



Agnieszka Czechowicz, MD, PhD

Assistant Professor of Pediatrics (Stem Cell Transplantation)
Pediatrics - Stem Cell Transplantation

 Curriculum Vitae available Online

CONTACT INFORMATION

- **Administrative Contact**

Paula Vasquez - Administrative Associate

Email paulav22@stanford.edu

Tel (650) 723-5535

Bio

BIO

Prof. Agnieszka Czechowicz is a physician-scientist and entrepreneur with 20 years of experience in stem cell biology and translational research. Dr. Czechowicz is a faculty member in Stanford University's Department of Pediatrics, Division of Hematology, Oncology, Stem Cell Transplantation and Regenerative Medicine and is also a member of Stanford's Institute for Stem Cell Biology and Regenerative Medicine.

Dr. Czechowicz's primary clinical interests are in bone marrow failure with a focus on Fanconi anemia and other diverse diseases that necessitate or could benefit from stem cell transplantation. Her primary research interests are in hematopoietic stem cell biology, transplantation, gene-therapy/gene-editing and regenerative medicine. Multiple pre-clinical and clinical therapies are in development based upon her studies.

Dr. Czechowicz's pioneering work has led to critical developments within the life sciences ecosystem. She was the scientific co-founder of Magenta Therapeutics which amongst other things was developing antibody-drug-conjugates for conditioning for hematopoietic stem cell transplantation, and parallel antibody-based conditioning also based upon her work are additionally being pursued by Jasper Therapeutics, Forty Seven Inc (acquired by Gilead in 2020) and Inograft Biotherapeutics. Her efforts have also led to important patents that are assigned to Editas Medicine and Decibel Therapeutics. Previously, Dr. Czechowicz spent nearly a decade as a consultant at Third Rock Ventures, where she assisted and started companies including Global Blood Therapeutics, Bluebird Bio, Editas Medicine, Decibel Therapeutics and Magenta Therapeutics. She has also advised Prime Medicines, Beam Therapeutics, Land Medicine and Spotlight Therapeutics.

Dr. Czechowicz completed her Ph.D. work in Developmental Biology at Stanford University with Prof. Irv Weissman. She then completed her residency in Pediatrics at the Boston Children's Hospital, and subsequently pursued subspecialty training in Pediatric Hematology and Oncology at the Dana Farber Cancer Institute while simultaneously conducting postdoctoral research with Prof. Derrick Rossi in collaboration with Prof. David Scadden. She received her M.D., Ph.D., and Bachelor's Degree from Stanford University.

Dr. Czechowicz is a strong physician-scientist and advocate of translational research. She has done pioneering work showing that hematopoietic stem cell depletion is a critical component to donor hematopoietic stem cell engraftment. She has also led several clinical trials exploring new cell and gene therapy treatments for Fanconi Anemia. She is passionate about mentoring and training future generations of physicians and scientists, and is very supportive of helping diverse trainees on various traditional and non-traditional career paths. Dr. Czechowicz can best be reached through her administrative assistant Paula Vasquez (paulav22@stanford.edu).

Press releases on her efforts can be found here:

<https://med.stanford.edu/news/all-news/2019/02/antibody-drug-combo-may-obviate-need-for-tissue-matching.html>

<http://med.stanford.edu/news/all-news/2019/02/antibody-could-increase-cure-rate-for-blood-immune-disorders.html>

<https://stanmed.stanford.edu/reducing-toxicity-children-stem-cell-transplants/>

<https://med.stanford.edu/news/insights/2026/03/fanconi-anemia-prenatal-stem-cell-transplant-trial.html>

ACADEMIC APPOINTMENTS

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

ADMINISTRATIVE APPOINTMENTS

- Member, Center for Definitive and Curative Medicine (CDCM), (2019- present)
- Member, Stanford Institute for Stem Cell Biology and Regenerative Medicine, (2018- present)
- Member, Stanford Diabetes Research Center, (2018- present)

HONORS AND AWARDS

- Lorry I. Lokey Faculty Scholar, Stanford University School of Medicine (2019- present)

PROFESSIONAL EDUCATION

- Clinical Fellowship III, Stanford University , Pediatric Hematology/Oncology/Transplant (2017)
- Clinical Fellowship I-II, Dana Farber Cancer Institute , Pediatric Hematology/Oncology/Transplant (2016)
- Clinical Residency, Boston Children's Hospital , Pediatrics (2014)

