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CAR T and Related Immune Effector Cell Therapies Workshop Sponsors

The American Society of Gene & Cell Therapy is honored to acknowledge Legend Bio and bluebird bio for their support of the CAR T Cell Therapies Workshop:
Legend Biotech is an integrated biopharmaceutical company specialized in the discovery and development of novel cell therapies, focused on hematologic malignancies, solid tumors, autoimmune and infectious diseases.

Our story is just beginning. We are already seeing success and are looking for you to help us write the next chapter. Do you have a passion for science and a commitment to improving patients’ lives? We are looking for people with a depth of scientific experience and an appreciation for the rapid pace and steady change in cell therapy.

Now is a unique chance to join us and help ignite the future of cell therapy.

Join the Legend legacy.

Connect with us:
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Program

10:00 AM – 10:10 AM

Welcome and Introductory Remarks
SPEAKER: Cameron Turtle, MBBS, PhD

10:10 AM – 12:05 PM

Clinical Long Term Follow-Up

Now that CAR T cells have been in clinical use for over 5 years, this session will focus on the clinical results and longer-term follow up of CAR T cell studies in hematologic malignancies, on the consensus grading systems that have emerged as more studies are developed and CAR T cells enter general practice, and on how clinical centers manage the administration and collection of long-term follow-up data required by regulatory bodies.

CHAIR: Cameron Turtle, MBBS, PhD
SPEAKERS:
Cameron Turtle, MBBS, PhD
Shannon Maude, MD, PhD
Jim Kochenderfer, MD
Sattva Neelapu, MD
Sarah Nikiforow, MD, PhD
Matt Frigault, MD

12:05 PM – 1 PM

Lunch

1 PM – 3:00 PM

Novel Engineering and Gene Editing

This session will focus on the scientific innovation as well as the safety and regulatory aspects of next-generation genetic engineering strategies that are in development that will enable complex strategies to target novel antigens not amenable to standard autologous CAR T cell therapies or to enable allogeneic cellular products.

CHAIR: Marcela Maus, MD, PhD
SPEAKERS:
Wendell Lim, PhD
Justin Eyquem, PhD
Saar Gill, MD, PhD
J. Keith Joung, MD, PhD

3 PM – 3:30 PM

Coffee Break

3:30 PM – 6 PM

Beyond Autologous CAR-T Cells for Cancer

CAR T cells are in wide clinical use in B cell cancers as autologous cellular products that have been transduced to express a single transgene. This session will focus on innovations with alternative cellular products and indications, such as allogeneic NK cells, stem cells, and potential uses of engineered cell therapies on non-cancer indications.

CHAIR: Marcela Maus, MD, PhD
SPEAKERS:
Katy Rezvani, MD, PhD
Michel Sadelain, MD, PhD
Marcela Maus, MD, PhD
Mike Milone, MD, PhD
Cyril Konto, MD
Co-Chairs

Marcela Maus, MD, PhD
Massachusetts General Hospital Cancer Center
Boston, MA

Cameron Turtle, MBBS, PhD
Fred Hutchinson Cancer Research Center
Seattle, WA

Committee Members

Kara Wacker, MBA
Foundation for the Accreditation of Cellular Therapy
Omaha, NE

Phyllis I. Warkentin, MD
Foundation for the Accreditation of Cellular Therapy
Omaha, NE

Faculty Listing

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University of California, San Francisco
San Francisco, CA

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Massachusetts General Hospital Cancer Center
Boston, MA

Saar Gill, MD, PhD
University of Pennsylvania
Philadelphia, PA

J. Keith Joung, MD, PhD
Massachusetts General Hospital
Boston, MA

James Kochenderfer, MD
National Cancer Institute
Bethesda, MD

Cyril Konto, MD
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Philadelphia, PA

Sattva Neelapu, MD
The University of Texas MD Anderson Cancer Center
Houston, TX

Sarah Nikiforow, MD, PhD
Dana-Farber Cancer Institute
Boston, MA

Katy Rezvani, MD, PhD
The University of Texas MD Anderson Cancer Center
Houston, TX

