Stanford



Maria Grazia Roncarolo

George D. Smith Professor of Stem Cell and Regenerative Medicine, Emerita

Pediatrics - Ped Stem Cell Transplantation

NIH Biosketch available Online

CONTACT INFORMATION

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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

- Honorary Faculty Emeritus, Pediatrics - Ped Stem Cell 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
- Donor Umbilical Cord Blood Transplant With or Without Ex-vivo Expanded Cord Blood Progenitor Cells in Treating Patients With Acute Myeloid Leukemia, Acute Lymphoblastic Leukemia, Chronic Myelogenous Leukemia, or Myelodysplastic Syndromes, Not Recruiting

Publications

PUBLICATIONS

- **STAGE-SPECIFIC BM NICHE DETERIORATION AND HSPC DYSFUNCTION IN FANCONI ANEMIA: IMPLICATIONS FOR BONE MARROW FAILURE AND HEMATOLOGIC DISEASE**

  Soco, C., Chan, Y., Sen, S., Willner, H., Klinger, E., Nofal, R., Singh, M., Parkman, R., Roncarolo, M., Weinberg, K., Porteus, M., Bertaina, A., Argawal, et al

ELSEVIER SCIENCE INC.2026

- **Epigenetic landscape, key transcriptional regulators, and in vivo identification of human Tr1 cells.** *Science advances*
  Cepika, A. M., Amaya, L., Waichler, C., Narula, M., Mantilla, M., Thomas, B. C., Chen, P. P., Freeborn, R. A., Pavel-Dinu, M., Nideffer, J., Porteus, M., Bacchetta, R., Müller, et al
  2026; 12 (28): eaec6358

- **Phase 1 gene therapy for patients with IPEX syndrome: preliminary in vivo data of the treatment with engineered Treg-like cell CD4^{LVFOXP3}**
  Alexander, J. L., Agarwal, R., Namjoshi, S., Ramachandran, A., Wang, B., Borna, S., Shah, S., Schaefer, K., Alkayed, A., Kuo, Y., Wani, P., Faseli, F., Kandar, et al
  SPRINGERNATURE.2026: 48-49

- **CXCR6+CD127- Tr1 cells balance immunity and persistence in Plasmodium falciparum infection.** *The Journal of clinical investigation*
  Nideffer, J., Bach, F., Strubbe, S., Lopez, L., Zedi, M., Nankya, F., Briggs, J., van der Ploeg, K., Musinguzi, K., Kim, S., Garcia Romero, A., Keya, A., Camanag, et al
  2026

- **Off-the-Shelf Engineered Tr1 Cells (TRX103) in Patients with Hematological Malignancies Undergoing HLA-Mismatched Hematopoietic Cell Transplantation (HCT) Show Safety and Dose Dependent Effects**
  Al Malki, M. M., Juckett, M. B., Perales, M., Koreth, J., Kato, Y., Powers, J., Davis, M., Londei, M., Roncarolo, M., Milano, F.
  ELSEVIER SCIENCE INC.2026: S6-S7

- **T-allo10 Cell Addback Post-Abdepleted-HSCT Drives Lasting Leukemia-Free Survival with Distinct Immune Regulation Biomarkers**
  Bertaina, A., Bacchetta, R., Shyr, D. C., Saini, G., Lee, J., Agarwal, R., Klein, O. R., Wang, G., Margittai-Gotesman, D., Al Dakheel, A., Barbarito, G., Oppizzi, L., Limaye, et al
  ELSEVIER SCIENCE INC.2026: S99

- **THERAPEUTIC DOSES OF T-ALLO10 IMMUNOTHERAPY POST-ABDEPLETED HSCT ENHANCE IMMUNE RECONSTITUTION AND ACHIEVE 100% LEUKEMIA-FREE SURVIVAL IN PEDIATRIC AND YOUNG ADULT PATIENTS WITH HEMATOLOGIC MALIGNANCIES**
  Bertaina, A., Bacchetta, R., Cepika, A., Shyr, D., Barbarito, G., Oppizzi, L., Chen, P., Saini, G., Lee, J., Kristovich, K., Agarwal, R., Klein, O., Melsop, et al
  SPRINGERNATURE.2025: 74-75