PATENTS

- Irving L. Weissman, Agnieszka Czechowicz, Deepta Bhattacharya, Daniel Kraft. "United States Patent Application US12447634 Selective immunodepletion of endogenous stem cell niche for engraftment", Leland Stanford Junior University
- Alexandra Glucksmann, Deborah Palestrant, Louis Anthony Tartaglia, Jordi Mata-Fink, Agnieszka Czechowicz, Alexis Borisy. "United States Patent Application US14536319 CRISPR-RELATED METHODS AND COMPOSITIONS WITH GOVERNING gRNAs", EDITAS MEDICINE Inc, University of Iowa Research Foundation (UIRF), Massachusetts Institute of Technology, Broad Institute Inc
- David T. Scadden, Rahul Palchaudhuri, Derrick J. Rossi, Agnieszka Czechowicz. "United States Patent Application US15148837 Compositions and methods for non-myeloablative conditioning", President And Fellows Of Harvard College, The General Hospital Corporation, The Children's Medical Center Corporation
- Derrick J. Rossi, Agnieszka D. Czechowicz, Philip M. Murphy, Zhanzhao Li. "United States Patent US20250345450A1 Antibody-mediated conditioning with immunosuppression to enable allogeneic transplantation", US Department of Health and Human Services
- Albert Edge, Michael Venuti, Agnieszka Czechowicz. ""United States Patent Application US62288958 Expansion and differentiation of inner ear supporting cells and methods of use thereof"", Massachusetts Eye and Ear Infirmary, Decibel Therapeutics

Research & Scholarship

CURRENT RESEARCH AND SCHOLARLY INTERESTS

Dr. Czechowicz's research is aimed primarily at understanding how hematopoietic stem cells interact with their microenvironment in order to subsequently modulate these interactions to ultimately improve bone marrow transplantation and unlock biological secrets that further enable regenerative medicine broadly. She is interested in increasing our basic science understanding of these interactions and also developing new novel therapies that stem from this work to expand treatment options for a wide variety of pediatric and adult diseases. Her group is primarily focused on studying the cell surface receptors on hematopoietic stem/progenitor cells and bone marrow stromal cells, and is actively learning how manipulating these can alter cell state and cell fate. Her group is using cells and serum from both mice and primary specimens from healthy and diseased patients for these studies and using a variety of exciting new tools and methods to unlock future discoveries. There are many exciting opportunities that stem from her work across a variety of disease states ranging from rare genetic diseases, autoimmune diseases, solid organ transplantation, microbiome and cancer. While her group is primarily focused on blood and immune diseases, the expanded potential of this work is much broader and can be applied to other organ systems as well and she is very eager to develop collaborations across disease areas.

Dr. Czechowicz has also been part of the initial founding team of several companies including Global Blood Therapeutics, Editas Medicine, Decibel Therapeutics, Magenta Therapeutics, and Inograft Biotherapeutics and advises multiple other transformative companies. As a true physician scientist, she has done pioneering work showing that hematopoietic stem cell depletion is a critical component to donor hematopoietic stem cell engraftment, and multiple pre-clinical and clinical therapies are in development based upon her studies. Stanford has had several clinical trials derived from Dr. Czechowicz's research.

Research Interests: Hematopoietic Stem Cells (HSC), Hematopoietic Stem Cell Transplantation (HSCT), Monoclonal Antibodies, Immunotoxins, Cell Cycle, Cell Fate, Cell Membrane, Cell Surface Antigens, Microenvironment, Stem Cell Niche, Cell Proliferation, Stem Cell Quiescence, DNA Damage, DNA Repair, Rare Genetic Diseases, Bone Marrow Failure, Aplastic Anemia, Genomics, Fanconi Anemia (FA), Immunodeficiency (SCID), Gastrointestinal Stromal Tumors (GIST), Rhabdomyosarcoma, Neuroblastoma, Myelodysplastic Syndrome (MDS), Acute Myeloid Leukemia (AML), Graft vs Host Disease (GVHD), Immune Tolerance, Histocompatibility Testing, Immunologic Deficiency Syndromes, Hemoglobinopathies, Sickle Cell Anemia, Diamond Blackfan Anemia, Transplantation Conditioning, Immune Tolerance, Gene Therapy, Gene-Editing, Base-Editing, Prime-Editing, Cytokines, Cytokine Receptors, Serum, Clinical Trials, Autoimmune diseases, Multiple Sclerosis, Microbiome, Cancer, Cell Therapy, Allogenic Bone Marrow Transplantation (BMT), Metabolic Diseases, Hurler Syndrome.