Michel Sadelain, MD, PhD
Memorial Sloan Kettering Cancer Center
New York, NY

Cameron Turtle, MBBS, PhD
Fred Hutchinson Cancer Research Center
Seattle, WA
Faculty Bios

Justin Eyquem, PhD
Justin Eyquem received his PhD from the University of Paris-Diderot in collaboration with the biotech company Cellectis. During his PhD, he participated to the development of gene editing tools such as Meganuclease or TALEN in primary human cells and notably identified genomic location for safe integration of therapeutic genes. In 2014, he joined Michel Sadelain’s lab at the MSKCC and used CRISPR/Cas9 to engineer CAR T cells. He showed how targeting CAR transgene into specific loci enhance T cell efficacy, advance CAR immuno-biology and facilitate T cell manufacturing. Early 2019, he opened his lab in the department of Microbiology and Immunology at UCSF where he is developing a gene editing platform to enhance CAR T cell functions in solid tumors.

Matthew Frigault, MD
Dr. Frigault is a medical oncologist in the Hematologic Malignancy Program at the Massachusetts General Hospital Cancer Center, as well as Assistant Director of the Cellular Immunotherapy Program. In addition, he serves as an instructor at Harvard Medical School. Dr. Frigault completed his oncology fellowship at the combined MGH/Dana-Farber Cancer Institute training program where he worked with Dr. Marcela Maus, head of the Cellular Immunotherapy Program. In addition, he serves as an instructor at Harvard Medical School. Dr. Frigault completed his oncology fellowship at the combined MGH/Dana-Farber Cancer Institute training program where he worked with Dr. Marcela Maus, head of the Cellular Immunotherapy Program at MGH.

Dr. Frigault received his Bachelor of Arts degree in Biology and Biological Sciences from the College of the Holy Cross, and his master’s and MD degrees from the University of Pennsylvania. His research experience includes pre-clinical development and correlative studies relevant to T-cell immunotherapy in the lab of Dr. Carl June while in graduate school at Penn. During his post-graduate training at Johns Hopkins, he focused on cellular therapies utilizing marrow infiltrating lymphocytes and chimeric switch receptors in the lab of Dr. Ivan Borrelo.

Dr. Frigault’s current clinical activities include attending on the inpatient bone marrow transplant and leukemia services, managing outpatient cellular therapy patients and overseeing the cellular therapy service. His current research is focused on the translational aspects of cellular therapy with the goal of developing the next generation of cellular therapies through phase I clinical trials.

Saar Gill, MD, PhD, FRACP
Dr. Gill obtained his medical degree and PhD in immunology from the University of Melbourne in Australia, and a post-doctoral fellowship in cellular therapy at Stanford University. Dr. Gill is now an assistant professor of medicine at the University of Pennsylvania where he specializes in the treatment of patients with leukemia and in bone marrow transplantation. He has led clinical trials of chimeric antigen receptor (CAR) T cell trials for chronic and acute leukemias. Dr. Gill’s research laboratory focuses on the interface between adoptive cellular therapy and genetic engineering.

J. Keith Joung, MD, PhD
J. Keith Joung is a leading innovator in the field of genome editing. He is currently Desmond and Ann Heathwood Research Scholar and Pathologist at Massachusetts General Hospital (MGH) and Professor of Pathology at Harvard Medical School. He is also a member of the Center for Cancer Research and the Center for Computational and Integrative Biology at MGH. Dr. Joung has been a pioneer in the development of important technologies for targeted genome editing and epigenetic editing of human cells. He has received numerous awards including an NIH Director’s Pioneer Award, an NIH Director’s Transformative Research Project R01 Award, the MGH Research Scholar Award, an NIH R35 MIRA (Maximizing Investigators Research Award), election into the American Association of University Pathologists, and designation as a “Highly Cited Researcher” in 2016, 2017, and 2018 by Thomson Reuters/Clarivate Analytics. He serves on the Board of Directors for the American Society of Gene and Cell Therapy and the editorial boards of Genome Biology, Human Gene Therapy, and Trends in Biotechnology. He has co-founded and advises multiple biotechnology companies including Editas Medicine, Beam Therapeutics, and Pairwise Plants. Dr. Joung holds a PhD. in genetics from Harvard University, an MD from Harvard Medical School and an A.B. in biochemical sciences from Harvard College.
Faculty Bios – continued

