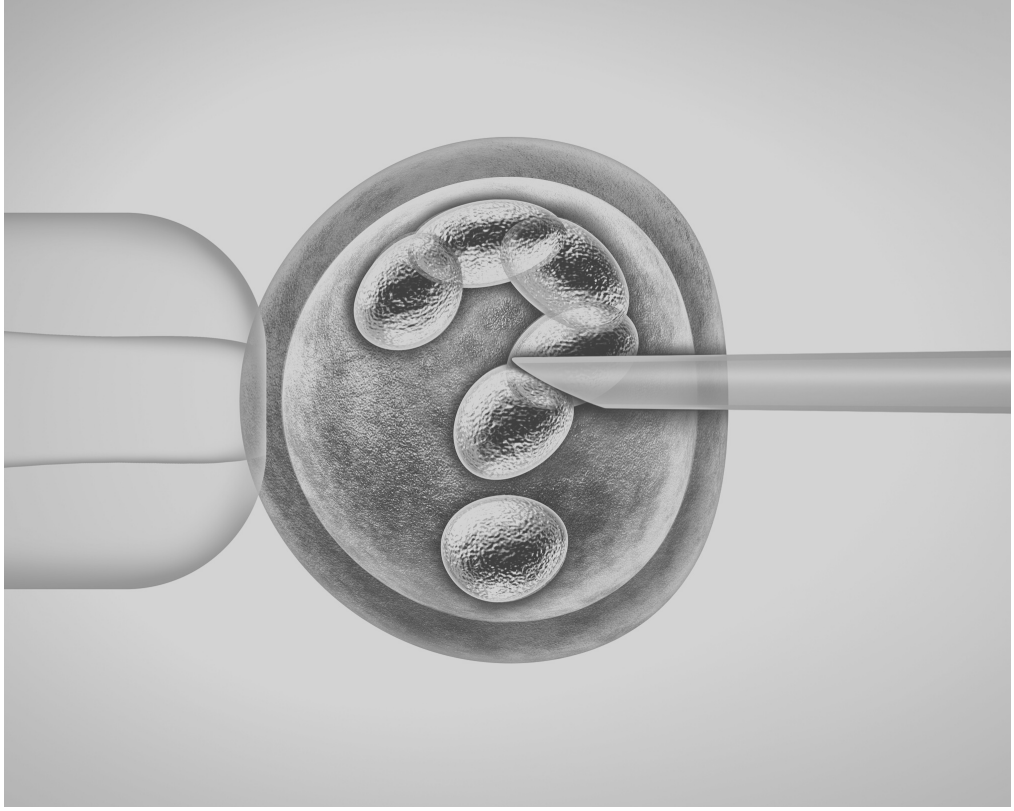

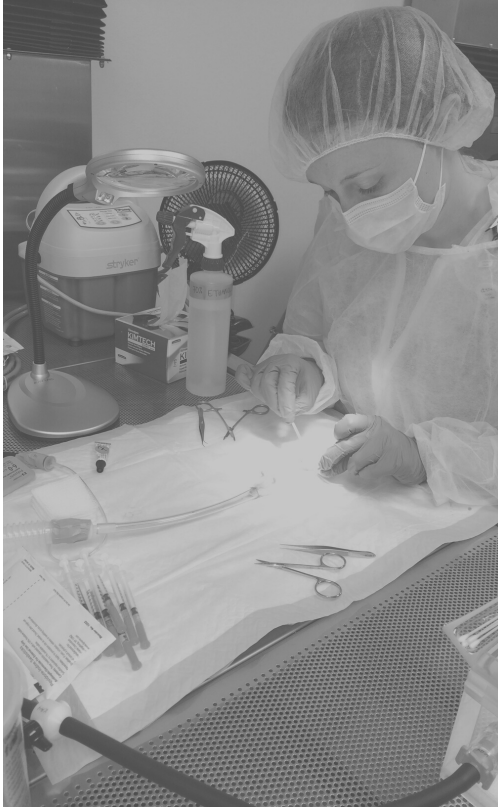

Moving Genome Editing to the Clinic: From Technology to Therapeutics

Pre-Meeting Workshop

Monday, May 10

10 a.m. - 2 p.m. ET

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The American Society of Gene & Cell Therapy is honored to acknowledge the following organizations for their support of Moving Genome Editing to the Clinic: From Technology to Therapeutics:

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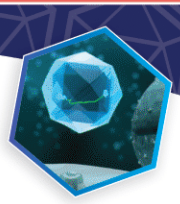

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Homology Medicines' mission is focused on curing disease & transforming patients' lives.

