Stanford



Rosa Bacchetta

Professor (Research) of Pediatrics (Stem Cell Transplantation)

Pediatrics - Stem Cell Transplantation

NIH Biosketch available Online

CONTACT INFORMATION

- **Administrative Assistant**

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Bio

BIO

My professional goal as a pediatrician specializing in immunology, has been to challenge the limits of "inexplicable" and "untreatable" diseases, and apply current scientific knowledge to understand the mechanisms of impaired cellular immune function underlying the clinical manifestations in order to develop curative treatments.

Following the completion of my pediatrics residency, I received training in molecular and cellular immunology in France (UNICET, Lyon) and the United States (DNAX Research Institute of Molecular and Cellular Biology, Palo Alto) under scientists, who critically influenced my scientific development. There, I was first exposed to the importance of integrating in depth laboratory research with clinical observations to develop a translational research approach to science. I then worked for fifteen years at the San Raffaele Scientific Institute in the Telethon Institute for Gene Therapy (HSR-TIGET), where I focused on dissecting the genetic and immunological basis of primary immunodeficiencies with autoimmune manifestations that might be treated by gene therapy.

ACADEMIC APPOINTMENTS

- Professor-Research, Pediatrics - Stem Cell Transplantation
- Member, Bio-X
- Member, SPARK at Stanford
- Member, Institute for Stem Cell Biology and Regenerative Medicine
- Member, Maternal & Child Health Research Institute (MCHRI)
- Member, Stanford Medicine Children’s Health Center for IBD and Celiac Disease

HONORS AND AWARDS

- Associate Professor in Pediatrics, Italian Ministry of University (01/2014)

BOARDS, ADVISORY COMMITTEES, PROFESSIONAL ORGANIZATIONS

- Member, European Society of Immunodeficiency (ESID) (2014 - present)

- Member, Federal of Clinical Immunology Societies (FOCIS and CIS) (2014 - present)
- Associate Editor, Frontiers in Primary Immunodeficiencies (2014 - present)
- Reviewer, J Autoimmunity (2014 - present)
- Reviewer, European Journal of Immunology (2014 - present)
- Reviewer, Transplantation (2014 - present)
- Grants revisions, European Research Council (2014 - present)
- Grants revisions, INSERM (2014 - present)
- Grants revision, LSBR Foundation (2014 - present)
- Grants revision, Wellcome Trust (2014 - present)
- Grants revision, ANR-France (2014 - present)
- Teaching activities ad Hoc lessons, Vita-Salute University Medical School and Biotechnology, Milan (2014 - present)
- Teaching activities ad hoc lessons, Tor Vergata University, Rome (2014 - present)
- Teaching activities ad hoc lessons, Undergraduate Course at Stanford University, Stanford, Palo Alto, CA (2014 - present)

PROFESSIONAL EDUCATION

- Fellowship, University of Turin, Italy , General Pediatrics Immunology (1991)
- MD, University of Turin, Italy , Medicine (1987)

PATENTS

- Rosa Bacchetta. "United States Patent 16/640,306 Lentiviral vectors expressing FOXP3 in hematopoietic stem cells to treat immune deficiencies and autoimmune diseases", Rosa Bacchetta
- Rosa Bacchetta. "United States Patent 17/255,657 Epigenetic method to detect and distinguish IPEX and IPEX-like syndromes, in particular in newborns", Rosa Bacchetta
- Rosa Bacchetta. "United States Patent 17/615,766 FOXP3 Engineered CD4+ T Cells Suitable for Treg-based Immunotherapy", Rosa Bacchetta
- Rosa Bacchetta. "United States Patent 17/760,264 CRISPR-based FOXP3 gene Engineered T cells and Hematopoietic Stem Cell Precursors to treat IPEX syndrome patients", Rosa Bacchetta
- Rosa Bacchetta. "United States Patent PCT/US2022/053529 CRISPR-based gene editing to preserve splicing and expression of FOXP3 isoforms 1 and 2", Rosa Bacchetta
- Rosa Bacchetta. "United States Patent WO2007/131575 Tr1 dendritic cells, method to generate regulatory type 1 T (Tr1) cells and uses thereof", Rosa Bacchetta, Apr 1, 2007
- Rosa Bacchetta. "United States Patent 6884410 Methods for modulating antigen-specific immune responses", Rosa Bacchetta, Apr 26, 2005
- Rosa Bacchetta. "United States Patent 6277635 Use of Interleukin-10 produce a population of suppressor cells", Rosa Bacchetta, Aug 21, 2001

