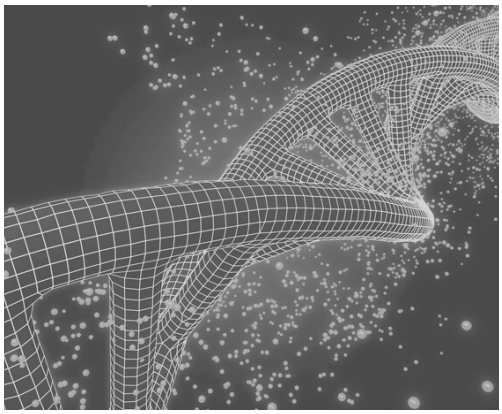

ASGCT 2021

Early-Stage Development

Pre-Meeting Workshop

Monday, May 10

3 - 7 p.m. ET

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Sponsors

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

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Early-Stage Development

Co-Chairs: Katy Spink, Ph.D., and Rasika Kalamegham, Ph.D.

Session 1
Challenges and Successes in Preparing for Clinical Trials

3 – 3:15 p.m.
Engineering and Evolving Next Generation AAVs: From the Lab to the Clinic
David Schaffer, Ph.D., UC Berkley Stem Cell Center and 4D Molecular Therapeutics

3:15 – 3:30 p.m.
Lessons Learned on the Road to the Clinic in Cell Therapy
Kim Noonan, Ph.D., WindMIL Therapeutics

3:30 – 3:45 p.m.
Considerations for Materials and Manufacturing
Joe Gold, Ph.D., City of Hope

3:45 – 4 p.m.
Developing a New Product: Considerations From the Regulatory Perspective
Florence Salmon, Ph.D., formerly Novartis

4 – 4:45 p.m.
Panel Discussion With Speakers
Moderators

- Rasika Kalamegham, Ph.D., Genetech Inc.
- Katy Spink, Ph.D., Dark Horse Consulting Group

Early-Stage Development

Co-Chairs: Katy Spink, Ph.D., and Rasika Kalamegham, Ph.D.

Session 2
Funding and Investment for a Promising New Therapy

5:15 – 5:30 p.m.
The Partnering Approach for a Promising New Therapeutic Modality
Barbara Lueckel, Ph.D., Roche

5:30 – 6 p.m.
Venture Capital Funding in Gene and Cell Therapy
Anna French, D.Phil., Qiming Venture Partners USA
Deborah Palestrant Ph.D., 5 AM Ventures

6 – 6:15 p.m.
The Venture Philanthropy Model
Debra Miller, CureDuchenne

6:15 – 7 p.m.
Panel Discussion With Speakers
Moderators:

- Rasika Kalamegham, Ph.D., Genetech Inc.
- Katy Spink, Ph.D., Dark Horse Consulting Group

Anna French, D.Phil.
Qiming Venture Partners USA

Anna French, D.Phil. is a Partner at Qiming Venture Partners USA, based in Cambridge, MA. She serves on the board of WindMIL Therapeutics, Jasper Therapeutics and Umoja Biopharma and is a board observer with Talaris Therapeutics and PlateletBio. Prior to joining Qiming, Dr. French was a management consultant at the Boston Consulting Group (BCG) where she advised leading biopharma companies on their strategy and operations. She also led a global industry-academic consortium focused on cell therapy commercialization. Dr. French earned a D.Phil. from the University of Oxford, UK where her research focused on hematological differentiation of human induced pluripotent stem (iPS) cells. She has over 20 publications in the field of stem cell research.

Joseph Gold, Ph.D.
City of Hope

Joseph Gold received his Ph.D. in Cell and Developmental Biology from Harvard University in 1991 and was a postdoctoral fellow at UCSF in the Laboratory of Radiobiology and Environmental Health. He subsequently worked at Geron Corporation for 16 years, where he directed basic research into stem cells and their therapeutic applications, with a strong emphasis on cardiovascular disease and diabetes. Prior to assuming his role at City of Hope in 2017, Dr. Gold served as Stanford's Cardiovascular Institute Assistant Director of Translational Research from 2012-2016, working to bring cell therapies for heart disease to fruition. His current role at City of Hope as Senior Director of Manufacturing and director of the Center for Biomedicine and Genetics, a GMP facility that helps researchers move their novel cell and gene therapies to the clinic, allows him to apply his 30 years of experience in cell therapy development to truly translational work that will impact cell and gene-based therapeutics for diseases including cancer, heart failure, and sickle cell disease.

Rasika Kalamegham, Ph.D.
Genentech Inc.