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pheNIX Our investigational gene therapy for adults with the rare disease phenylketonuria (PKU) is recruiting for the Phase 2 dose expansion phase of the trial, with initial data anticipated by the end of the year.

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We plan to initiate two additional Phase 1/2 dose-escalation clinical trials in 2021 with our investigational gene therapy for MPS II (Hunter syndrome) and our first gene editing candidate, which is for PKU. We are also optimizing our gene therapy candidate for metachromatic leukodsytrophy (MLD).

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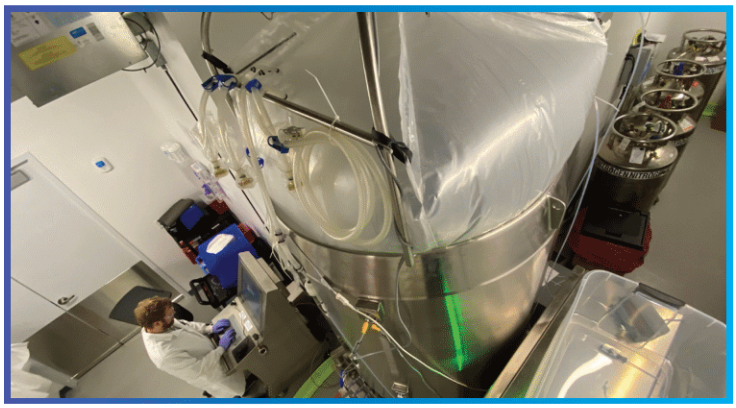

Learn more about our commitment to the rare disease community at **homologymedicines.com.**

Homology's ASGCT 2021 Presentations:

Long-Term Expression of HMI-203: Investigational Gene Therapy Candidate for Mucopolysaccharidosis Type II (MPS II), or Hunter Syndrome
Date/Time: Tuesday, May 11; 8:00-10:00 a.m.
Abstract #: 507

Functional Characterization of AAVHSCs Compared to AAV Serotypes: Activation of Cellular Pathways *In Vitro* and *In Vivo* Transduction Properties
Date/Time: Tuesday, May 11; 8:00-10:00 a.m.
Abstract #: 304

Wildtype AAV2 Rep Protein Produces Higher Titer AAVHSC Vectors With Improved Packaging Profiles Compared to Clade F Associated Chimeric Rep
Date/Time: Tuesday, May 11; 8:00-10:00 a.m.
Abstract #: 804

Oral Presentation

Transducing the Liver as an Antibody Factory Using AAVHSCs
Date/Time: Tuesday, May 11; 8:00-10:00 a.m.
Abstract #: 336

Investigational Genetic Medicine Approaches for Phenylketonuria (PKU)
Date/Time: Tuesday, May 11; 8:00-10:00 a.m.
Abstract #: 405

Next Generation AAV Drug Products: Enhanced Stability & Clinical Ease for High Titer Preparations
Date/Time: Tuesday, May 11; 6:45–7:00 p.m.
Abstract #: 27

Gene Therapy Candidate for Metachromatic Leukodystrophy (MLD): Summary of Preclinical *In Vivo* Data Following an Intravenous Delivery of HMI-202
Date/Time: Thursday, May 13; 5:30–5:45 p.m.
Abstract #: 159

Moving Genome Editing to the Clinic: From Technology to Therapeutics

Co-Chairs: Shengdar Tsai, Ph.D., and Laura Sepp-Lorenzino, Ph.D.

10 - 10:25 a.m.
Towards Safe and Effective Genome Editing Therapies in Human HSCs and T-cells
Shengdar Tsai, Ph.D., St. Jude Children's Research Hospital

10:25 - 10:50 a.m.
Expanding Genome Editing with Engineered CRISPR-Cas Enzymes
Benjamin Kleinstiver, Ph.D., Massachusetts General Hospital

10:50 - 11:15 a.m.
CRISPR T-cell Screens for Immunotherapy
Alex Marson, M.D., Ph.D., Gladstone-UCSF Institute of Genomic Immunology

11:15 - 11:40 a.m.
The Development of CRISPR Based Medicines for the Treatment of Ocular Diseases
Heather MacLeod, Ph.D., Editas Medicine

11:40 a.m. - 12:05 p.m.
Developing RNP-based Biologics for In Vivo Gene Editing
Mary Janatpour, Ph.D., Spotlight Therapeutics

Moving Genome Editing to the Clinic: From Technology to Therapeutics

12:15 - 12:40 p.m.