- **Hematopoietic stem cell transplantation using briquilimab (Anti-CD117 Antibody-Conditioning), immunosuppression and TCRαβ+ T-cell/ CD19+B-cell depleted haploidentical grafts in patients with fanconi anemia: An approach without irradiation, busulfan and calcineurin inhibitors.**
  Agarwal, R., Bertaina, A., Soco, C., Long-Boyle, J., Saini, G., Kunte, N., Hiroshima, L., Chan, Y., Willner, H., Krampf, M., Van Hentenryck, M., Perriman, R., Istomina, et al
  ELSEVIER.2025: 2432-2433

- **IL10 gene mutations do not impair Tr1 differentiation in vivo and can be functionally corrected by precise gene editing**
  Strubbe, S., Boss, A., Wang, B., Mantilla, M., Freeborn, R., Guevara Becerra, M., Gharahdaghi, N., Uhlig, H. H., Porteus, M., Arkwright, P. D., Roncarolo, M., Bacchetta, R.
  OXFORD UNIV PRESS.2025

- **CXCR6+ CD127- Tr1 Cells Balance Immunity and Persistence in Plasmodium falciparum Infection.** *bioRxiv : the preprint server for biology*
  Nideffer, J., Bach, F., Strubbe, S., Lopez, L., Zedi, M., Nankya, F., Briggs, J., van der Ploeg, K., Musinguzi, K., Kim, S., Romero, A. G., Keya, A., Camanag, et al
  2025

- **Irradiation- and busulfan-free stem cell transplantation in Fanconi anemia using an anti-CD117 antibody: a phase 1b trial.** *Nature medicine*
  Agarwal, R., Bertaina, A., Soco, C., Long-Boyle, J. R., Saini, G., Kunte, N., Hiroshima, L., Chan, Y. Y., Willner, H., Krampf, M. R., Nofal, R., Barbarito, G., Sen, et al
  2025

- **Distinct type I and II interferon responses direct cortical and medullary thymic epithelial cell development.** *Science immunology*
  Mohammed, A., Wang, W., Arreola, M., Solomon, B. D., Slepicka, P. F., Hubka, K. M., Nguyen, H. D., Zheng, Z., Chavez, M. G., Yeh, C. Y., Kim, D. K., Ma, M. R., Martin, et al
  2025; 10 (107): eado4720

- **Clone tracking through repeated malaria identifies high-fidelity memory CD4 T cell responses.** *Science immunology*

Nideffer, J., Bach, F., Nankya, F., Musinguzi, K., Borna, Š., Mantilla, M., Zedi, M., Garcia Romero, A., Gerungan, C., Yang, N., Kim, S., van der Ploeg, K., Camanag, et al

2025; 10 (106): eads2957

- **From promise to practice: CAR T and Treg cell therapies in autoimmunity and other immune-mediated diseases.** *Frontiers in immunology*

  Bulliard, Y., Freeborn, R., Uyeda, M. J., Humes, D., Bjordahl, R., de Vries, D., Roncarolo, M. G.

  2024; 15: 1509956

- **Lentiviral-Mediated Gene Therapy for Patients with Fanconi Anemia [Group a]: Updated Results from Global RP-L102 Clinical Trials**

  Czechowicz, A. D., Sevilla, J., Booth, C., Zubicaray, J., Rio, P., Navarro, S., Cherry, K., O'Toole, G., Xu-Bayford, J., Ancliff, P., Sebastian, E., Rothe, M., Bastone, et al

  ELSEVIER.2024: 7463-7464

- **T-allo10 Immunotherapy Results in Enhanced Early Immune Reconstitution and Reduced GvHD with Excellent Clinical Outcomes Post Aβ-Depleted HSCT in Pediatric and Young Adult Hematologic Malignancy Patients**