LINKS

- Division of Stem Cell Transplantation and Regenerative Medicine: http://med.stanford.edu/ptrm.html
- Bacchetta Lab: http://med.stanford.edu/bacchettalab.html

Research & Scholarship

CURRENT RESEARCH AND SCHOLARLY INTERESTS

In the coming years, I plan to further determine the genetic and immunological basis of diseases with autoimmunity or immune dysregulation in children. I believe that much can still be learned from the in depth mechanistic studies of pediatric autoimmune diseases. Genomic analysis of the patients' samples has become possible which may provide a rapid indication of altered target molecules. I plan to implement robust functional studies to define the consequences of these genetic abnormalities and bridge them to the patient's clinical phenotype.

Understanding functional consequences of gene mutations in single case/family first and then validating the molecular and cellular defects in other patients with similar phenotypes, will anticipate and complement cellular and gene therapy strategies.

For further information please visit the Bacchetta Lab website:

http://med.stanford.edu/bacchettalab.html

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
- Sirolimus and Mycophenolate Mofetil in Preventing GVHD in Patients With Hematologic Malignancies Undergoing HSCT, Not Recruiting
- Use of T-allo10 in Hematopoietic Stem Cell Transplantation (HSCT) for Blood Disorders, Not Recruiting

Teaching

STANFORD ADVISEES

Doctoral Dissertation Reader (AC)

Jessica Diarra

Postdoctoral Faculty Sponsor

Merve Nida Gokbak, Neetu Saini, Bing Wang

GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

- Immunology (PhD Program)
- Pediatric Hem/Onc (Fellowship Program)
- Stem Cell Biology and Regenerative Medicine (PhD Program)

Publications

PUBLICATIONS

- **Refractory infantile IPEX with Treg-restricted FOXP3null expression caused by a novel variant in FOXP3.** *Journal of human immunity*
  Rayes, A., Ramachandran, A., Pulsipher, M. A., Dolan, J. G., Cho, S., Johnson, A. K., Alexander, J. L., Borna, S., Bacchetta, R.
  2026; 2 (3): e20250249

- **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

- **Regulatory T cell therapies: from patient data to biological insights.** *Frontiers in immunology*
  Rodrigues, K. B., Eggenhuizen, P. J., Bacchetta, R., Good, Z.
  2025; 16: 1675114