For more information, please visit our lab webpage: <http://med.stanford.edu/czechowiczlab.html>

CLINICAL TRIALS

- A Clinical Trial to Evaluate the Safety of RP-L102 in Pediatric Subjects With Fanconi Anemia Subtype A, Not Recruiting
- Gene Therapy for Fanconi Anemia, Complementation Group A, Not Recruiting

Teaching

COURSES

2024-25

- Biology and Disease of Hematopoiesis: IMMUNOL 223, STEMREM 223 (Win)

2023-24

- Biology and Disease of Hematopoiesis: IMMUNOL 223, STEMREM 223 (Win)

2022-23

- Biology and Disease of Hematopoiesis: IMMUNOL 223, STEMREM 223 (Win)

STANFORD ADVISEES

Doctoral Dissertation Reader (AC)

Maria Korah, Julie Sanchez, Archana Shankar

Postdoctoral Faculty Sponsor

Shan Huang, Charmaine Fay Soco, Aaron Tan

Doctoral Dissertation Advisor (AC)

Quenton Bubbs, Joseph Ward

Doctoral Dissertation Co-Advisor (AC)

Andrew Burden

GRADUATE AND FELLOWSHIP PROGRAM AFFILIATIONS

- Cancer Biology (Phd Program)
- Cancer Biology (Scholarly Concentration Application)
- Developmental Biology (Phd Program)
- Hematology (Fellowship Program)
- Immunology (Scholarly Concentration Application)
- Immunology (Phd Program)
- Maternal-Fetal Medicine (Fellowship Program)
- Molecular and Genetic Medicine (Fellowship Program)
- Pediatric Hem/Onc (Fellowship Program)
- Pediatric Nonmalignant Hematology and Stem Cell Biology Training Grant (Fellowship Program)
- Pediatric Surgery (Fellowship Program)
- Stem Cell Biology and Regenerative Medicine (Phd Program)
- Women's Health - Sexual & Gender Minority Health (Scholarly Concentration Application)

Publications

PUBLICATIONS

- **Attitudes Toward Prenatal Interventions in the Fanconi Anemia Community.** *Prenatal diagnosis*
Lum, T., Lee, C., MacKenzie, T., Lianoglou, B., Czechowicz, A.
2026
- **GFP-on mouse model for interrogation of in vivo gene editing.** *Nature communications*
Dib, C., Queenan, J. A., Swartzrock, L., Willner, H., Denis, M., Ahmed, N., Moulana Zada, F., Borges, B., Charlesworth, C. T., Lum, T., Yates, B. P., Kwon, C. Y., Scorzo, et al
2025; 16 (1): 7017
- **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

