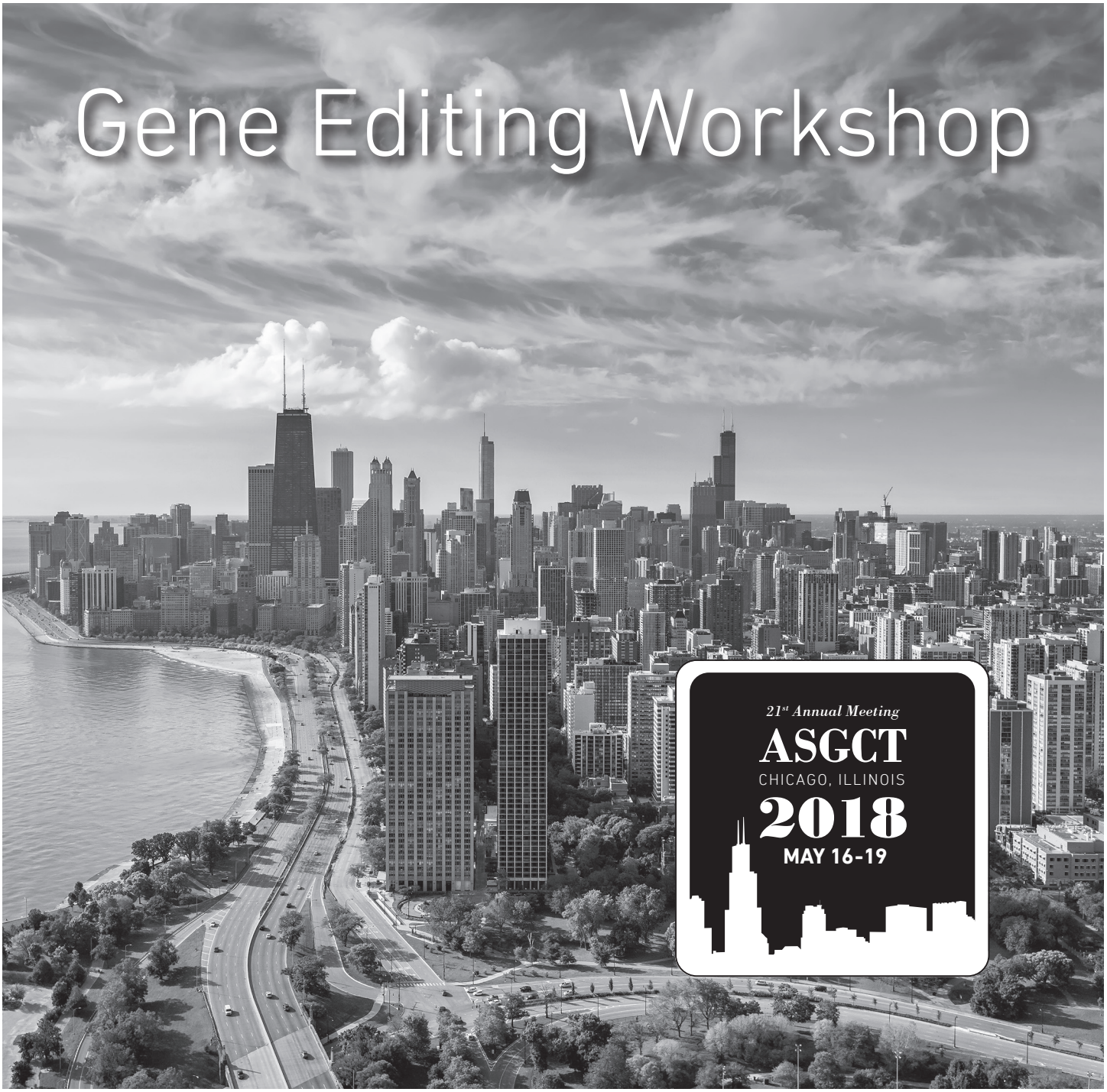
Gene Editing Workshop



AMERICAN SOCIETY of GENE & CELL THERAPY

May 15, 2018

Hilton Chicago
Continental C
Chicago

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The American Society of Gene & Cell Therapy is honored to acknowledge the following organizations for their support of the Gene Editing Workshop:

