



Maria Grazia Roncarolo

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

Pediatrics - Stem Cell Transplantation

 NIH Biosketch available Online

CLINICAL OFFICES

- **Lucile Packard Children's Hospital**

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Bio

BIO

Maria Grazia Roncarolo, MD is the co-director of the Institute for Stem Cell Biology and Regenerative Medicine, the George D. Smith Professor in Stem Cell and Regenerative Medicine, Professor of Pediatrics and of Medicine (blood and marrow transplantation), chief of the Division of Pediatric Stem Cell Transplantation and Regenerative Medicine, and co-director of the Bass Center for Childhood Cancer and Blood Diseases.

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. Recently, 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. That trial is now considered the gold standard for gene therapy in inherited immune diseases. 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's goal at Stanford is to build the teams and infrastructure to move stem cell and gene therapy to the clinic quickly and to translate basic science discoveries into patient treatments. In addition, her laboratory continues to work on T regulatory cell-based treatments to induce immunological tolerance after transplantation of donor tissue stem cells. In Nature Medicine, Dr. Roncarolo recently published her discovery of new biomarkers for T regulatory type 1 cells, which will be used to purify the cells and to track them in patients. She also is investigating genetic chronic inflammatory and autoimmune diseases that occur due to impairment in T regulatory cell functions.

CLINICAL FOCUS

- Pediatrics

ACADEMIC APPOINTMENTS

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

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, European Society of Gene and Cell Therapy (2010)
- Elected Member, Academia Europaea of Sciences (2005)
- Knighthood "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 (CCCCF) (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

- Board Certification: Allergy and Immunology, Provincial Association of Surgeons and Dentists of Turin (1987)
- Board Certification: Pediatrics, Provincial Association of Surgeons and Dentists of Turin (1983)
- Fellowship: University of Milan School of Medicine (1990) Italy
- Residency: University of Turin Children Hospital, Italy (1986) Italy
- Medical Education: University of Turin (1982) Italy
- 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, Andrés 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>

Research & Scholarship

CURRENT RESEARCH AND SCHOLARLY INTERESTS

Research Interests

Immunotolerance: 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 allogeneic stem cell transplantation; Cytokines/Cytokine receptors: Role in regulation of immune and inflammatory responses

Clinical Interests

Primary Immunodeficiencies

Monogenic Autoimmune Disorders

Allogeneic Bone Marrow Transplantation

Gene Therapy Clinical Trials

Cell Therapy Clinical Trials

Clinical Trials in Autoimmune Diseases and Organ Transplantation

Clinical Trials in Hemoglobinopathies

Teaching

STANFORD ADVISEES

Doctoral Dissertation Reader (AC)

Benson George

Postdoctoral Faculty Sponsor

Brandon Cieniewicz, Jeff Liu, Rosa Romano, Ece Sayitoglu

Doctoral Dissertation Advisor (AC)