Adenine Base Editing Strategy for the Treatment of Sickle Cell Disease by Elimination of the Pathogenic Globin Protein

Nicole Gaudelli, Ph.D., Beam Therapeutics

12:40 - 1:05 p.m.

CBER's Regulatory Approach for Gene Therapies Incorporating Human Genome Editing

Anna Kwilas, Ph.D., FDA

1:05 - 1:30 p.m.

Characterization of Potential Unintended Genome Editing with CRISPR/Cas9 for New Therapeutics

Daniel O'Connell, Ph.D., Intellia Therapeutics

Moving Genome Editing to the Clinic: From Technology to Therapeutics

1:30 - 2 p.m.

Panel Discussion

- Fyodor Urnov, Ph.D., University of California-Berkeley
- Daniel O'Connell, Ph.D., Intellia Therapeutics
- Anna Kwilas, Ph.D., FDA
- Nicole Gaudelli, Ph.D., Beam Therapeutics
- Heather MacLeod, Ph.D., Editas Medicine

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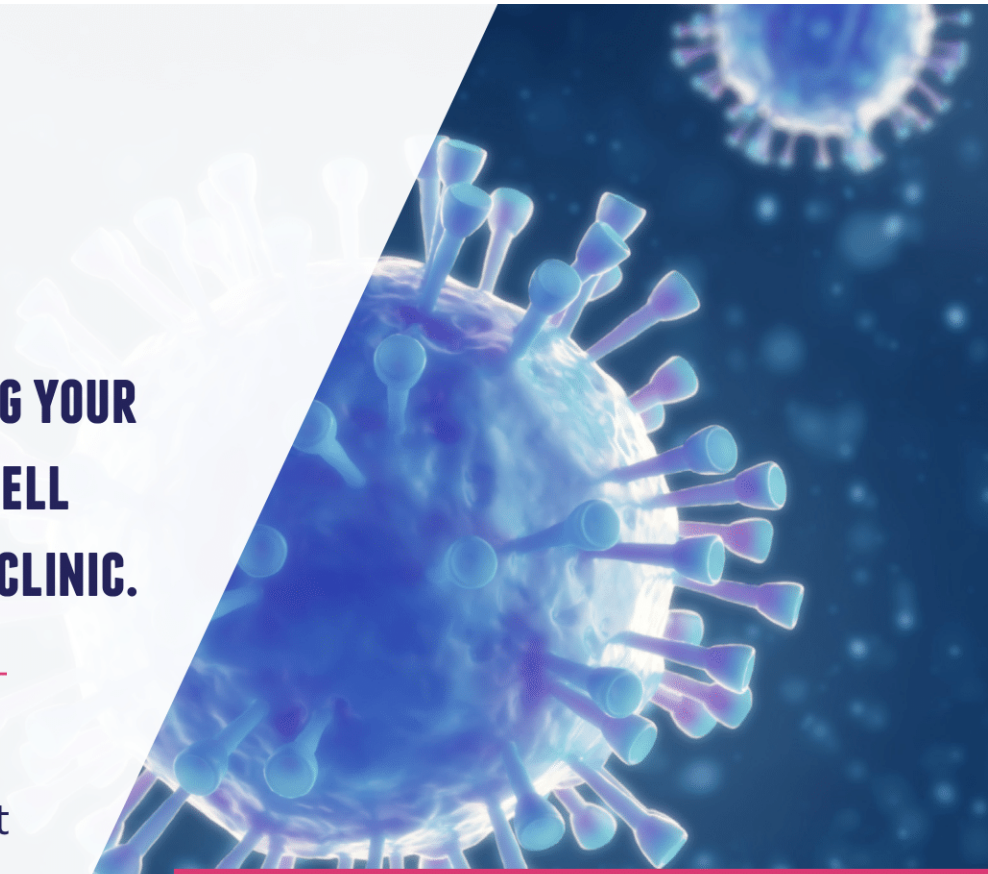

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Nicole Gaudelli, Ph.D.
Beam Therapeutics