**James Kochenderfer, MD**

James Kochenderfer, MD is a Tenure-track Investigator and attending physician in the Experimental Transplantation and Immunology Branch (ETIB) of the National Cancer Institute. Dr. Kochenderfer’s career has combined laboratory research and clinical research aimed at developing new immune therapies for hematologic malignancies. For the past 12 years, Dr. Kochenderfer has focused on a full range of chimeric antigen receptor (CAR) research from designing and constructing new CARs to leading clinical trials. Dr. Kochenderfer’s ultimate goal for CAR T cell approaches is to cure currently incurable lymphoid malignancies by using autologous CAR-expressing T cells in combination with chemotherapy and other agents.

**Cyril Konto, MD**

Cyril Konto is Vice President of Clinical Development at Allogene Therapeutics. He leads the development of the anti-CD19 allogeneic CAR T program, including the UCART19 program in Acute Lymphoblastic Leukemia (ALL) and ALLO-501 program in Non-Hodgkin Lymphoma (NHL). He most recently ensured successful IND clearance for both ALLO-501 and ALLO-647, an anti-CD52 monoclonal antibody intended to be used prior to AlloCAR T™.

Cyril is a physician scientist with over 15 years of experience in the pharmaceutical industry in oncology, immuno-oncology early and late-stage clinical development, drug safety and medical affairs. He joined Allogene from Pfizer, where he held the position of Head of Early Immuno-Oncology Clinical Development. In that capacity, he oversaw portfolio management, business development and clinical development strategy for the allogeneic CAR T programs and other immuno-oncology assets.

Cyril obtained his MD in Medical Oncology from Paris Descartes University. From 2007-2015, he held various positions at Bristol-Myers Squibb including the role of Clinical Development Lead of the YERVOY® melanoma program. Cyril also served as an Adjunct Assistant Professor at the Pierre & Marie Curie University in Paris, France until recently.

**Wendell Lim, PhD**

Wendell Lim is the Byers Distinguished Professor and Chair of the Department of Cellular and Molecular Pharmacology at the University of California San Francisco, and an Investigator of the Howard Hughes Medical Institute. He received his A.B. in Chemistry, summa cum laude, from Harvard College, his PhD. in Biochemistry and Biophysics at the Massachusetts Institute of Technology and completed his postdoctoral training at Yale University. His research focuses on the design principles of molecular circuits that govern cell decision-making and responses. His lab has made contributions in understanding the molecular machinery of cell signaling and how molecular modules have been used in evolution to build novel new behaviors. Most recently he has been a pioneer in the emerging field of synthetic biology, exploring how these design principles can be harnessed to engineer cells with customized therapeutic response programs. He is an author of the textbook, Cell Signaling (Garland Science 2014) and was the founder of the cell therapy biotech startup, Cell Design Labs, which was acquired by Gilead Biosciences in 2017.

**Shannon Maude, MD, PhD**

Dr. Shannon Maude is an oncologist in the Cancer Immunotherapy Program at the Children’s Hospital of Philadelphia and an Assistant Professor of Pediatrics at the University of Pennsylvania Perelman School of Medicine. After earning her undergraduate degree in Biology from the University of Virginia, Dr. Maude received her MD and PhD. from the University of Pennsylvania School of Medicine, and completed her residency in pediatrics as well as a fellowship in pediatric hematology-oncology at the Children's Hospital of Philadelphia. Dr. Maude is Fellowship Director of the Cancer Immunotherapy and BMT Fellowship at Children's Hospital of Philadelphia and a Medical Director in the Center for Cellular Immunotherapies at the University of Pennsylvania and leads clinical trials of engineered T cell therapies for childhood cancers. Dr. Maude has a special interest in novel therapies for acute lymphoblastic leukemia, particularly targeted therapy approaches and engineered T cell therapy.
Faculty Bios – continued