Rasika Kalamegham, Ph.D, is Executive Director and Head of US Product Development Regulatory Policy at Genentech, a member of the Roche Group. She joined Genentech in 2015 bringing considerable scientific, regulatory and policy experience to her role. Her portfolio includes personalized medicine including cell and gene therapy. Rasika has recently been focused on influencing FDA's regulation of digital health - from SaMDs to development of novel digital endpoints to decentralized clinical trials. A geneticist by training, she studied cell-fate specification during her post-doctoral work at the NIH. She then worked in increasingly senior positions at Friends of Cancer Research (FoCR), The Pew Charitable Trusts and The American Association for Cancer Research (AACR) before joining Genentech. She plays an active role in many trade groups including BIO, PhRMA, Transcelerate and ARM and serves in leadership positions on several workgroups. Her work has led to important regulatory policy outcomes including FDA guidances (Codevelopment of Two or More New Investigational Drugs for Use in Combination, Use of Public Human Genetic Variant Databases to Support Clinical Validity for Genetic and Genomic-Based In Vitro Diagnostics etc.), passage of the Generating Antibiotics Incentives Now or GAIN Act (signed into law by President Barack Obama), Research to Accelerate Cures and Equity (RACE) for Children Act, Vice President Biden's Cancer Moonshot Initiative and others. She is widely published in peer-reviewed journals and has authored text book chapters. Rasika and her husband Jim reside in Washington, D.C with their recently adopted retired racing greyhound Fynn and her sourdough starter Ffyeasty.

Barbara Lueckel, Ph.D.
Roche

Barbara is the Global Head of Research Technologies Partnering and a member of the Roche Pharma Partnering leadership team since May 2019. In her role she is working closely with the two research and early development organizations at Genentech and Roche (gRED and pRED) to identify transformative science developed by biotechs around the globe and help set up partnerships with such biotechs to enable novel discovery programs with the goal to increase the number of medicines impacting patients' lives. Prior to that role, Barbara worked for 12 years as BD project leader and alliance manager in Roche Partnering on a broad range of discovery partnerships. Prior to Roche, Barbara worked at Novartis for ten years focusing on drug delivery technologies. She is trained as a pharmacist with a Ph.D. from the University of Basel.

Debra Miller
CureDuchenne

Debra Miller is Founder and C.E.O. of CureDuchenne, a leading nonprofit focused on saving this generation of children and young adults with Duchenne muscular dystrophy. She and her husband started the organization in 2003 after their only son, Hawken, was diagnosed with Duchenne. Debra and her team have brought many firsts to the Duchenne community, including a Duchenne-focused venture philanthropy model, an annual nationwide community education event tour (virtual for now), a Duchenne Physical Therapy Certification program and a novel Biobank. Debra proudly serves as Patient Representative on TREAT-NMD's Executive Committee and reviews Duchenne applications for the Department of Defense's Congressionally Directed Medical Research Program. A native Californian, Debra graduated from Mariner's High School in Huntington Beach and earned a Bachelor of Arts in Communication Studies from the University of California Los Angeles. Prior to CureDuchenne, Debra had a career in publishing with positions at IDG Communications, Cahners Publishing, Ziff-Davis Publishing, and Scholastic Publishing. She also worked in management at PC Magazine and was an independent stock trader.

Kimberly Noonan, Ph.D.
WindMIL Therapeutics

Kimberly Noonan, Ph.D., is a scientific co-founder of WindMIL Therapeutics along with Ivan Borrello, M.D. Together Dr.'s Borrello and Noonan pioneered the use of marrow infiltrating lymphocytes (MILs) for the treatment of cancer. Together with a small team, Drs. Borrello and Noonan translated this laboratory finding into a clinical trial, the first ever adoptive T-cell therapy at Johns Hopkins University. The first patients were treated in 2007 with the seminal work described in a 2015 Science Translational Research publication. Dr. Noonan received her B.S. in Microbiology and Ph.D. in Immunology from the University of Pittsburgh. She also holds an M.P.H. from Johns Hopkins University. Dr. Noonan has published many peer-reviewed papers, review articles, and book chapters and holds several patents. She joined the faculty of Johns Hopkins University in 2004 and maintains an adjunct faculty appointment. In her current role as Executive Vice President & Chief Scientific Officer at WindMIL, Dr. Noonan leads a dynamic scientific team in continuing to understand the mechanism and function of MILs, CAR-MILs and novel gene modifications of MILs. They are working toward the next generation of cellular therapies utilizing the MILs platform and work closely with the Process Science and Manufacturing team to revolutionize the manufacturing of MILs. Dr. Noonan also works closely with the C.E.O., Board of Directors, and the WindMIL Leadership Team to strategically advance the scientific, clinical, manufacturing and business prospects that will lead WindMIL to the forefront of the future of adoptive cell therapy for cancer patients.