Nicole Gaudelli received her B.S. degree in biochemistry from Boston College in 2006 where she studied the structural and mechanistic underpinnings of neocarzinostatin biosynthesis and a non-heme iron oxygenase involved in vancomycin assembly. She earned her Ph. D. in chemistry from Johns Hopkins University where she studied monocyclic beta-lactam antibiotics and elucidated the mechanism through which they are biosynthesized. She completed her postdoctoral fellowship at Harvard University in the laboratory of Professor David R. Liu where she expanded the capabilities of base editing technology by inventing and creating the first adenine base editor (ABE) through 7 rounds of directed evolution and engineering. Both her doctoral and postdoctoral work culminated in prominent Nature publications in the fields of natural product chemistry and gene editing respectively. Nicole is an inventor on numerous base editing patents and is the Director and Head of the Gene Editing Technologies platform group at Beam Therapeutics where her team advances and engineers precision genetic medicines using base editors as well as other gene editing tools. Nicole is a recipient of the 2018 American Chemical Society's “Talented 12” award, recognized as a 2018 STAT News Wunderkind, a 2018 TEDMED Hive honoree, Genetic Engineering and Biotechnology News “Top 10 Under 40 of 2019”, BioSpace’s 2019 “10 Life Science Innovators Under 40 to Watch”, MIT’s 2019 Technology Reviews’ 35 Innovators Under 35, Business Insider’s 30 Under 40 in 2020, and Fortune Magazine’s 40 Under 40 in Healthcare.

Mary Janatpour, Ph.D.
Spotlight Therapeutics

Dr. Janatpour serves as Senior Vice President of Biology at Spotlight Therapeutics. She has over 20 years of pharma and biotech discovery and research experience with core expertise in cancer biology and building first-in-class preclinical pipelines. Just prior to joining Spotlight, Dr. Janatpour served as Vice President Oncology Research at Dynavax Technologies where she led the clinical DV281 lung cancer program through Phase 1b and headed a laboratory aimed at identifying rational combinations with TLR9 agonists and expanding strategies for targeting TLR agonists to tumors. Before Dynavax, she was an independent consultant for immuno-oncology start-ups with novel platforms. Dr. Janatpour began her focus on cancer research in industry at Chiron Corporation, Schering-Plough Biopharma then Novartis, where she held positions of increasing responsibility, leading programs and building oncology early stage portfolios, notably in biologics. Dr. Janatpour received her BA in Molecular Biology from the University of California, Berkeley and a Ph.D. in Biomedical Science from the University of California, San Francisco. She did her post-doctoral training in Immunology at the DNAX Research Institute in Palo Alto, CA.

Benjamin Kleinstiver, Ph.D.
Massachusetts General Hospital

Ben Kleinstiver is a biochemist and genome editor whose interests include genome editing technology development, protein engineering, and translating technologies into molecular medicines. He received his Ph.D in Biochemistry from the University of Western Ontario, and completed his postdoctoral studies at Massachusetts General Hospital and Harvard Medical School. Within the Center for Genomic Medicine at MGH, the Kleinstiver laboratory develops scalable methods for molecular engineering to accelerate the development of CRISPR technologies. The longer term goal of his research program is to address limitations of these technologies that will help solve important research questions at the forefront of the genome editing field, while contributing to the development of new treatments for various genetic diseases.

Anna Kwilas, Ph.D.
FDA

Dr. Kwilas received her Ph.D. in Biomedical Science from The Ohio State University in 2010 with an emphasis in Molecular Virology & Gene Therapy and Translational Science. She performed her graduate research at The Research Institute at Nationwide Children's Hospital examining the potential application of respiratory syncytial virus as a gene therapy vector for the treatment of cystic fibrosis. Dr. Kwilas performed her post-doctoral research at the National Cancer Institute investigating the efficacy of modified vaccinia virus Ankara and adenovirus-based cancer vaccines alone and in combination with other approved and investigational cancer therapeutics. Dr. Kwilas joined FDA in 2015 on an Interagency Oncology Task Force Fellowship. During her fellowship she was involved in the CMC review of gene therapy products and the generation of safer vector producing cells with the use of CRISPR/Cas9 genome editing technology. In 2016, Dr. Kwilas became a full-time gene therapy CMC reviewer in the Gene Therapy Branch of CBER OTAT and in 2019 was promoted to a Team Lead in the Gene Therapy Branch.