- **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

- **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

- **IPEX Syndrome from diagnosis to cure, learning along the way.** *The Journal of allergy and clinical immunology*
  Bacchetta, R., Roncarolo, M. G.
  2023
- **FOXP3 deficiency, from the mechanisms of the disease to curative strategies.** *Immunological reviews*
  Borna, S., Meffre, E., Bacchetta, R.
  2023
- **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
- **Towards gene therapy for IPEX syndrome.** *European journal of immunology*
  Borna, S., Lee, E., Sato, Y., Bacchetta, R.
  2022
- **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
- **Thymic origins of autoimmunity-lessons from inborn errors of immunity.** *Seminars in immunopathology*
  Bacchetta, R., Weinberg, K.
  2021
- **CRISPR-based gene editing enables FOXP3 gene repair in IPEX patient cells** *SCIENCE ADVANCES*
  Goodwin, M., Lee, E., Lakshmanan, U., Shipp, S., Froessl, L., Barzaghi, F., Passerini, L., Narula, M., Sheikali, A., Lee, C. M., Bao, G., Bauer, C. S., Miller, et al
  2020; 6 (19)
- **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
- **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.
  ELSEVIER SCIENCE INC.2020: S272–S273
- **Human inborn errors of immunity: An expanding universe.** *Science immunology*
  Notarangelo, L. D., Bacchetta, R. n., Casanova, J. L., Su, H. C.
  2020; 5 (49)
- **Hematopoietic Cell Transplantation in Patients With Primary Immune Regulatory Disorders (PIRD): A Primary Immune Deficiency Treatment Consortium (PIDTC) Survey.** *Frontiers in immunology*
  Chan, A. Y., Leiding, J. W., Liu, X., Logan, B. R., Burroughs, L. M., Allenspach, E. J., Skoda-Smith, S., Uzel, G., Notarangelo, L. D., Slatter, M., Gennery, A. R., Smith, A. R., Pai, et al
  2020; 11: 239
- **Human-engineered Treg-like cells suppress FOXP3-deficient T cells but preserve adaptive immune responses in vivo.** *Clinical & translational immunology*
  Sato, Y. n., Passerini, L. n., Piening, B. D., Uyeda, M. J., Goodwin, M. n., Gregori, S. n., Snyder, M. P., Bertaina, A. n., Roncarolo, M. G., Bacchetta, R. n.
  2020; 9 (11): e1214
- **The autoimmune targets in IPEX are dominated by gut epithelial proteins** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*
  Eriksson, D., Bacchetta, R., Gunnarsson, H., Chan, A., Barzaghi, F., Ehl, S., Hallgren, A., van Gool, F., Sardh, F., Lundqvist, C., Laakso, S. M., Ronnblom, A., Ekwall, et al

2019; 144 (1): 327-+

- **Immunoregulatory Cell Therapy with Lentiviral-Mediated FOXP3 Converted CD4+T Cells into Treg Cells: Towards the Proof-of-Concept Application in IPEX Syndrome**
  Sato, Y., Passerini, L., Roncarolo, M., Bacchetta, R.
  CELL PRESS.2019: 311

- **Case Study: Mechanism for Increased Follicular Helper T Cell Development in Activated PI3K Delta Syndrome.** *Frontiers in immunology*
  Thauland, T. J., Pellerin, L., Ohgami, R. S., Bacchetta, R., Butte, M. J.
  2019; 10: 753

- **Epigenetic Immune Cell Quantification for Diagnosis and Monitoring of Patients with Primary Immune Deficiencies and Immune Regulatory Disorders**
  Schulze, J., Werner, J., Schildknecht, K., Lakshmanan, U., Gruetzkau, A., Chu, J., Gernez, Y., Speckmann, C., Weinacht, K. G., Bertaina, A., Baron, U., Borte, S., Olek, et al
  SPRINGER/PLENUM PUBLISHERS.2019: S30

- **Treatment with Rapamycin Can Restore Regulatory T Cell Function in IPEX Patients.** *The Journal of allergy and clinical immunology*
  Passerini, L. n., Barzaghi, F. n., Curto, R. n., Sartirana, C. n., Barera, G. n., Tucci, F. n., Albarello, L. n., Mariani, A. n., AlbertoTestoni, P. n., Bazzigaluppi, E. n., Bosi, E. n., Lampasona, V. n., Neth, et al
  2019

- **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

- **Epigenetic immune cell counting in human blood samples for immunodiagnostics** *SCIENCE TRANSLATIONAL MEDICINE*
  Baron, U., Werner, J., Schildknecht, K., Schulze, J. J., Mulu, A., Liebert, U., Sack, U., Speckmann, C., Gossen, M., Wong, R. J., Stevenson, D. K., Babel, N., Schuermann, et al
  2018; 10 (452)

- **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-+

- **Neutralizing Anti-Cytokine Autoantibodies Against Interferon-alpha in Immunodysregulation Polyendocrinopathy Enteropathy X-Linked** *FRONTIERS IN IMMUNOLOGY*
  Rosenberg, J. M., Maccari, M. E., Barzaghi, F., Allenspach, E. J., Pignata, C., Weber, G., Torgerson, T. R., Utz, P. J., Bacchetta, R.
  2018; 9: 544