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Committee Listing

Co-Chairs

J. Keith Joung, MD, PhD
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Charlestown, MA

Matthew H. Porteus, MD, PhD
Stanford University School of Medicine
Stanford, CA

Members

Paula M. Cannon, PhD
University of Southern California
Los Angeles, CA

Toni Cathomen, PhD
Medical Center - University of Freiburg
Freiburg, Germany

Charles A. Gersbach, PhD
Duke University
Durham, NC

Faculty Listing

Omar Abudayyeh
Broad Institute of MIT and Harvard
Cambridge, MA

Charlie Albright, PhD
Editas Medicine
Cambridge, MA

Leonela Amoasii, PhD
UTSW Medical Center
Dallas, TX

Thomas Barnes, PhD
Intellia Therapeutics
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Mark A. Behlke, MD, PhD
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Tirtha Chakraborty, PhD
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Children's Hospital of Philadelphia
Philadelphia, PA

Suk See De Ravin, MD, PhD
National Institutes of Health, NIAID
Bethesda, MD

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Stanford University Medical Center
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Justin Eyquem, PhD
Memorial Sloan Kettering Cancer Center
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Nicole Gaudelli, PhD
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Charles A. Gersbach, PhD
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Ayal Hendel, PhD
Bar Ilan University
Ramat-Gan, Israel

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Sangamo BioSciences, Inc
Richmond, CA

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Massachusetts General Hospital
Charlestown, MA

Alexis C. Komor, PhD
UC San Diego
La Jolla, CA

Vikram Pattanayak, MD, PhD
Massachusetts General Hospital
Boston, MA

Krishanu Saha, PhD
University of Wisconsin-Madison
Madison, WI

Julianne Smith, PhD
Cellectis
New York, NY

James M. Wilson, MD, PhD
University of Pennsylvania
Philadelphia, PA

Faculty Bios

Omar Abudayyeh
Omar O. Abudayyeh is an MD–PhD. student in the Harvard–Massachusetts Institute of Technology (MIT) Health Sciences and Technology program. In the laboratory of Feng Zhang at the Broad Institute of MIT and Harvard, his doctoral research focuses on the discovery and characterization of novel CRISPR proteins, such as Cpf1, C2c1, and Cas13a/C2c2 in bacteria for the purpose of expanding the genome-editing toolbox and studying mammalian biology. His recent work has focused on using the RNA-targeting CRISPR system Cas13 for building a transcriptome engineering toolbox for applications in diagnostics and therapeutics. In 2012, Abudayyeh graduated with degrees in Mechanical Engineering and Biological Engineering from MIT.

Charlie Albright, PhD
Charlie Albright joined Editas Medicine as Chief Scientific Officer in August 2016. He brings more than 25 years of life sciences industry and academic leadership experience, most recently serving as vice president of genetically defined diseases and genomics at Bristol-Myers Squibb (BMS). Over his career, Albright has led discovery programs that advanced investigational medicines into clinical development in a wide range of therapeutic areas, including neurodegeneration, pain, psychiatry, oncology, and inflammation. Prior to his position as vice president of genetically defined diseases and genomics, he held multiple scientific leadership roles in neuroscience biology at BMS. Previously, he held positions at Incyte Corporation and DuPont Pharmaceuticals and was an assistant professor of biochemistry at Vanderbilt University. Charlie received a Bachelor of Science in Chemical Engineering and a PhD. in biology from the Massachusetts Institute of Technology (MIT). He was a postdoctoral fellow in the laboratory of Professor Robert Weinberg at the Whitehead Institute for Biomedical Research at MIT.