Heather MacLeod, Ph.D.
Editas Medicine

Dr. Heather MacLeod is a lab head and project team leader at Editas Medicine. As a member of the Discovery Biology team, Dr. MacLeod focuses on developing in vivo CRISPR editing medicines for the treatment of ocular diseases. Prior to joining Editas, Dr. MacLeod was part of the Ophthalmology Department at Novartis contributing to the drug discovery efforts for both small molecule and biologics.

Alex Marson, M.D., Ph.D.
Gladstone-UCSF Institute of Genomic Immunology

Alex Marson is an Associate Professor at UCSF and the Director of the Gladstone-UCSF Institute of Genomic Immunology. As a physician-scientist with a background in immune genomics, Alex's lab is focused on adapting CRISPR genome editing techniques to human immune cells in order to understand the genetic programs controlling immune cell function and to manipulate T cells to generate cell-based therapies for a wide range of diseases.

Daniel O'Connell, Ph.D.
Intellia Therapeutics

Dr. O'Connell is a Director at Intellia Therapeutics where he specializes in genomics and the characterization of potential CRISPR off-target editing. Before joining Intellia Therapeutics he was a post-doctoral associate at the Broad Institute focused on genomic approaches to the functional genetics of inflammatory bowel disease. Dan earned his BS in Biological Sciences from Cornell University and Ph.D. in Genetics from Harvard Medical School in 2011.

Laura Sepp-Lorenzino, Ph.D.
Intellia Therapeutics

Laura Sepp-Lorenzino, Ph.D., is executive vice president, chief scientific officer of Intellia Therapeutics, Inc. Prior to joining Intellia in May 2019, she was vice president, head of nucleic acid therapies, member of the research leadership and external innovation at Vertex Pharmaceuticals, Inc. from September 2017 to May 2019. From April 2014 to September 2017, Dr. Sepp-Lorenzino was vice president, entrepreneur-in-residence and head of the hepatic infectious disease strategic therapeutic area at Alnylam Pharmaceuticals, Inc., and was at Merck from 1999 to 2014, most recently as Executive Director in RNA Therapeutics. Laura is a member of the scientific advisory boards of Thermo Fisher Scientific, Lodo Therapeutics, and the U.K. Medical Research Council Nucleic Acid Therapy Accelerator. In December 2020 she joined the board of directors of Taysha Gene Therapies, a biopharmaceutical company focused on developing treatments for monogenic diseases in the central nervous system. Dr. Sepp-Lorenzino is also a member of the board of directors of the Oligonucleotide Therapeutics Society. Dr. Sepp-Lorenzino earned a professional degree in biochemistry from the Universidad de Buenos Aires in Argentina and a Ph.D. in biochemistry from New York University.

Shengdar Tsai, Ph.D.
St. Jude Children's Research Hospital

Dr. Tsai is an Assistant Member in the Department of Hematology at St. Jude Children's Research Hospital. His lab's research focuses on genome editing technologies for therapeutics, with a special interest in editing human HSCs for treatment of hemoglobinopathies such as sickle cell disease and T-cells for cancer immunotherapy. His group has recently developed CHANGE-seq, a state-of-the-art, sensitive, unbiased, high-throughput method for defining the genome-wide activity of genome editors. Previously, he has developed methods for high-throughput genome editing with TALENs, and for defining and improving the genome-wide specificity of CRISPR-Cas nucleases such as GUIDE-seq and CIRCLE-seq. Dr. Tsai completed a postdoctoral fellowship at Massachusetts General Hospital & Harvard Medical School, Ph.D. in Functional Genomics and M.S. in Bioinformatics from North Carolina State University, and B.S. from the University of Michigan.

Fyodor Urnov, M.D., Ph.D.
University of California-Berkeley

Fyodor Urnov is a professor in the MCB department at UC Berkeley and the scientific director for technology and translation at the Innovative Genomics Institute. In his work (2000-2016) at Sangamo Therapeutics, he co-developed the toolbox of human native loci genome editing using engineered nucleases and made multiple contributions to its advancement to first-in-human clinical trials of genome editing, including efforts that discovered editing the erythroid enhancer of BCL11a as a method to treat the hemoglobinopathies. In his work at the IGI Fyodor leads collaborative efforts to develop CRISPR-Cas approaches to treat the hemoglobinopathies, inherited disorders of the immune system, and radiation injury.

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