- **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-+

- **Forkhead-Box-P3 Gene Transfer in Human CD4(+) T Conventional Cells for the Generation of Stable and Efficient Regulatory T Cells, Suitable for Immune Modulatory Therapy** *FRONTIERS IN IMMUNOLOGY*
  Passerini, L., Bacchetta, R.
  2017; 8: 1282

- **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

- **Congenital diarrhoeal disorders: advances in this evolving web of inherited enteropathies** *NATURE REVIEWS GASTROENTEROLOGY & HEPATOLOGY*
  Canani, R. B., Castaldo, G., Bacchetta, R., Martin, M. G., Goulet, O.
  2015; 12 (5): 293-302

- **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-Anergized Donor T Cells.** *Frontiers in immunology*
  Bacchetta, R., Lucarelli, B., Sartirana, C., Gregori, S., Lupo Stanghellini, M. T., Miqueu, P., Tomiuk, S., Hernandez-Fuentes, M., Gianolini, M. E., Greco, R., Bernardi, M., Zappone, E., Rossini, et al
  2014; 5: 16-?

- **Gene/Cell Therapy Approaches for Immune Dysregulation Polyendocrinopathy Enteropathy X-Linked Syndrome** *CURRENT GENE THERAPY*
  Passerini, L., de Sio, F. R., Porteus, M. H., Bacchetta, R.
  2014; 14 (6): 422-428

- **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., Fousteri, G., Bondanza, A., Naldini, L., Roncarolo, M. G., Bacchetta, R.
  2013; 5 (215)

- **Autoantibodies to Harmonin and Villin Are Diagnostic Markers in Children with IPEX Syndrome** *PLOS ONE*
  Lampasona, V., Passerini, L., Barzaghi, F., Lombardoni, C., Bazzigaluppi, E., Brigatti, C., Bacchetta, R., Bosi, E.
  2013; 8 (11)

- **Coexpression of CD49b and LAG-3 identifies human and mouse T regulatory type 1 cells** *NATURE MEDICINE*
  Gagliani, N., Magnani, C. F., Huber, S., Gianolini, M. E., Pala, M., Licona-Limon, P., Guo, B., Herbert, D. R., Bulfone, A., Trentini, F., Di Serio, C., Bacchetta, R., Andreani, et al
  2013; 19 (6): 739-?

- **Human IL2RA null mutation mediates immunodeficiency with lymphoproliferation and autoimmunity** *CLINICAL IMMUNOLOGY*
  Goudy, K., Aydin, D., Barzaghi, F., Gambineri, E., Vignoli, M., Mannurita, S. C., Doglioni, C., Ponzoni, M., Cicalese, M. P., Assanelli, A., Tommasini, A., Brigida, I., Dellepiane, et al
  2013; 146 (3): 248-261

- **Accumulation of peripheral autoreactive B cells in the absence of functional human regulatory T cells** *BLOOD*
  Kinnunen, T., Chamberlain, N., Morbach, H., Choi, J., Kim, S., Craft, J., Mayer, L., Cancrini, C., Passerini, L., Bacchetta, R., Ochs, H. D., Torgerson, T. R., Meffre, et al
  2013; 121 (9): 1595-1603

- **Case Report: Effective IL-4/IL-13 axis suppression and resolution of severe atopic dermatitis by dupilumab monotherapy in a patient with IPEX syndrome.** *Frontiers in immunology*
  Alshanti, M., MacDougall, M. S., Gokbak, M. N., Ramachandran, A., Alzaabi, A. A., Alexander, J. L., Bacchetta, R., Shendi, H. M.
  2026; 17: 1923415