Leonela Amoasii, PhD
Leonela Amoasii works in the laboratory of Dr. Eric Olson at the University of Texas Southwestern Medical Center in Dallas. Amoasii pursued her graduate studies at the Institut de Genetique Biologie Moléculaire et Cellulaire (IGBMC) at the University of Strasbourg in France. During her doctoral studies, she uncovered the mechanistic basis of myotubular centronuclear myopathy and acquired expertise in the use of adeno-associated virus (AAV) for manipulation of gene expression in muscle. Over the last years, Amoasii has been involved in developing a new therapeutic approach for correction of Duchenne muscular dystrophy using CRISPR/Cas9 genomic editing. Amoasii pursued the in vivo optimization of the CRISPR/Cas9 genomic editing, her recent work reveals promising results for translation of the genome editing technology to human patients.

Thomas Barnes, PhD
Tom Barnes has led platform-based research and drug discovery teams for over 20 years, and is responsible for extending the reach of Intellia's CRISPR platform into new areas. A veteran entrepreneur who has helped launch several companies, he has turned creative scientific visions into successful businesses for both startups and established organizations.

Barnes has wide-ranging knowledge of biological systems through his work across diverse platforms, including genomics and gene discovery, small molecule drug repositioning, and protein engineering. Previously, as vice president of discovery at Eleven Biotherapeutics, Tom led his team in creating a novel chimeric cytokine antagonist, as well as two novel technologies. As senior vice president and site head of GeneLogic's drug repositioning division, he oversaw technology platforms in metabolomics, toxicogenomics, gene expression informatics, and the genetics of drug metabolism and transport. Barnes received his PhD. from the University of Cambridge, and completed research fellowships at Harvard Medical School and McGill University.

Mark A. Behlke, MD, PhD
As the chief scientific officer, Dr. Mark A. Behlke has directed research activities at IDT since joining the company in 1995 with a focus on novel molecular biology applications of oligonucleotide-based technologies. In addition, Behlke is also a scientific co-founder of Dicerna Pharmaceuticals, located in Boston. Before joining IDT, Behlke was a Physician Postdoctoral Fellow of the Howard Hughes Medical Institute at the Whitehead Institute, MIT. He was a resident physician in internal medicine at Brigham and Women's Hospital, Boston. Behlke received his MD and PhD degrees from Washington University, St. Louis in 1988 and his B.S. degree from the Massachusetts Institute of Technology in 1981. Behlke is an inventor on more than 45 issued US patents, has numerous pending patent applications, and is an author on over 125 scientific publications and book chapters. He is a recognized expert on oligonucleotide-based technologies.

Paula M. Cannon, PhD
Paula Cannon, PhD. is a distinguished professor in the Keck School of Medicine of the University of Southern California in Los Angeles. She studies genome engineering in hematopoietic stem cells, with an emphasis on developing therapies for HIV/AIDS. Cannon earned her PhD. from the University of Liverpool and did post-doctoral training at Harvard and Oxford Universities. In 2010, her team were the first to show that genome engineering could be used to knock-out the CCR5 gene in human hematopoietic stem cells. The discovery has now led to an ongoing clinical trial in HIV-infected individuals. She continues to develop new applications for genome engineering, with a goal of applying this technology to treat infectious and genetic diseases of the blood and immune systems.

Toni Cathomen, PhD
Toni Cathomen is professor of cell and gene therapy and director of the Institute for Transfusion Medicine and Gene Therapy at the Medical Center of the University of Freiburg, Germany. After receiving his PhD. from the University of Zurich, Switzerland, he was a postdoctoral fellow at the Salk Institute in San Diego, assistant professor of Molecular Virology at Charité Medical School in Berlin, and associate professor of experimental hematology at

Faculty Bios – continued

Hannover Medical School. His research activities focus on the development of immune cell therapies based on induced pluripotent stem cells (iPSCs); the improvement of the effectiveness and safety of designer nucleases (CRISPR-Cas and TALEN) for targeted genome editing; and the development and manufacturing of genome edited stem cell and immune cell preparations for the treatment of HIV infection, primary immunodeficiencies and cancer.