**Marcela Maus, MD, PhD**

Marcela Maus, MD, PhD, is the Director of Cellular Immunotherapy at the Massachusetts General Hospital Cancer Center. Dr. Maus was recently recruited to the Massachusetts General Hospital Cancer Center to lead a new program in Cellular Immunotherapy. She is a member of the Center for Cancer Immunology and the Department of Medicine at the MGH, and she is an Assistant Professor at Harvard Medical School. Her laboratory is generating new forms of chimeric antigen receptors directed to new targets and bringing them to the clinical setting to treat patients with hematologic malignancies and solid tumors.

Dr. Maus trained in internal medicine at U. Penn and at Memorial Sloan Kettering as a hematologist and medical oncologist. Her post-doctoral works was with Michel Sadelain and Carl June, where she focused on pre-clinical development and correlative studies relevant to T cell immunotherapies, designing early-phase trials of T cell therapies for multiple myeloma, chronic lymphocytic leukemia, and glioblastoma.

**Michael Milone, MD, PhD**

Dr. Milone received his MD and PhD in experimental pathology in 1999 from New Jersey Medical School and UMDNJ-Graduate School of Biomedical Sciences. After an Internship in Internal Medicine at the Hospital of the University of Pennsylvania, he completed post-graduate medical training in Clinical Pathology, Transfusion Medicine and Laboratory Toxicology. Dr. Milone went on to pursue post-doctoral research training in cancer immunology and adoptive immunotherapy at the University of Pennsylvania in the Laboratory of Dr. Carl June where he performed basic research to develop CTL019 (tisagenlecleucel), the first FDA-approved genetically-engineered cell therapy. He is currently an Associate Professor of Pathology and Laboratory Medicine at the University of Pennsylvania School of Medicine. Dr. Milone’s laboratory is focused on the design of synthetic immunoreceptors and the development of adoptive T cell immunotherapies for cancer, autoimmunity and other non-malignant conditions.

**Sattva Neelapu, MD**

Dr. Sattva S Neelapu is a tenured Professor, Deputy Chair ad interim, and Director of Translational Research in the Department of Lymphoma and Myeloma at The University of Texas MD Anderson Cancer Center, Houston, Texas, USA. He is internationally recognized for his research in tumor immunology and immunotherapy in lymphoid malignancies. He completed his clinical fellowship in Medical Oncology and postdoctoral fellowship in tumor immunology and immunotherapy at the National Cancer Institute, National Institutes of Health, Bethesda, Maryland. As a physician-scientist at MD Anderson, he is focused on clinical and translational development of novel immunotherapies for B-cell malignancies. His laboratory characterized some of the major immunosuppressive mechanisms in the tumor microenvironment of B-cell malignancies, identified TCL1 as a novel shared tumor-associated antigen for B-cell lymphomas, and investigated novel targets for CAR T-cell therapy in lymphoma and myeloma. Recently, his work on the pivotal trial of axicabtagene ciloleucel CD19 CAR T-cell therapy in aggressive B-cell lymphomas led to its FDA approval as the first CAR T therapy for lymphoma. He has authored or co-authored over 150 publications.

**Sarah Nikiforow, MD, PhD**

Dr. Nikiforow is currently a clinical instructor with the Stem Cell Transplant Program at Dana-Farber Cancer Institute, Assistant Medical Director of the Cell Manipulation Core Facility (CMCF), and Technical Director of DFCI’s Immune Effector Cell Program. Dr. Nikiforow earned her MD and a PhD in Immunobiology at Yale University working on the differential roles of CD4 and CD8 T cells in immune control over Epstein-Barr virus-induced B cell transformation.