- **Clinical Hematopoietic Stem Cell-Based Gene Therapy.** *Molecular therapy : the journal of the American Society of Gene Therapy*
John, T., Czechowicz, A.
2025
- **Robust Immune Reconstitution in Fanconi Anemia Mice Post in Utero Hematopoietic Stem Cell Transplantation**
Swartzrock, L., Pan, W., Krampf, M. R., Hoang, H., Denis, M., Cool, T., Ho, K., Steele, M., Brown, B., Han, J., Mackenzie, T. C., Czechowicz, A. D.
ELSEVIER.2024: 4095-4096
- **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., Girsan, A., Blumenfeld, et al
2024
- **Hematopoiesis post anti-CD117 monoclonal antibody treatment in wild-type and Fanconi anemia settings.** *Haematologica*
Denis, M., Swartzrock, L., Willner, H., Bubb, Q. R., Haslett, E., Chan, Y. Y., Chen, A., Krampf, M. R., Czechowicz, A. D.
2024
- **Radiation and Busulfan-Free Hematopoietic Stem Cell Transplantation Using Briquilimab (JSP191) Anti-CD117 Antibody-Conditioning, Transient Immunosuppression and TCR $\alpha \beta$ + 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
- **Non-Genotoxic Restoration of the Hematolymphoid System in Fanconi Anemia Mice through Antibody-Mediated Hematopoietic Stem Cell Transplantation**
Chan, Y., Ho, P., Swartzrock, L., Rayburn, M., Nofal, R., Thongthip, S., Weinberg, K. I., Czechowicz, A.
AMER SOC HEMATOLOGY.2022: 7342-7344
- **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
- **Anti-CD117 antibody depletes normal and myelodysplastic syndrome human hematopoietic stem cells in xenografted mice.** *Blood*
Pang, W. W., Czechowicz, A., Logan, A. C., Bhardwaj, R., Poyser, J., Park, C. Y., Weissman, I. L., Shizuru, J. A.
2019
- **Hematopoietic chimerism and donor-specific skin allograft tolerance after non-genotoxic CD117 antibody-drug-conjugate conditioning in MHC-mismatched allotransplantation.** *Nature communications*
Li, Z., Czechowicz, A., Scheck, A., Rossi, D. J., Murphy, P. M.
2019; 10 (1): 616
- **Selective hematopoietic stem cell ablation using CD117-antibody-drug-conjugates enables safe and effective transplantation with immunity preservation.** *Nature communications*
Czechowicz, A., Palchadhuri, R., Scheck, A., Hu, Y., Hoggatt, J., Saez, B., Pang, W. W., Mansour, M. K., Tate, T. A., Chan, Y. Y., Walck, E., Wernig, G., Shizuru, et al
2019; 10 (1): 617
- **Clonal-level lineage commitment pathways of hematopoietic stem cells in vivo.** *Proceedings of the National Academy of Sciences of the United States of America*
Lu, R., Czechowicz, A., Seita, J., Jiang, D., Weissman, I. L.
2019
- **Efficient transplantation via antibody-based clearance of hematopoietic stem cell niches** *SCIENCE*
Czechowicz, A., Kraft, D., Weissman, I. L., Bhattacharya, D.
2007; 318 (5854): 1296-1299
- **Restoration of the immune system with base editing and non-genotoxic conditioning in a Rag2 point-mutant mouse model.** *Molecular therapy : the journal of the American Society of Gene Therapy*
Dib, C., Queenan, J. A., Willner, H., Swartzrock, L., Charlesworth, C. T., Denis, M., Davis, J. R., Mepani, R., Ho, K., Castro, M., Wilson, R. C., Nakauchi, H., Liu, et al