Tirtha Chakraborty, PhD
Tirtha Chakraborty is currently the Head of Hematology, Executive Director, at CRISPR Therapeutics. His team is involved in research on hemoglobin disorders, including beta-thalassemia, that led to the first Clinical trial application filed by CRISPR therapeutics, and any CRISPR company in 2017. Prior to joining CRISPR Therapeutics, he was involved in platform technology research at Moderna Therapeutics and led the mRNA Sciences Department. In his academic life, he was trained as a molecular geneticist and focused on role of small RNA in B lymphocyte biology and lymphoma generation , with Prof Klaus Rajewsky at Harvard. His PhD work involved studying naturally acquired resistance against the malarial parasite. Dr. Chakraborty has authored more than 20 papers, and is a contributor on more than 40 patent applications.

Beverly L. Davidson, PhD
Beverly L. Davidson, is the director of the Raymond G. Perelman Center for Cellular and Molecular Therapeutics, the chief scientific strategy officer, and holds the Arthur V. Meigs Chair in Pediatrics at the Children's Hospital of Philadelphia. She is also professor of pathology and laboratory medicine at the Perelman School of Medicine, University of Pennsylvania.

Davidson received her PhD. in biological chemistry from the University of Michigan in 1987, and in 1994 was recruited to the University of Iowa where she was promoted to associate professor in 1999 and professor in 2001. From 1999-2014 she held the Roy J. Carver Chair in Biomedical Research, and was named vice chair for research, internal medicine from 2004-2014. She was named an AAAS fellow in 2007, and in 2009, received the NIH Mathilde Solowey award, and was named member, electorate nominating committee, as well as chair, medical sciences, AAAS. In 2011, Dr. Davidson was the S.J. DeArmond Lecturer, American Association of Neuropathologists, and University of Iowa Presidential Lecturer. In 2012, received the Carver College of Medicine Faculty Service award, and the Iowa Innovator award. She was awarded the Leslie Gehry Brenner Prize for Innovation in Science in 2015. In April 2017 she became a member of the American Academy of Arts and Sciences.

Davidson's research is focused on inherited brain disorders and the development of novel therapies to treat these fatal diseases. She has served on numerous NIH study sections, was co-chair of the Editors Panel, Transformative Award Review Committee from the Office of the Director (NIH) and currently serves on Council for NINDS, NIH. She is a member of the scientific advisory board of the Huntington Study Group and the medical research advisory board of the National Ataxia Foundation, Davidson is a co-founder of Spark Therapeutics, Inc., and serves on the advisory boards of Sarepta Therapeutics and Intellia Therapeutics.

Suk See De Ravin, MD, PhD
Suk See De Ravin completed her pediatric specialty training, followed by research focusing on the molecular diagnosis and gene therapy on X-linked severe combined immunodeficiency at Sydney Children's Hospital, Australia. She completed her post-doctoral fellowship at the Laboratory of Host Defenses, NIAID, National Institutes of Health on gene therapy development for primary immunodeficiencies (PID), including a preclinical canine model of SCID-X1. Upon transitioning to a staff clinician, she has worked on clinical trials relating to PID, while in the laboratory worked on ways to advance novel approaches to gene therapy, including targeted gene editing of hematopoietic stem and progenitor cells, or correction of primary immune cells for treatment of primary immunodeficiency diseases.