Molly Uyeda

Postdoctoral Research Mentor

Rosa Romano

Publications

PUBLICATIONS

- **Gene therapy for primary immunodeficiency.** *Human molecular genetics*
Booth, C., Romano, R., Roncarolo, M. G., Thrasher, A. J.
2019
- **Graft Engineering and Adoptive Immunotherapy: New Approaches to Promote Immune Tolerance After Hematopoietic Stem Cell Transplantation** *FRONTIERS IN IMMUNOLOGY*
Bertaina, A., Roncarolo, M.
2019; 10
- **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*
Ferrua, F., Cicalese, M., Galimberti, S., Giannelli, S., Dionisio, F., Barzaghi, F., Migliavacca, M., Bernardo, M., Calbi, V., Assanelli, A., Facchini, M., Fossati, C., Albertazzi, et al
2019; 6 (5): E239–E253
- **Gene correction for SCID-X1 in long-term hematopoietic stem cells.** *Nature communications*
Pavel-Dinu, M., Wiebking, V., Dejene, B. T., Srifa, W., Mantri, S., Nicolas, C. E., Lee, C., Bao, G., Kildebeck, E. J., Punjya, N., Sindhu, C., Inlay, M. A., Saxena, et al
2019; 10 (1): 1634
- **Coexpression of CD163 and CD141 identifies human circulating IL-10-producing dendritic cells (DC-10).** *Cellular & molecular immunology*
Comi, M., Avancini, D., Santoni de Sio, F., Villa, M., Uyeda, M. J., Floris, M., Tomasoni, D., Bulfone, A., Roncarolo, M. G., Gregori, S.
2019
- **Driving Medical Innovation Through Interdisciplinarity: Unique Opportunities and Challenges** *FRONTIERS IN MEDICINE*
Gohar, F., Maschmeyer, P., Mfarrej, B., Lemaire, M., Wedderburn, L. R., Roncarolo, M., van Royen-Kerkhof, A.
2019; 6
- **Author Correction: Gene correction for SCID-X1 in long-term hematopoietic stem cells.** *Nature communications*
Pavel-Dinu, M., Wiebking, V., Dejene, B. T., Srifa, W., Mantri, S., Nicolas, C. E., Lee, C., Bao, G., Kildebeck, E. J., Punjya, N., Sindhu, C., Inlay, M. A., Saxena, et al
2019; 10 (1): 2021
- **Molecular and functional heterogeneity of IL-10-producing CD4+ T cells.** *Nature communications*
Brockmann, L., Soukou, S., Steglich, B., Czarnewski, P., Zhao, L., Wende, S., Bedke, T., Ergen, C., Manthey, C., Agaloti, T., Geffken, M., Seiz, O., Parigi, et al
2018; 9 (1): 5457
- **Tregopathies: Monogenic diseases resulting in regulatory T-cell deficiency.** *The Journal of allergy and clinical immunology*
Cepika, A., Sato, Y., Liu, J. M., Uyeda, M. J., Bacchetta, R., Roncarolo, M. G.
2018; 142 (6): 1679–95
- **Reprogramming human T cell function and specificity with non-viral genome targeting** *NATURE*
Roth, T. L., Puig-Saus, C., Yu, R., Shifrut, E., Carnevale, J., Li, P., Hiatt, J., Saco, J., Krystofinski, P., Li, H., Tobin, V., Nguyen, D. N., Lee, et al
2018; 559 (7714): 405+