2026

- **Development and characterization of anti-CXCR4 chimeric antigen receptor T cells.** *Translational oncology*
Seir, G., Bubb, Q. R., Sotillo, E., Gruber, T., Richards, R. M., Czechowicz, A.
2026; 66: 102711
- **Targeting CD117 with engineered invariant natural killer T cells (iNKT Cells) as a novel approach for acute myeloid leukemia treatment**
Boonchalermvichian, C., Yan, H., Baker, J., Bubb, Q., Gupta, B., Czechowicz, A., Negrin, R.
ELSEVIER.2025: 7662-7663
- **Non-genotoxic conditioning to increase donor chimerism levels in a mismatched murine transplant model for sickle cell disease**
Ali, M., Joseph, C., Gaskin, D., Xu, X., Kabore, D., Mukendi, J., Idowu, A., Ahmed, M., Scheck, A., Czechowicz, A., Fitzhugh, C.
ELSEVIER.2025: 5858-5859
- **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
- **Transcriptomic Diversity of Pediatric Acute Myeloid Leukemia Genetic Drivers Correlates With Clinical Outcome and Expression of Stemness-Related Genes.** *Cancer medicine*
Bubb, Q. R., Sotillo, E., Richards, R. M., Mackall, C. L., Gruber, T. A., Czechowicz, A.
2025; 14 (21): e71325
- **Editorial: Mobilization of hematopoietic cells from the bone marrow to the peripheral blood: Challenges and new therapeutic targets.** *Frontiers in pharmacology*
Szade, A., Gulati, G. S., Czechowicz, A. D.
2025; 16: 1592559
- **Development of multivalent CAR T cells as dual immunotherapy and conditioning agents** *MOLECULAR THERAPY ONCOLOGY*
Bubb, Q., Balood, M., Seir, G., Swartzrock, L., Haslett, E., Ho, K., Xu, P., Wiltz, S. G., Sotillo, E., Gruber, T. A., Richards, R. M., Mackall, C. L., Czechowicz, et al
2025; 33 (1)
- **Development of multivalent CAR T cells as dual immunotherapy and conditioning agents.** *Molecular therapy. Oncology*
Bubb, Q. R., Balood, M., Seir, G. E., Swartzrock, L., Haslett, E., Ho, K., Xu, P., Wiltz, S. G., Sotillo, E., Gruber, T. A., Richards, R. M., Mackall, C. L., Czechowicz, et al
2025; 33 (1): 200944
- **Highly efficient in vivo hematopoietic stem cell transduction using an optimized self-complementary adeno-associated virus** *MOLECULAR THERAPY METHODS & CLINICAL DEVELOPMENT*
Charlesworth, C. T., Homma, S., Amaya, A. K., Dib, C., Vaidyanathan, S., Tan, T., Miyauchi, M., Nakauchi, Y., Suchy, F. P., Wang, S., Igarashi, K. J., Cromer, M., Dudek, et al
2025; 33 (1)
- **Highly efficient in vivo hematopoietic stem cell transduction using an optimized self-complementary adeno-associated virus.** *Molecular therapy. Methods & clinical development*
Charlesworth, C. T., Homma, S., Amaya, A. K., Dib, C., Vaidyanathan, S., Tan, T. K., Miyauchi, M., Nakauchi, Y., Suchy, F. P., Wang, S., Igarashi, K. J., Cromer, M. K., Dudek, et al
2025; 33 (1): 101438
- **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
- **Disease Correction of a Diamond-Blackfan Anemia Mouse Model Using Non-Genotoxic Conditioning and Hematopoietic Stem Cell Transplantation**
Swartzrock, L., Liu, Y., Hoang, H., Ho, K., Neoman, N., Krampf, M. R., Sakamoto, K. M., Czechowicz, A. D.
ELSEVIER.2024: 194-195

- **Evaluation of Bone Marrow in Fanconi Anemia Patients Treated with Briquilimab Antibody-Based Conditioning and TCR $\alpha\beta$ B-Cell Depleted Haploidentical Grafts**
Soco, C., Krampf, M. R., Chan, Y., Hoang, H., Kunte, N., Saini, G., Weinberg, K. I., Parkman, R., Bertaina, A., Agarwal, R., Porteus, M., Czechowicz, A. D.
ELSEVIER.2024: 2015-2016
- **Targeted hematopoietic stem cell depletion through SCF-blockade.** *Stem cell research & therapy*
Chan, Y. Y., Ho, P. Y., Dib, C., Swartzrock, L., Rayburn, M., Willner, H., Ko, E., Ho, K., Down, J. D., Wilkinson, A. C., Nakauchi, H., Denis, M., Cool, et al
2024; 15 (1): 387
- **Development of Extracellularly Linked Concatemeric Trivalent Cytokine (ELeCTriC) Chimeric Antigen Receptor (CAR) T-Cells as Dual Conditioning and Immunotherapeutic Agents**
Bubb, Q. R., Balood, M., Seir, G. E., Swartzrock, L., Haslett, E., Ho, K., Wiltz, S. G., Sotillo, E., Richards, R. M., Gruber, T. A., Mackall, C. L., Czechowicz, A.
CELL PRESS.2024: 75
- **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
- **Recruitment and Retention of Hematopoietic Cell Transplantation and Cellular Therapy Physicians: A Report from the ASTCT Talent Acquisition Task Force.** *Transplantation and cellular therapy*
Sharma, A., Czechowicz, A., Mavers, M., Chao, N., DiPersio, J., Reddy, P., Perales, M. A., Smith, M.
2024
- **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
SPRINGER NATURE.2023: 276-277
- **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 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
- **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
- **Non-Genotoxic Restoration of the Hematolymphoid System in Fanconi Anemia.** *Transplantation and cellular therapy*
Chan, Y. Y., Ho, P. Y., Swartzrock, L., Rayburn, M., Nofal, R., Thongthip, S., Weinberg, K. I., Czechowicz, A.
2022
- **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