- **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

- **IMPACT (Immune Monitoring and Phenotype Assessment of Clinical Trajectory) for Patients with Inborn Errors of Immunity from the Primary Immune Deficiency Treatment Consortium (PIDTC).** *The journal of allergy and clinical immunology. In practice*
  Arnold, D. E., Eissa, H., Dunn, E., Cuvelier, G. D., Wright, N. A., Marsh, R. A., Parikh, S., Saldana, B. D., Vander Lugt, M. T., Bacchetta, R., Satter, L. F., Chandrakasan, S., Kellner, et al
  2026

- **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

- **Editorial: Inborn Errors of Immunity (IEI) breaking immune homeostasis and tolerance: a key role for T regulatory cells.** *Frontiers in immunology*
  De Rosa, V., Sogkas, G., Bacchetta, R.
  2024; 15: 1532079

- **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

- **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

- **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

- **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

- **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

- **FOXP3 TSDR Measurement Could Assist Variant Classification and Diagnosis of IPEX Syndrome.** *Journal of clinical immunology*
  Wyatt, R. C., Olek, S., De Franco, E., Samans, B., Patel, K., Houghton, J., Walter, S., Schulze, J., Bacchetta, R., Hattersley, A. T., Flanagan, S. E., Johnson, M. B.
  2023

- **Analyses of thymocyte commitment to regulatory T cell lineage in thymus of healthy subjects and patients with 22q11.2 deletion syndrome.** *Frontiers in immunology*
  Borna, S., Dejene, B., Lakshmanan, U., Schulze, J., Weinberg, K., Bacchetta, R.
  2023; 14: 1088059

- **Rare immune diseases paving the road for genome editing-based precision medicine.** *Frontiers in genome editing*
  Pavel-Dinu, M., Borna, S., Bacchetta, R.
  2023; 5: 1114996

- **Case report: Refractory Evans syndrome in two patients with spondyloenchondrodysplasia with immune dysregulation treated successfully with JAK1/JAK2 inhibition.** *Frontiers in immunology*
  Gernez, Y., Narula, M., Cepika, A., Valdes Camacho, J., Hoyte, E. G., Mouradian, K., Glader, B., Singh, D., Sathi, B., Rao, L., Tolin, A. L., Weinberg, K. I., Lewis, et al
  2023; 14: 1328005

- **Autoantibody discovery across monogenic, acquired, and COVID19-associated autoimmunity with scalable PhIP-Seq.** *eLife*
  Vazquez, S. E., Mann, S. A., Bodansky, A., Kung, A. F., Quandt, Z., Ferre, E. M., Landegren, N., Eriksson, D., Bastard, P., Zhang, S., Liu, J., Mitchell, A., Proekt, et al
  2022; 11

- **Design of experiments as a decision tool for cell therapy manufacturing.** *Cytotherapy*
  Lee, E., Shah, D., Porteus, M., Wright, J. F., Bacchetta, R.
  2022

- **Editorial: IPEX 2020: An Expanding Disease Spectrum and Novel Precision Therapies.** *Frontiers in pediatrics*
  Bacchetta, R., Chatila, T.
  2022; 10: 856920

- **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

- **BHLHE40 Regulates IL-10 and IFN-γ 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 FOXP3Δ2 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

- **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

- **Early Epigenetic Immune Quantification Following Alpha/Beta T-Cell/CD19 B-Cell Depleted Haploidentical Stem Cell Transplant Correlates with CD4+T Cell Recovery at Day+100**

  Mayers, M., Schulze, J., Barbarito, G., Lakshmanan, U., Parkman, R., Weinberg, K. I., Chu, J., Agarwal, R., Roncarolo, M., Sachsenmaier, C., Bacchetta, R., Bertaina, A.