Daniel Dever, PhD
Daniel Dever is a research instructor in the laboratory of Dr. Matthew Porteus at Stanford University, in the Department of Pediatrics, Division of Stem Cell Transplantation and Regenerative Medicine. He completed his PhD in molecular toxicology at the University of Rochester where he studied the mechanisms of the aryl hydrocarbon receptor in mediating cerebellar transcriptional programs. During his postdoctoral work in the Porteus group, he (with others) developed a CRISPR/Cas9-based beta-globin (HBB) gene editing by homologous recombination methodology (gene targeting) in CD34+ hematopoietic stem cells as a potential therapeutic strategy to treat severe sickle cell disease. Dever (along with collaborators) has now successfully used this methodology to efficiently target >15 genes in primary blood cells that are associated with hematopoiesis, hematopoietic genetic diseases, hematopoietic malignancies, or safe harbor sites. Dever's primary research interests are to continue to leverage CRISPR/Cas9-based genome editing technologies to study the molecular mechanisms of gene targeting in human hematopoietic stem cells with the ultimate goal of optimizing and further developing novel cell and gene therapies for disease of the blood and the immune system. Currently, he is leading IND-enabling preclinical efficacy, feasibility, safety and tumorigenicity studies for FDA approval of a first-in-human clinical trial at Stanford in 2018 for the treatment of severe sickle cell disease using CRISPR/Cas9-based HBB gene targeting in autologous hematopoietic stem cells.

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 in the development of genome 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 the lab of Michel Sadelain, a pioneer in the Chimeric Antigen Receptor (CAR) T cells field, at the Memorial Sloan Kettering Cancer Center. Eyquem is now using

Faculty Bios – continued

CRISPR/Cas9 to engineer CAR T cells and my recent work showed how precise genome editing can augment CAR T cell efficacy, advance CAR immuno-biology, and facilitate T cell manufacturing.

Nicole Gaudelli, PhD
Nicole Gaudelli received her B.S. in biochemistry from Boston College in May of 2006. While at Boston College she conducted research under the guidance of Steve Bruner to elucidate the enzymatic mechanisms of an aminotransferase involved in neocrazinostatin biosynthesis and a non-heme iron oxygenase involved in vancomycin assembly. She then joined the laboratory of Craig Townsend at Johns Hopkins University where she studied a non-ribosmal peptide synthetase (NRPS) implicated in the biosynthesis of the β-lactam antibiotic nocardicin. In her doctoral work she elucidated the mechanism through which monobactam anitbiotics are biosynthesized. She next pursued postdoctoral work at Harvard University in the laboratory of David R. Liu where she expanded the capabilities of base-editing technology by creating an adenine base editor (ABE), through seven rounds of evolution and engineering, which cleanly converts A•T base pairs to G•C base pairs in a programmable manner, with low indel percent, and without double-stranded DNA breaks. She recently joined Beam Therapeutics in order to further expand and apply base editing technology to human genetic diseases.

Charlie Gersbach, PhD
Charles A. Gersbach is the Rooney family associate professor at Duke University in the Departments of Biomedical Engineering and Orthopaedic Surgery, an investigator in the Duke Center for Genomic and Computational Biology, and director of the Duke Center for Biomolecular and Tissue Engineering. His research interests are in genome and epigenome editing, gene therapy, regenerative medicine, biomolecular and cellular engineering, synthetic biology, and genomics. Dr. Gersbach's work has been recognized through awards including the NIH Director's New Innovator award, the NSF CAREER award, the Outstanding New Investigator award from the American Society of Gene and Cell Therapy, the Allen Distinguished Investigator award, and induction as a Fellow of the American Institute for Medical and Biological Engineering.

Ayal Hendel, PhD
Ayal Hendel is a principal investigator and a senior lecturer in the Mina and Everard Goodman Faculty of Life Sciencesin at Bar-Ilan University. Hendel's research focuses on developing genome editing and CRISPR technology as a method of gene therapy for genetic diseases of the blood and the immune system such as severe combined immunodeficiency. In addition Hendel's lab develops novel genome editing approaches for cancer immunotherapy. Hendel received his B.A. with high honor in biology from the Hebrew University of Jerusalem and his M.Sc. and PhD. from the Weizmann Institute of Science. Hendel carried out post-doctoral research at Stanford University School of Medicine with Dr. Matthew Porteus, where he demonstrated that chemical alterations to synthesized CRISPR-single guide RNAs enhance genome editing efficiency in human primary T cells and hematopoietic stem and progenitor cells (Nat. Biotechnol. 2015. doi: 10.1038/nbt.3290). In addition, in a recent study he was part of a team that achieved therapeutically relevant genome editing frequencies in CD34+ human hematopoietic stem and progenitor cells (Nature. 2016. doi: 10.1038/nature20134).

