Bacchetta, R., Roncarolo, M.
  AMER ASSOC IMMUNOLOGISTS.2020

- **A beta T-Cell/CD19 B-Cell Depleted Haploidentical Stem Cell Transplantation: A New Platform for Curing Rare and Monogenic Disorders**
  Bertaina, A., Bacchetta, R., Lewis, D. B., Grimm, P. C., Shah, A. J., Agarwal, R., Concepcion, W., Czechowicz, A., Bhatia, N., Lahiri, P., Weinberg, K. I., Parkman, R., Porteus, et al
  ELSEVIER SCIENCE INC.2020: S288

- **Regulatory Type 1 T Cell Infusion in Mismatched Related or Unrelated Hematopoietic Stem Cell Transplantation (HSCT) for Hematologic Malignancies**
  Agarwal, R., Bacchetta, R., Bertaina, A., Chen, P., Saini, G., Shiraz, P., Bhatia, N., Roncarolo, M.
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