- **Peptide nucleic acid-dependent artifact can lead to false-positive triplex gene editing signals.** *Proceedings of the National Academy of Sciences of the United States of America*
Ho, P. Y., Zhang, Z., Hayes, M. E., Curd, A., Dib, C., Rayburn, M., Tam, S. N., Srivastava, T., Hriniaik, B., Li, X., Leonard, S., Wang, L., Tarighat, et al
2021; 118 (45)
- **Safe and Effective In Vivo Targeting and Gene Editing in Hematopoietic Stem Cells: Strategies for Accelerating Development National Institutes of Health/Bill & Melinda Gates Foundation Expert Scientific Roundtable Webinar Meeting.** *Human gene therapy*
Cannon, P., Asokan, A., Czechowicz, A., Hammond, P., Kohn, D. B., Lieber, A., Malik, P., Marks, P., Porteus, M., Verhoeven, E., Weissman, D., Weissman, I., Kiem, et al
2021
- **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
- **Changing the Natural History of Fanconi Anemia Complementation Group-A with Gene Therapy: Early Results of US Phase I Study of Lentiviral-Mediated Ex-Vivo FANCA Gene Insertion in Human Stem and Progenitor Cells**
Czechowicz, A., Rio, P., Bueren, J. E., Beard, B., Nicoletti, E., Schwartz, J., Soni, S.
ELSEVIER SCIENCE INC.2020: S39–S40
- **Changing the Natural History of Fanconi Anemia Complementation Group-A with Gene Therapy: Early Results of US Phase I Study of Lentiviral-Mediated Ex-Vivo FANCA Gene Insertion in Human Stem and Progenitor Cells**
Czechowicz, A., Roncarolo, M., Beard, B. C., Law, K., Nicoletti, E., Rio, P., Bueren, J. A., Schwartz, J. D., Soni, S.
AMER SOC HEMATOLOGY.2019
- **Nongenotoxic antibody-drug conjugate conditioning enables safe and effective platelet gene therapy of hemophilia A mice.** *Blood advances*
Gao, C., Schroeder, J. A., Xue, F., Jing, W., Cai, Y., Scheck, A., Subramaniam, S., Rao, S., Weiler, H., Czechowicz, A., Shi, Q.
2019; 3 (18): 2700–2711
- **Anti-CD117 antibody depletes normal and myelodysplastic syndrome human hematopoietic stem cells in xenografted mice** *BLOOD*
Pang, W. W., Czechowicz, A., Logan, A. C., Bhardwaj, R., Poyser, J., Park, C. Y., Weissman, I. L., Shizuru, J. A.
2019; 133 (19): 2069–78
- **Anti-human CD117 antibody-mediated bone marrow niche clearance in nonhuman primates and humanized NSG mice** *BLOOD*
Kwon, H., Logan, A. C., Chhabra, A., Pang, W. W., Czechowicz, A., Tate, K., Le, A., Poyser, J., Hollis, R., Kelly, B. V., Kohn, D. B., Weissman, I. L., Prohaska, et al
2019; 133 (19): 2104–8
- **Hematopoietic chimerism and donor-specific skin allograft tolerance after non-genotoxic CD117 antibody-drug-conjugate conditioning in MHC-mismatched allotransplantation** *NATURE COMMUNICATIONS*
Li, Z., Czechowicz, A., Scheck, A., Rossi, D. J., Murphy, P. M.
2019; 10
- **Selective hematopoietic stem cell ablation using CD117-antibody-drug-conjugates enables safe and effective transplantation with immunity preservation** *NATURE COMMUNICATIONS*
Czechowicz, A., Palchaudhuri, R., Scheck, A., Hu, Y., Hoggatt, J., Saez, B., Pang, W. W., Mansour, M. K., Tate, T. A., Chan, Y., Walck, E., Wernig, G., Shizuru, et al
2019; 10
- **Anti-human CD117 antibody-mediated bone marrow niche clearance in non-human primates and humanized NSG mice.** *Blood*
Kwon, H., Logan, A. C., Chhabra, A., Pang, W. W., Czechowicz, A., Tate, K., Le, A., Poyser, J., Hollis, R., Kelly, B. V., Kohn, D. B., Weissman, I. L., Prohaska, et al
2019
- **The MarrowMiner: A Novel Minimally Invasive and Effective Device for the Harvest of Bone Marrow.** *Biology of blood and marrow transplantation : journal of the American Society for Blood and Marrow Transplantation*
Kraft, D. n., Walck, E. n., Carrasco, A. n., Crocker, M. n., Song, L. n., Long, M. n., Mosse, M. n., Nadeem, B. n., Imanbyev, G. n., Czechowicz, A. n., McCullough, M. n.