Michael C. Holmes, PhD
Michael Holmes, PhD., serves as SVP and chief technology officer and currently oversees Sangamo Therapeutic's research activities. He joined Sangamo in May 2001 as a scientist and became a team leader in 2002, focusing on the development of novel cell-based approaches to screen compound libraries. He was promoted to director, therapeutic gene modification in 2004, senior director in 2009, and vice president of research in 2015, where he pioneered the use of ZFNs for genome editing in transformed and primary human cells, including hematopoietic stem cells. His group was also responsible for the testing, optimization, and pre-clinical validation of the ZFNs for the SB-728 HIV/AIDS programs using autologous T-cells and hematopoietic stem/progenitor cells (HSPC), both of which are in early stage clinical trials.

More recently, Holmes' group spearheaded the development of a genome editing approach to modify autologous HSPC ex vivo to reactivate fetal globin in erythrocytes for the potential treatment of patients with transfusion-dependent beta-thalassemia. This program (ST-400) was done in partnership with Bioverativ, Inc. and is currently being evaluated in a Phase I/II clinical study. His group has also been responsible for the pre-clinical studies testing the use of ZFNs to genome edit liver hepatocytes in vivo to drive expression of factors that are normally defective in patients with hemophilia and lysosomal storage diseases. The use of this approach as an In Vivo Protein Replacement Platform (IVPRP) is currently being evaluated in early stage clinical trials in patients with Hemophilia B, MPS I, and MPS II. Holmes has authored over 50 publications in the field of genome editing and gene regulation, as well as numerous patents. Prior to joining Sangamo, he worked as a post-doctoral fellow in Dr. Gerald Rubin's lab at the University of California-Berkeley. Holmes received a B.S. in molecular biology from the University of California-San Diego, and his PhD. in molecular and cell biology from the University of California-Berkeley.

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, pathologist, and associate chief of pathology for research at Massachusetts General Hospital (MGH) and is professor of pathology at Harvard Medical School. Joung has been a pioneer in the development of important technologies for targeted genome editing and epigenome 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

Faculty Bios – continued

Cited Researcher" in 2016 and 2017 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, Monitor Biotechnologies, Pairwise Plants, and Beam Therapeutics. 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.

Alexis Komor
Alexis received her B.S. degree in chemistry from the University of California-Berkeley in December of 2008. She then joined the lab of Jacqueline K. Barton at the California Institute of Technology for her doctoral studies. While at Caltech, she worked as an NSF Graduate Research Fellow on the design, synthesis, and study of DNA mismatch-binding metal complexes and received her PhD. in 2014. She pursued postdoctoral work as a Ruth L. Kirschstein NIH Postdoctoral Fellow in the laboratory of David R. Liu where she developed base editing, a new approach to genome editing that enables the direct, irreversible chemical conversion of one target DNA base into another in a programmable manner without requiring double-stranded DNA backbone cleavage. Komor joined the department of chemistry and biochemistry at the University of California-San Diego in 2017.

Vikram Pattanayak, MD, PhD
Vikram Pattanayak is an assistant in pathology at Massachusetts General Hospital and an instructor in pathology at Harvard Medical School, leading a subgroup of Keith Joung's laboratory focused on the development of genome editing tools. During his MD, PhD. thesis work with David Liu in the Harvard chemistry department, he developed assays to define the specificities of designer endonucleases, including homing endonucleases, zinc finger nucleases (ZFNs), transcription activator-like effector nucleases (TALENs), and Cas9. As a member of the Joung Lab, he engineered a variant of Cas9 (SpCas9-HF1) with minimal off-target effects. In addition to his research activities, Pattanayak has also completed a residency in clinical pathology and fellowship in molecular genetic pathology and serves as the assistant director of the MGH histocompatibility (HLA) laboratory.