  ELSEVIER SCIENCE INC.2020: S305

- **Alloantigen-specific Tr1 cells designed to prevent GvHD have a distinct molecular identity and suppress through CTLA-4 and PD-1** *Society for Immunotherapy of Cancer's (SITC) 35th Anniversary Annual Meeting*

  Cepika, A., Chen, P. P., Uyeda, M. J., Cieniewicz, B., Narula, M., Amaya, L., Louis, D. M., Xu, L., Ji, X., Bertaina, A., Agarwal-Hashmi, R., Davis, M. M., Meyer, et al

  2020: A159–A159

- **Engineered type 1 regulatory T cells designed for clinical use kill primary pediatric acute myeloid leukemia cells** *Haematologica*

  Cieniewicz, B., Uyeda, M. J., Chen, P. P., Sayitoglu, E. C., Liu, J. M., Andolfi, G., Greenthal, K., Bertaina, A., Gregori, S., Bacchetta, R., Lacayo, N. J., Cepika, A., Roncarolo, et al

  2020

- **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

- **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

- **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

- **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., Hilkens, C., Hutchinson, et al

2015; 7 (304): 304ps18-?

- **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., Hilkens, C., Hutchinson, et al
  2015; 7 (304): 304ps18-?

- **Chemically modified guide RNAs enhance CRISPR-Cas genome editing in human primary cells.** *Nature biotechnology*
  Hendel, A., Bak, R. O., Clark, J. T., Kennedy, A. B., Ryan, D. E., Roy, S., Steinfeld, I., Lunstad, B. D., Kaiser, R. J., Wilkens, A. B., Bacchetta, R., Tsalenko, A., Dellinger, et al
  2015; 33 (9): 985-989

- **Chemically modified guide RNAs enhance CRISPR-Cas genome editing in human primary cells.** *Nature biotechnology*
  Hendel, A., Bak, R. O., Clark, J. T., Kennedy, A. B., Ryan, D. E., Roy, S., Steinfeld, I., Lunstad, B. D., Kaiser, R. J., Wilkens, A. B., Bacchetta, R., Tsalenko, A., Dellinger, et al
  2015; 33 (9): 985-989

- **Clinical Features and Follow-Up in Patients with 22q11.2 Deletion Syndrome** *JOURNAL OF PEDIATRICS*
  Cancrini, C., Puliafito, P., Digilio, M. C., Soresina, A., Martino, S., Rondelli, R., Consolini, R., Ruga, E. M., Cardinale, F., Finocchi, A., Romiti, M. L., Martire, B., Bacchetta, et al
  2014; 164 (6): 1475-?

- **Identification of STAT5A and STAT5B Target Genes in Human T Cells.** *PloS one*
  Kanai, T., Seki, S., Jenks, J. A., Kohli, A., Kawli, T., Martin, D. P., Snyder, M., Bacchetta, R., Nadeau, K. C.
  2014; 9 (1)

- **Identification of STAT5A and STAT5B target genes in human T cells.** *PloS one*
  Kanai, T., Seki, S., Jenks, J. A., Kohli, A., Kawli, T., Martin, D. P., Snyder, M., Bacchetta, R., Nadeau, K. C.
  2014; 9 (1)

- **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

- **IL-21 signalling via STAT3 primes human naive B cells to respond to IL-2 to enhance their differentiation into plasmablasts** *BLOOD*
  Berglund, L. J., Avery, D. T., Ma, C. S., Moens, L., Deenick, E. K., Bustamante, J., Boisson-Dupuis, S., Wong, M., Adelstein, S., Arkwright, P. D., Bacchetta, R., Bezrodnik, L., Dadi, et al
  2013; 122 (24): 3940-3950

- **Combined DOCK8 and CLEC7A mutations causing immunodeficiency in 3 brothers with diarrhea, eczema, and infections** *JOURNAL OF ALLERGY AND CLINICAL IMMUNOLOGY*
  Dinwiddie, D. L., Kingsmore, S. F., Caracciolo, S., Rossi, G., Moratto, D., Mazza, C., Sabelli, C., Bacchetta, R., Passerini, L., Magri, C., Bell, C. J., Miller, N. A., Hateley, et al
  2013; 131 (2): 594-597

- **Immunodeficiency with autoimmunity: beyond the paradox.** *Frontiers in immunology*
  Bacchetta, R., Notarangelo, L. D.
  2013; 4: 77-?