2019

- **Selective HSC-Ablation using Anti-CD117 Antibody Drug Conjugate Enables Safe and Effective Murine and Human Hematopoietic Stem Cell Transplantation**
Czechowicz, A., Palchaudhuri, R., Scheck, A., Hu, Y., Hoggatt, J., Saez, B., Mansour, M. K., Winau, F., Scadden, D. T., Rossi, D. J.
NATURE PUBLISHING GROUP.2018: 83-84
- **Safe and Effective Platelet-Targeted Gene Therapy of Hemophilia A Enabled Using Non-Genotoxic, Antibody-Drug-Conjugate Conditioning**
Gao, C., Schroeder, J., Czechowicz, A., Shi, Q.
CELL PRESS.2018: 25
- **Selective HSC-Ablation Using CD117 Antibody-Drug-Conjugates Enables Safe and Effective Murine and Human Hematopoietic Stem Cell Transplantation**
Czechowicz, A., Palchaudhuri, R., Scheck, A., Hu, Y., Hoggatt, J., Saez, B., Pang, W. W., Mansour, M. K., Shizuru, J. A., Winau, F., Scadden, D. T., Rossi, D. J.
CELL PRESS.2018: 23–24
- **Immune Sparing Conditioning for BMT/HSCT Using Anti-Ckit Immunotoxins**
Czechowicz, A., Palchaudhuri, R., Scheck, A., Hu, Y., Winau, F., Hoggatt, J., Saez, B., Mansour, M. K., Sykes, D., Scadden, D., Rossi, D.
ELSEVIER SCIENCE INC.2018: S60–S61
- **Anti-Human CD117 Antibodies Mediate Clearance of Myelodysplastic Syndrome Hematopoietic Stem Cells and Facilitate Establishment of Normal Hematopoiesis in Transplantation**
Pang, W. W., Czechowicz, A., Poyser, J., Park, C. Y., Weissman, I. L., Shizuru, J. A.
ELSEVIER SCIENCE INC.2018: S230–S231
- **Non-genotoxic conditioning for hematopoietic stem cell transplantation using a hematopoietic-cell-specific internalizing immunotoxin** *NATURE BIOTECHNOLOGY*
Palchaudhuri, R., Saez, B., Hoggatt, J., Schajnovitz, A., Sykes, D. B., Tate, T. A., Czechowicz, A., Kfoury, Y., Ruchika, F. N., Rossi, D. J., Verdine, G. L., Mansour, M. K., Scadden, et al
2016; 34 (7): 738-?
- **A trial of plerixafor adjunctive therapy in allogeneic hematopoietic cell transplantation with minimal conditioning for severe combined immunodeficiency** *PEDIATRIC TRANSPLANTATION*
Dvorak, C. C., Horn, B. N., Puck, J. M., Czechowicz, A., Shizuru, J. A., Ko, R. M., Cowan, M. J.
2014; 18 (6): 602-608
- **A trial of alemtuzumab adjunctive therapy in allogeneic hematopoietic cell transplantation with minimal conditioning for severe combined immunodeficiency** *PEDIATRIC TRANSPLANTATION*
Dvorak, C. C., Horn, B. N., Puck, J. M., Adams, S., Veys, P., Czechowicz, A., Cowan, M. J.
2014; 18 (6): 609-616
- **In utero depletion of fetal hematopoietic stem cells improves engraftment after neonatal transplantation in mice.** *Blood*
Derderian, S. C., Togarrati, P. P., King, C., Moradi, P. W., Reynaud, D., Czechowicz, A., Weissman, I. L., MacKenzie, T. C.
2014; 124 (6): 973-980
- **Anti-KIT monoclonal antibody inhibits imatinib-resistant gastrointestinal stromal tumor growth** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*
Edris, B., Willingham, S. B., Weiskopf, K., Volkmer, A. K., Volkmer, J., Muehlenberg, T., Montgomery, K. D., Contreras-Trujillo, H., Czechowicz, A., Fletcher, J. A., West, R. B., Weissman, I. L., van de Rijn, et al
2013; 110 (9): 3501-3506
- **Anti-CD117 (c-Kit) Monoclonal Antibodies Deplete Human Hematopoietic Stem Cells and Facilitate Their Replacement in Humanized NOD/SCID/IL2R gamma(-/-) Mice: A Non-Toxic Conditioning Regimen for Allotransplantation**
Logan, A. C., Czechowicz, A., Kelley, B. V., Thway, T. M., Magana, I., Krampf, M. R., Poyser, J., Hollis, R. P., Kohn, D. B., Weissman, I. L., Shizuru, J. A.
AMER SOC HEMATOLOGY.2012
- **Clonal Level Lineage Commitment of Mouse Hematopoietic Stem Cells in Vivo** *54th Annual Meeting and Exposition of the American-Society-of-Hematology (ASH)*
Lu, R., Czechowicz, A., Seita, J., Weissman, I. L.
AMER SOC HEMATOLOGY.2012