Dr. Nikiforow has embarked on a translational research career focusing on immune reconstitution after stem cell transplant and therapeutic use of adoptive cellular products. Through the CMCF and as Principal Investigator of Phase I and II clinical trials, she is working to bring cellular therapies such as chimeric antigen-receptor T cells, genetically-modified stem cells, and regulatory T-cell infusions into the clinic at Dana-Farber. Her work with the International Society of Cellular Therapy, the Foundation for the Accreditation of Cellular Therapy and the Center for International Blood & Marrow Transplant Research aims to promote education and safe implementation within the broader and ever-growing cellular therapy field.
Faculty Bios – continued

**Katy Rezvani, MD, PhD**

Dr. Katy Rezvani was recruited to MD Anderson in 2012 as Professor of Medicine, Director of Translational Research and Medical Director of the GMP Facility. She was appointed Chief of the Section for Cellular Therapy in the Department of Stem Cell Transplantation in 2017. She is the Principal Investigator on numerous grants and trials. Dr. Rezvani is Specialty Chief Editor for the section of Cancer Immunity and Immunotherapy for Frontiers in Immunology and an Associate Editor for Cytotherapy. She has over 150 peer-reviewed publications related to Immunotherapy, Cellular Therapy and Hematology/Oncology and hematopoietic transplantation. She is on the Center for International Blood and Marrow Transplant (CIBMTR) clinical trials advisory committee, a member of the BMT CTN Cell and Gene Therapy and is on the organizing committees of the International Society of Cellular Therapy meeting and the Society for Natural Immunity NK 2018 meeting. Dr. Rezvani serves on as a reviewer for the National Cancer Institute, Leukemia Lymphoma Society, Cancer research UK, The Medical Research Council UK, the Dutch Cancer Society and the Kay Kendall Leukemia Fund. Dr. Rezvani has an active research laboratory program in transplantation immunology where the focus of her research group is to study the role of natural killer cells (NK) cells in mediating immunity against hematologic disorders such as acute leukemia and myelodysplastic syndromes as well as solid tumors, and to understand the mechanisms of tumor-induced NK cell dysfunction. Her laboratory program in transplant immunology has led to the approval and funding of a number of Phase I/II studies of immunotherapy in patients with hematologic malignancies and solid tumors, as well as the first-in-human clinical trial of off-the-shelf CAR-transduced cord blood NK cells in patients with relapsed/refractory lymphoid malignancies.

**Michel Sadelain, MD, PhD**

Michel Sadelain, MD, PhD, is the Director of the Center for Cell Engineering and the incumbent of the Stephen and Barbara Friedman Chair at Memorial Sloan-Kettering Cancer Center. He is a Member of the Immunology Program and the Departments of Medicine and Pediatrics. Dr. Sadelain's research focuses on human cell engineering and cell therapy to treat cancer and hereditary blood disorders. His laboratory has made several seminal contributions to the field of chimeric antigen receptors (CARs), from their conceptualization and optimization to their clinical translation for cancer immunotherapy. His group was the first to publish dramatic molecular remissions in patients with chemorefractory acute lymphoblastic leukemia following treatment with autologous CD19-targeted T cells.

Dr. Sadelain is the recipient of the Cancer Research Institute's Coley Award for Distinguished Research in Tumor Immunology, the Sultan Bin Khalifa International Award for Innovative Medical Research on Thalassemia, the NYPLA Inventor of the Year award, the Passano award and the Pasteur-Weizmann award. He previously served on the NIH Recombinant DNA Advisory Committee and as President of the American Society for Gene and Cell Therapy.

**Cameron Turtle, MBBS, PhD**

Dr. Turtle completed medical training at the University of Sydney, Australia, followed by Fellowships of the Royal Australasian College of Physicians and the Royal College of Pathologists of Australasia, and a PhD in Immunology. He is an Associate Member at Fred Hutchinson Cancer Research Center (FHCRC) and Associate Professor at the University of Washington (UW) in Seattle, WA. He serves as an attending physician on the Hematopoietic Cell Transplant (HCT) Service and the Immunotherapy Service at FHCRC, Seattle Cancer Care Alliance (SCCA) and the UW Medical Center, and has a research laboratory in the Clinical Research Division at FHCRC. His laboratory is focused on understanding the characteristics of distinct subsets of human CD8+ T cells, their potential utility for tumor immunotherapy, and their role in immune reconstitution after HCT. Dr. Turtle is Principal Investigator and IND sponsor of several investigator-initiated clinical trials of CD19-targeted chimeric antigen receptor (CAR)-modified T cell therapy for patients with B cell malignancies.
# Disclosure of Relevant Financial Relationships