  Bertaina, A., Bacchetta, R., Cepika, A., Shyr, D. C., Barbarito, G., Oppizzi, L., Chen, P., Saini, G., Lee, J., Kristovich, K., Agarwal, R., Klein, O., Melsop, et al

  ELSEVIER.2024: 3525-3526

- **Transcriptional and epigenetic characterization of a new in vitro platform to model the formation of human pharyngeal endoderm.** *Genome biology*

  Cipriano, A., Colantoni, A., Calicchio, A., Fiorentino, J., Gomes, D., Moqri, M., Parker, A., Rasouli, S., Caldwell, M., Briganti, F., Roncarolo, M. G., Baldini, A., Weinacht, et al

  2024; 25 (1): 211

- **In Utero Hematopoietic Stem Cell Transplantation for Fanconi Anemia.** *Blood advances*

  Swartzrock, L., Dib, C., Denis, M., Willner, H., Ho, K., Haslett, E., Han, J., Pan, W., Byrne-Steele, M., Brown, B., Krampf, M. R., Girsen, A., Blumenfeld, et al

  2024

- **AML/T cell interactomics uncover correlates of patient outcomes and the key role of ICAM1 in T cell killing of AML.** *Leukemia*

  Sayitoglu, E. C., Luca, B. A., Boss, A. P., Thomas, B. C., Freeborn, R. A., Uyeda, M. J., Chen, P. P., Nakauchi, Y., Waichler, C., Lacayo, N., Bacchetta, R., Majeti, R., Gentles, et al

  2024

- **Molecular and functional identification of unstable regulatory and autoreactive effector T cells that are expanded in patients with FOXP3 mutation**

  Borna, S., Lee, E., Nideffer, J., Ramachandran, A., Wang, B., Mavers, M., Narula, M., Garrett, A., Schulze, J., Marois, L., Gernez, Y., Bathia, M., Chong, et al

  ACADEMIC PRESS INC ELSEVIER SCIENCE.2024

- **Lentiviral-Mediated Gene Therapy (RP-L102) for Fanconi Anemia [Group A], is Associated with Polyclonal Integration Patterns in the Absence of Conditioning**

  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., Rothe, et al

  CELL PRESS.2024: 132

- **Gene Therapy for Adult and Pediatric Patients with Severe Pyruvate Kinase Deficiency: Results from a Global Study of RP-L301**

  Shah, A., Sevilla, J., Lopez Lorenzo, J., Navarro, S., Llanos, L., de Camino Gaisse, B., Sanchez, S., Zubicaray, J., Glader, B., Chien, M., Quintana Bustamante, O., Gonzalez Vicent, M., Iriondo, et al

  CELL PRESS.2024: 2-3

- **A multidisciplinary approach to unraveling genetic forms of immune dysregulation in children with refractory multilineage cytopenia**

  Gernez, Y., Sathi, B., Camacho, J., Rao, L., Glader, B., Singh, D., Hoyte, E., Lewis, D., Roncarolo, M., Chien, M., Bacchetta, R., Weinacht, K.

  MOSBY-ELSEVIER.2024: AB186

- **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 α β + 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

- **One Year Follow-up on the First Patient Treated with Nula-Cel: An Autologous CRISPR/Cas9 Gene Corrected CD34+Cell Product to Treat Sickle Cell Disease**
  Shyr, D. C., Lowsky, R., Miller, W., Schroeder, M. A., Buchholz, T., Dougall, K., Intondi, A., Charles, A., Lehrer, J., Bouge, A., Wolf, S., MacDonald, B., Din, 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
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- **Downregulation of SATB1 by miRNAs Reduces Megakaryocyte/Erythroid Progenitor Expansion in pre-clinical models of Diamond Blackfan Anemia.** *Experimental hematology*
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