Matt Porteus, MD, PhD
Matt Porteus was raised in California and was a local graduate of Gunn High School before completing A.B. degree in history and science at Harvard University where he graduated Magna Cum Laude and wrote a thesis entitled "Safe or Dangerous Chimeras: The recombinant DNA controversy as a conflict between differing socially constructed interpretations of recombinant DNA technology."

He then returned to the area and completed his combined MD, PhD at Stanford Medical School with his PhD focused on understanding the molecular basis of mammalian forebrain development with his PhD thesis entitled "Isolation and Characterization of TES-1/DLX-2: A Novel Homeobox Gene Expressed During Mammalian Forebrain Development." After completion of his dual degree program, he was an intern and resident in pediatrics at Boston Children's Hospital and then completed his pediatric hematology/oncology fellowship in the combined Boston Children's Hospital/Dana Farber Cancer Institute program.

For his fellowship and post-doctoral research he worked with Dr. David Baltimore at MIT and Caltech where he began his studies in developing homologous recombination as a strategy to correct disease causing mutations in stem cells as definitive and curative therapy for children with genetic diseases of the blood, particularly sickle cell disease.

Following his training with Baltimore, he took an independent faculty position at UT Southwestern in the departments of pediatrics and biochemistry before again returning to Stanford in 2010 as an associate professor. During this time his work has been the first to demonstrate that gene correction could be achieved in human cells at frequencies that were high enough to potentially cure patients and is considered one of the pioneers and founders of the field of genome editing—a field that now encompasses thousands of laB.S. and several new companies throughout the world.

Krishanu Saha, PhD
Krishanu Saha is an assistant professor in the department of biomedical engineering at the University of Wisconsin-Madison. He is also a member of the Wisconsin Institute for Discovery, Carbone Cancer Center, and Stem Cell and Regenerative Medicine Center as well as the National Academies' Forum on Regenerative Medicine. Prior to his arrival in Madison, Saha studied chemical engineering at Cornell University and at the University of California in Berkeley. He was a Society in Science: Branco-Weiss fellow at the Whitehead Institute for Biomedical Research at MIT and in the Science and Technology Studies program at Harvard University. Major thrusts of his lab involve gene editing and cell engineering of human cells found in the retina, central nervous system and blood.

Julianne Smith, PhD
Julianne Smith joined Cellectis in 2002 and has been working in the therapeutic division since its inception. As the vice president of CAR T development, she was involved in the development of Cellectis' Universal CART (UCART) products. She is currently vice president of translational sciences and is leading nonclinical development as well as overseeing the correlative studies in the current clinical trials. Smith has a PhD. in genetics and development from Columbia University and a B.A. in biology from Johns Hopkins University.

James M. Wilson, MD, PhD
James M. Wilson is a professor in the Perelman School of Medicine at the University of Pennsylvania where he has led an effort to develop the field of gene therapy. Wilson began his work in gene therapy during his graduate studies at the University of Michigan

over 30 years ago. He created the first and largest academic-based program in gene therapy after being recruited to Penn in 1993. He initially focused on the clinical translation of existing gene transfer technologies but soon redirected his efforts to the development of second and third generation gene transfer platforms; the first of which was licensed to a biotechnology company he founded that resulted in the first, and only, commercially approved gene therapy in the western hemisphere. He is currently leading a national dialogue on the challenges of commercializing these potentially lifesaving treatments due to the disruptive nature they will have on traditional business models. Dr. Wilson was noted by the journal Nature Biotechnology to be the "second most productive bio-entrepreneur in life sciences."