<table>
<thead>
<tr>
<th>Name</th>
<th>Disclosure</th>
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<tbody>
<tr>
<td>Justin Eyquem, PhD</td>
<td>Licensing Fees: Takeda; Consulting Fee: Novartis; Research Support and Royalty: Cell Medica; Ownership Equity: Viracyte; Ownership Equity: Marker Therapeutics; Research Support: Tessa Therapeutics</td>
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<tr>
<td>Matthew Frigault, MD</td>
<td>Consulting Fees: Novartis, Xenetic Bio, Celegene, Foundation Medicine, Nkarta, Arcellx; Honoraria: Kite Pharma</td>
</tr>
<tr>
<td>Saar Gill, MD, PhD, FRACP</td>
<td>Research Funding: Novartis; Research Funding and IP Rights: Tmunity; Research Funding and Ownership Interest: Carisma</td>
</tr>
<tr>
<td>J. Keith Joung, MD, PhD</td>
<td>Ownership Interest: Epilogic Therapeutics, Poseida Therapeutics, Hera Biolabs, Transposagen Biopharmaceuticals; Consulting Fee and Ownership Interest: Editas Medicine, Beam Therapeutics, Endcadia; Sponsored Research Agreement for Academic Research Laboratory: Takeda</td>
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<td>James Kochenderfer, MD</td>
<td>Research Funding and Royalties on Patents: Kite, a Gilead company; Research Funding: Celgene</td>
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<td>Cyril Konto, MD</td>
<td>Employee and Shareholder: Allogene Therapeutics; Shareholder: Pfizer</td>
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<td>Wendell Lim, PhD</td>
<td>Stock and Consulting Fee: Cell Design Labs</td>
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<tr>
<td>Shannon Maude, MD, PhD</td>
<td>Honoraria and Consulting Fee: Novartis, and Kite, a Gilead company</td>
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<td>Marcela Maus, MD, PhD</td>
<td>Consultant and Honoraria: Adaptimmune, Adaptive Biotechnologies, bluebird bio, Cellectics (SAB), Incysus, Juno, MPM, Novartis, Takeda, TCR2 (SAB), Third Rock Ventures, Windmil (SAB); Consultant, Honoraria and Grant/Research Support: Agentus, CRISPR Therapeutics, TCR2, and Kite, a Gilead company</td>
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<tr>
<td>Michael Milone, MD, PhD</td>
<td>Royalty: Novartis; Ownership Interest: Cabaletta Bio</td>
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<tr>
<td>Sattva Neelapu, MD</td>
<td>Consulting Fee and Honoraria: Merck, Celgene, Novartis, Unum Therapeutics, Pfizer, Cell Medica, and Kite, a Gilead company; Research Support: Merck, BMS, Cellectis, Poseida, Karus, Acerta, and Kite, a Gilead company</td>
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<tr>
<td>Sarah Nikiforow, MD, PhD</td>
<td>Consulting Fee: Novartis; Nkarta, and Kite, a Gilead company</td>
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<tr>
<td>Michel Sadelain, MD, PhD</td>
<td>Contract Research: Juno Therapeutics, Fate Therapeutics, Atara Biotherapeutics; Consulting Fee: Berkeley Lights</td>
</tr>
<tr>
<td>Cameron Turtle, MBBS, PhD</td>
<td>Research Funding, Consulting Fee and IP Rights: Juno Therapeutics/Celgene; Research Funding and Consulting Fee: Nektar Therapeutics; Consulting Fee and Ownership Interest (Option Grant): Precision Biosciences, Caribou Biosciences; Ownership Interest (Option Grant): Eureka Therapeutics, Consulting Fee: Aptevo, Humanigen, Novartis, and Kite, a Gilead company</td>
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