- **APVO210: A Bispecific Anti-CD86-IL-10 Fusion Protein (ADAPTIR (TM)) to Induce Antigen-Specific T Regulatory Type 1 Cells** *FRONTIERS IN IMMUNOLOGY*
Pellerin, L., Chen, P., Gregori, S., Hernandez-Hoyos, G., Bacchetta, R., Roncarolo, M.
2018; 9
- **DEVELOPMENT OF PERSISTENT MIXED CHIMERISM (MC) IN ALLO-HSCT TRANSPLANTED BETA-THAL PATIENTS IS ASSOCIATED WITH LOW MC EARLY AFTER TRANSPLANT AND TR1 CELLS**
Gregori, S., Galluccio, T., Roncarolo, M., Bacchetta, R., Andreani, M.
WILEY.2018: 399
- **CRISPR-Based Therapy for IPEX Syndrome as a Model of Genetic Autoimmunity**
Goodwin, M., Lee, E., Lakshmanan, U., Shipp, S., Roncarolo, M., Porteus, M., Bacchetta, R.
CELL PRESS.2018: 95–96
- **Genome Editing for IL-10 Deficiency in Purified Hematopoietic Stem Cells**
Romano, R., Pavel-Dinu, M., Bacchetta, R., Porteus, M. H., Roncarolo, M.
CELL PRESS.2018: 237–38
- **Genome Editing of Long-Term Human Hematopoietic Stem Cells for X-Linked Severe Combined Immunodeficiency**
Pavel-Dinu, M., Wiebking, V., Dejene, B. T., Srifa, W., Mantri, S., Nicolas, C., Lee, C. M., Bao, G., Kildebeck, E., Punjya, N., Sindhu, C., Inlay, M. A., Saxena, et al
SPRINGER/PLENUM PUBLISHERS.2018: 365–66
- **FOXP3 Gene Transfer in T cells and FOXP3 Gene Editing in HSC as Novel Treatment Options for IPEX Syndrome**
Goodwin, M., Sato, Y., Passerini, L., Barzaghi, F., Lee, E., Suzette, S. K., Roncarolo, M., Porteus, M., Bacchetta, R.
SPRINGER/PLENUM PUBLISHERS.2018: 427
- **Gene Therapy for Adenosine Deaminase Deficiency: A Comprehensive Evaluation of Short- and Medium-Term Safety** *MOLECULAR THERAPY*
Cicalese, M., Ferrua, F., Castagnaro, L., Rolfé, K., De Boever, E., Reinhardt, R. R., Appleby, J., Roncarolo, M., Aiuti, A.
2018; 26 (3): 917–31
- **Long-Term Treatment Outcome in IPEX Syndrome Patients: An International Multicenter Retrospective Study**
Barzaghi, F., Hernandez, L., Pai, S., Neven, B., Locatelli, F., Goldman, F., Seidel, M., Ehl, S., Albert, M. H., Dvorak, C. C., Carneiro-Sampaio, M., Gennery, A., Cowan, et al
ELSEVIER SCIENCE INC.2018: S81–S82
- **Long-term follow-up of IPEX syndrome patients after different therapeutic strategies: An international multicenter retrospective study** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*
Barzaghi, F., Hernandez, L., Neven, B., Ricci, S., Kucuk, Z., Bleesing, J. J., Nademi, Z., Slatter, M., Ulloa, E., Shcherbina, A., Roppelt, A., Worth, A., Silva, et al
2018; 141 (3): 1036+
- **Engineered T Regulatory Type 1 Cells for Clinical Application** *FRONTIERS IN IMMUNOLOGY*
Gregori, S., Roncarolo, M.
2018; 9: 233
- **First Occurrence of Plasmablastic Lymphoma in Adenosine Deaminase-Deficient Severe Combined Immunodeficiency Disease Patient and Review of the Literature** *FRONTIERS IN IMMUNOLOGY*
Migliavacca, M., Assanelli, A., Ponzoni, M., Pajno, R., Barzaghi, F., Giglio, F., Ferrua, F., Frittoli, M., Brigida, I., Dionisio, F., Nicoletti, R., Casiraghi, M., Roncarolo, et al
2018; 9: 113
- **Minimum Information about T Regulatory Cells: A Step toward Reproducibility and Standardization** *FRONTIERS IN IMMUNOLOGY*
Fuchs, A., Gliwinski, M., Grageda, N., Spiering, R., Abbas, A. K., Appel, S., Bacchetta, R., Battaglia, M., Berglund, D., Blazar, B., Bluestone, J. A., Bornhaeuser, M., ten Brinke, et al
2018; 8: 1844
- **Peanut-specific type 1 regulatory T cells induced in vitro from allergic subjects are functionally impaired** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*
Pellerin, L., Jenks, J., Chinthrajah, S., Dominguez, T., Block, W., Zhou, X., Noshirvan, A., Gregori, S., Roncarolo, M., Nadeau, K., Bacchetta, R.
2018; 141 (1): 202+