- **Transplantation Conditioning Regimens Induce Different Hematopoietic Stem Cell Differentiation in Mice At the Clonal Level** *53rd Annual Meeting and Exposition of the American-Society-of-Hematology (ASH)*
Lu, R., Czechowicz, A., Weissman, I. L.
AMER SOC HEMATOLOGY.2011: 67–67
- **Purified Hematopoietic Stem Cell Transplantation: The Next Generation of Blood and Immune Replacement** *HEMATOLOGY-ONCOLOGY CLINICS OF NORTH AMERICA*
Czechowicz, A., Weissman, I. L.
2011; 25 (1): 75-?
- **Targeted Clearance of Human Hematopoietic Stem Cell Niches Via Inhibition of SCF Signaling Using Monoclonal Antibody SR-1** *52nd Annual Meeting and Exposition of the American-Society-of-Hematology (ASH)*
Czechowicz, A., Bhardwaj, R., Park, C. Y., Weissman, I. L.
AMER SOC HEMATOLOGY.2010: 39–40
- **Inhibition of Mac-1 (CD11b/CD18) enhances tumor response to radiation by reducing myeloid cell recruitment** *PROCEEDINGS OF THE NATIONAL ACADEMY OF SCIENCES OF THE UNITED STATES OF AMERICA*
Ahn, G., Tseng, D., Liao, C., Dorie, M. J., Czechowicz, A., Brown, J. M.
2010; 107 (18): 8363-8368
- **Purified Hematopoietic Stem Cell Transplantation: The Next Generation of Blood and Immune Replacement** *IMMUNOLOGY AND ALLERGY CLINICS OF NORTH AMERICA*
Czechowicz, A., Weissman, I. L.
2010; 30 (2): 159-?
- **Niche recycling through division-independent egress of hematopoietic stem cells** *JOURNAL OF EXPERIMENTAL MEDICINE*
Bhattacharya, D., Czechowicz, A., Ooi, A. G., Rossi, D. J., Bryder, D., Weissman, I. L.
2009; 206 (12): 2837-2850
- **Niche Recycling through Division-Independent Egress of Hematopoietic Stem Cells.** *51st Annual Meeting and Exposition of the American-Society-of-Hematology*
Czechowicz, A., Bhattacharya, D., Ooi, L., Rossi, D. J., Bryder, D., Weissman, I. L.
AMER SOC HEMATOLOGY.2009: 37–37
- **Hematopoietic stem cell quiescence attenuates DNA damage response and permits DNA damage accumulation during aging** *CELL CYCLE*
Rossi, D. J., Seita, J., Czechowicz, A., Bhattacharya, D., Bryder, D., Weissman, I. L.
2007; 6 (19): 2371-2376
- **Adult human hematopoietic cells differentiate into mature T cells via a CD3-4+8-intermediate within the mouse thymic microenvironment; A new model system for the study of human thymocyte development.** *47th Annual Meeting of the American-Society-of-Hematology*
Kraft, D. L., Czechowicz, A., Weissman, I. L.
AMER SOC HEMATOLOGY.2005: 155A–156A