Disclosure of Relevant Financial Relationships

Name	Disclosure
Charlie Albright, PhD	Salary and Stock, Editas Medicine
Leonela Amoasii, PhD	Salary, Exonics Therapeutics
Mark Behlke, MD, PhD	Salary, Integrated DNA Technologies, Inc.
Paula M. Cannon, PhD	Honorarium, Sangamo Therapeutics
Toni Cathomen, PhD	Consulting Fee, TRACR Hematology; Research Support, Cellectis S.A.; Research Support, Miltenyi Biotec
Tirtha Chakraborty, PhD	Salary and Stock, CRISPR Therapeutics
Beverly L. Davidson, PhD	Equity and Consulting Fee, Spark Therapeutics; Consulting Fee, Inc. Intellia, Inc.; Friends of Telethon Italia; Consulting Fee, Homology Medicines
Nicole Gaudelli, PhD	Salary, Beam Therapeutics
Charlie Gersbach, PhD	Royalty, Consulting and Ownership, Element Genomics; Consulting and Ownership, Locus Biosciences; Consulting, Sarepta Therapeutics; Royalty, Editas Medicine
Michael C. Holmes, PhD	Salary, Sangamo Therapeutics
Alexis Komor, PhD	Consulting Fee and Common Stock, Beam Therapeutics; Consulting Fee and Class C Unit Grant, Pairwise Plants
Thomas M. Barnes, PhD	Salary and Stock, Intellia Therapeutics
J. Keith Joung, MD, PhD	Consulting Fee and Ownership Interest, Editas Medicine; Consulting Fee and Ownership Interest, Beam Therapeutics; Ownership Interest, Monitor Biotechnologies; Consulting Fee, Horizon Discovery Sponsored Research Agreement, Takeda Pharmaceuticals; Sponsored Research Agreement, AstraZeneca
Vikram Pattanayak, MD, PhD	Royalty from Licensed IP, Editas Medicine via Harvard University and Massachusetts General Hospital
Krishanu Saha, PhD	Honoraria, California Institute for Regenerative Medicine
Julianne Smith, PhD	Salary, Cellectis
James M. Wilson, MD, PhD	Royalty and Stock, REGENX Bio; Stock, Solid Bio

Program

8:30 AM – 9:00 AM

Introduction

SPEAKER: J. Keith Joung, MD, PhD

9:00 AM – 10:30 AM

Pre-Clinical Development

CHAIR: Matt Porteus, MD, PhD

SPEAKERS: ames Wilson, MD, PhD
Krishanu Saha, PhD
Paula Cannon, PhD
Daniel Dever, PhD

10:30 AM – 11AM

Coffee Break

11 AM – 12 PM

Clinical Gene Editing Programs

CHAIR: Toni Cathomen, PhD

SPEAKERS: Justin Eyquem, PhD
Beverly L. Davidson, PhD
Leonela Amoasii, PhD
Toni Cathomen, PhD

12 PM – 1 PM

Lunch

1 PM – 2:30 PM

Non-Cononical Gene Editing

CHAIR: Charlie Gersbach, PhD

SPEAKERS: Charlie Gersbach, PhD
Omar Abudayyeh
Nicole Gaudelli, PhD

2:30 PM – 3 PM

Junior Investigators

CHAIR: Paula Cannon, PhD

SPEAKERS: Vikram Pattanayak, MD, PhD
Ayal Hendel, PhD

3 PM – 3:30 PM

Coffee Break

3:30 PM – 4 PM

Junior Investigators

SPEAKERS: Suk See De Ravin, MD, PhD
Alexis Komor, PhD

4 PM – 5 PM

Corporate Review

CHAIR: J. Keith Joung, MD, PhD

SPEAKERS: Mike Holmes, PhD
Mark Behlke, MD, PhD
Tirtha Chakraborty, PhD
Tom Barnes, PhD
Charlie Albright, PhD
Julianne Smith, PhD

Notes

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Notes

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