- **Identity and Diversity of Human Peripheral Th and T Regulatory Cells Defined by Single-Cell Mass Cytometry** *JOURNAL OF IMMUNOLOGY*
Kunicki, M. A., Hernandez, L., Davis, K. L., Bacchetta, R., Roncarolo, M.
2018; 200 (1): 336–46
- **gene therapy in Europe: paving the road for the next generation of advanced therapy medicinal products.** *EMBO molecular medicine*
Aiuti, A., Roncarolo, M. G., Naldini, L.
2017; 9 (6): 737-740
- **Lentiviral haemopoietic stem-cell gene therapy in early-onset metachromatic leukodystrophy: an ad-hoc analysis of a non-randomised, open-label, phase 1/2 trial.** *Lancet*
Sessa, M., Lorioli, L., Fumagalli, F., Acquati, S., Redaelli, D., Baldoli, C., Canale, S., Lopez, I. D., Morena, F., Calabria, A., Fiori, R., Silvani, P., Rancoita, et al
2016; 388 (10043): 476-487
- **In Vivo Tracking of Human Hematopoiesis Reveals Patterns of Clonal Dynamics during Early and Steady-State Reconstitution Phases.** *Cell stem cell*
Biasco, L., Pellin, D., Scala, S., Dionisio, F., Basso-Ricci, L., Leonardelli, L., Scaramuzza, S., Baricordi, C., Ferrua, F., Cicalese, M. P., Giannelli, S., Neduva, V., Dow, et al
2016; 19 (1): 107-119
- **Update on the safety and efficacy of retroviral gene therapy for immunodeficiency due to adenosine deaminase deficiency** *BLOOD*
Cicalese, M. P., Ferrua, F., Castagnaro, L., Pajno, R., Barzaghi, F., Giannelli, S., Dionisio, F., Brigida, I., Bonopane, M., Casiraghi, M., Tabucchi, A., Carlucci, F., Grunebaum, et al
2016; 128 (1): 45-54
- **Concise Review: Cell-Based Therapies and Other Non-Traditional Approaches for Type 1 Diabetes** *STEM CELLS*
Creusot, R. J., Battaglia, M., Roncarolo, M., Fathman, C. G.
2016; 34 (4): 809-819
- **From IPEX syndrome to FOXP3 mutation: a lesson on immune dysregulation.** *Annals of the New York Academy of Sciences*
Bacchetta, R., Barzaghi, F., Roncarolo, M.
2016
- **Clinical Outlook for Type-1 and FOXP3(+) T Regulatory Cell-Based Therapy** *FRONTIERS IN IMMUNOLOGY*
Gregori, S., Passerini, L., Roncarolo, M.
2015; 6
- **Hurdles in therapy with regulatory T cells.** *Science translational medicine*
Trzonkowski, P., Bacchetta, R., Battaglia, M., Berglund, D., Bohnenkamp, H. R., Ten brinke, A., Bushell, A., Cools, N., Geissler, E. K., Gregori, S., Marieke van Ham, S., Hilken, C., Hutchinson, et al
2015; 7 (304): 304ps18-?
- **B-cell reconstitution after lentiviral vector-mediated gene therapy in patients with Wiskott-Aldrich syndrome** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*
Castiello, M. C., Scaramuzza, S., Pala, F., Ferrua, F., Uva, P., Brigida, I., Sereni, L., van der Burg, M., Ottaviano, G., Albert, M. H., Roncarolo, M. G., Naldini, L., Aiuti, et al
2015; 136 (3): 692-?
- **Fatal autoimmunity in mice reconstituted with human hematopoietic stem cells encoding defective FOXP3** *BLOOD*
Goettel, J. A., Biswas, S., Lexmond, W. S., Yeste, A., Passerini, L., Patel, B., Yang, S., Sun, J., Ouahed, J., Shouval, D. S., McCann, K. J., Horwitz, B. H., Mathis, et al
2015; 125 (25): 3886-3895
- **Insulin B chain 9-23 gene transfer to hepatocytes protects from type 1 diabetes by inducing Ag-specific FoxP3(+) T-regs** *SCIENCE TRANSLATIONAL MEDICINE*
Akbarpour, M., Goudy, K. S., Cantore, A., Russo, F., Sanvito, F., Naldini, L., Annoni, A., Roncarolo, M. G.
2015; 7 (289)
- **Abnormalities of acid-base balance and predisposition to metabolic acidosis in Metachromatic Leukodystrophy patients** *MOLECULAR GENETICS AND METABOLISM*
Lorioli, L., Cicalese, M. P., Silvani, P., Assanelli, A., Salvo, I., Mandelli, A., Fumagalli, F., Fiori, R., Ciceri, F., Aiuti, A., Sessa, M., Roncarolo, M. G., Lanzani, et al
2015; 115 (1): 48-52

- **HLA-G expression levels influence the tolerogenic activity of human DC-10** *HAEMATOLOGICA*
Amodio, G., Comi, M., Tomasoni, D., Gianolini, M. E., Rizzo, R., LeMaout, J., Roncarolo, M., Gregori, S.
2015; 100 (4): 551-560
- **In vivo tracking of T cells in humans unveils decade-long survival and activity of genetically modified T memory stem cells** *SCIENCE TRANSLATIONAL MEDICINE*
Biasco, L., Scala, S., Ricci, L. B., Dionisio, F., Baricordi, C., Calabria, A., Giannelli, S., Cieri, N., Barzaghi, F., Pajno, R., Al-Mousa, H., Scarselli, A., Cancrini, et al
2015; 7 (273)
- **Sirolimus-based graft-versus-host disease prophylaxis promotes the in vivo expansion of regulatory T cells and permits peripheral blood stem cell transplantation from haploidentical donors** *LEUKEMIA*
Peccatori, J., Forcina, A., Clerici, D., Crocchiolo, R., Vago, L., Stanghellini, M. T., Noviello, M., Messina, C., Crotta, A., Assanelli, A., Markt, S., Olek, S., Mastaglio, et al
2015; 29 (2): 396-405
- **BAT2 and BAT3 polymorphisms as novel genetic risk factors for rejection after HLA-related SCT** *BONE MARROW TRANSPLANTATION*
Piras, I. S., Angius, A., Andreani, M., Testi, M., Lucarelli, G., Floris, M., Markt, S., Ciceri, F., La Nasa, G., Fleischhauer, K., Roncarolo, M. G., Bulfone, A., Gregori, et al
2014; 49 (11): 1400-1404
- **Mixed chimerism evolution is associated with T regulatory type 1 (Tr1) cells in a β -thalassemic patient after haploidentical haematopoietic stem cell transplantation.** *Chimerism*
Andreani, M., Gianolini, M. E., Testi, M., Battarra, M., Tiziana, G., Morrone, A., Sodani, P., Lucarelli, G., Roncarolo, M., Gregori, S.
2014; 5 (3-4): 75-79
- **B-cell development and functions and therapeutic options in adenosine deaminase-deficient patients** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*
Brigida, I., Sauer, A. V., Ferrua, F., Giannelli, S., Scaramuzza, S., Pistoia, V., Castiello, M. C., Barendregt, B. H., Cicalese, M. P., Casiraghi, M., Brombin, C., Puck, J., Muller, et al
2014; 133 (3): 799-?
- **Forkhead box P3: The Peacekeeper of the Immune System** *INTERNATIONAL REVIEWS OF IMMUNOLOGY*
Passerini, L., de Sio, F. R., Roncarolo, M. G., Bacchetta, R.
2014; 33 (2): 129-145
- **Immunological outcome in haploidentical-HSC transplanted patients treated with IL-10-energized donor T Cells** *FRONTIERS IN IMMUNOLOGY*
Bacchetta, R., Lucarelli, B., Sartirana, C., Gregori, S., Stanghellini, M. T., Miqueu, P., Tomiuk, S., Hernandez-Fuentes, M., Gianolini, M. E., Greco, R., Bernardi, M., Zappone, E., Rossini, et al
2014; 5
- **Tr1 cells and the counter-regulation of immunity: natural mechanisms and therapeutic applications.** *Current topics in microbiology and immunology*
Roncarolo, M. G., Gregori, S., Bacchetta, R., Battaglia, M.
2014; 380: 39-68
- **CD4(+) T Cells from IPEX Patients Convert into Functional and Stable Regulatory T Cells by FOXP3 Gene Transfer** *SCIENCE TRANSLATIONAL MEDICINE*
Passerini, L., Mel, E. R., Sartirana, C., Foustier, G., Bondanza, A., Naldini, L., Roncarolo, M. G., Bacchetta, R.
2013; 5 (215)
- **Liver gene therapy by lentiviral vectors reverses anti-factor IX pre-existing immunity in haemophilic mice** *EMBO MOLECULAR MEDICINE*
Annoni, A., Cantore, A., Della Valle, P., Goudy, K., Akbarpour, M., Russo, F., Bartolaccini, S., D'Angelo, A., Roncarolo, M. G., Naldini, L.
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