Deborah Palestrant, Ph.D.
5AM Ventures

Deborah Palestrant, Ph.D., M.B.A. joined 5AM Ventures in 2018 as Partner, Head of 4:59. Previously, Dr. Palestrant was Vice President of Corporate Development & Strategy at Relay Therapeutics, where she executed business strategy including alliances, partnerships, and other collaborations and led communications. She has over 15 years of life sciences industry experience including drug discovery, company creation, operations, business development, and strategy. Following her postdoctoral fellowship, Dr. Palestrant was a lab head at the Novartis Institutes for Biomedical Research, where she advanced multiple structure-based drug discovery programs. In 2010, Dr. Palestrant joined Third Rock Ventures as a senior associate and helped to build and launch Blueprint Medicines (NASDAQ: BPMC). Upon its launch, Dr. Palestrant served an interim role in operations at Blueprint Medicines. Subsequent to her return to Third Rock Ventures, Dr. Palestrant helped to conceive and launch Editas Medicine (NASDAQ: EDIT), and as part of the original founding team, she led the business development group until joining Relay Therapeutics. Dr. Palestrant holds a Ph.D. in biochemistry and molecular biophysics from Columbia University, an M.B.A. from Northeastern University, and was Damon Runyon Cancer Research Foundation Postdoctoral Fellow at The Scripps Research Institute. She is based in the Boston, MA office.

Florence Salmon, Ph.D.
formerly Novartis

Florence Salmon was formerly Portfolio Head Regulatory Affairs CMC Cell and Gene Therapies at Novartis. Florence holds a Ph.D. in Molecular and Cellular Biology from Strasbourg University. She started her career in the area of Product Safety, moved to DMPK and PK modelling, and later into building early development packages for innovative medicines at TNO in Leiden/Zeist (Netherlands). She joined uniQure (Amsterdam, Netherlands) in 2009 where she led the approval of Glybera (first gene therapy in the EU) in 2012 and supported the development of a number of AAV-based gene therapy vectors for various disease areas. She joined Novartis in 2014 where she led the submission and approval of Kymriah (first CAR-T product) for the treatment of acute pediatric leukemia and adult lymphoma worldwide before working on the worldwide approvals of Luxturna and Zolgensma.

David Schaffer, Ph.D.
University of California, Berkeley

David Schaffer is the Hubbard Howe Professor of Chemical and Biomolecular Engineering, Bioengineering, and Molecular and Cell Biology at the University of California, Berkeley, where he also serves as the Director of the California Institute for Quantitative Biosciences (QB3). He received a B.S. from Stanford University in 1993 and a Ph.D. from MIT in 1998, both in chemical engineering. He then conducted a postdoctoral fellowship at the Salk Institute for Biological Studies before joining Berkeley in 1999. There, he applies engineering principles to optimize gene and stem cell therapies, work that includes developing the concept of applying directed evolution to engineer targeted and efficient viral gene therapy vectors as well as new technologies to investigate and control stem cell fate decisions. In addition, he has co-founded six companies, and one, 4D Molecular Therapeutics, has advanced this technology into numerous human clinical trials. He has published >220 papers, has advised >90 graduate students and postdoctoral fellows, is an inventor on >50 patents, and has received recognitions including the Andreas Acrivos Professional Progress Award, the American Institute of Chemical Engineers Pharmaceutical and Bioengineering Award, the American Chemical Society Marvin Johnson Award, the ACS BIOT Division Young Investigator Award, and the Biomedical Engineering Society Rita Shaffer Award.

Katy Spink, Ph.D.
Dark Horse Consulting Group

Dr. Spink has been COO and Managing Partner at Dark Horse since 2018. She has been in the Advanced Therapies space since 2003, most recently as Chief Operating Officer at Asterias Biotherapeutics from 2013 to 2018. She wore many hats at Asterias with responsibilities ranging from corporate strategy, business development, intellectual property and investor relations to process development, research, program management, manufacturing, quality and facilities. She was a founding member of the company's executive team, designed and led the portfolio prioritization process to establish a corporate strategy and operating plan at the company's founding, and also led the design, build and validation of an in-house cGMP manufacturing facility. Prior to Asterias, Katy was Senior Vice President of Cell Therapy Program Operations at Geron Corporation, where she served as project team leader for Geron's cell therapy programs, including the first pluripotent stem cell-derived therapy to receive FDA clearance to initiate clinical trials. She started her career at strategic management consulting firm McKinsey & Company, where she consulted for an array of biotechnology, medical device and pharmaceutical companies on topics ranging from R&D strategy to marketing and business development. Katy has a Ph.D. in Cancer Biology from Stanford University, where she was an HHMI predoctoral fellow, and a B.A. in Biochemistry (Phi Beta Kappa, Magna Cum Laude) from Rice University. She lives in the San Francisco Bay Area with her husband and 15- and 12-year old daughters. In her spare time she enjoys traveling with her family, running and baking.

Disclosures

K.A. Noonan
WindMIL Therapeutics; Salary, Stockholder; Employee

D.V. Schaffer
4D Molecular Therapeutics; Stock, Payments; Co-founder, Chief Scientific Advisor, Director

Axent Biosciences; Stock; Co-founder

Tessera; Stock, Payments; SAB Member

Acrigen; Stock; SAB Member

Catalent; Payments; SAB Member

GenEdit; Stock; SAB Member

K. Spink
Dark Horse Consulting Group; Salary, Bonus, Stock;
COO & Managing Partner

Personalis, Inc.; Salary, Bonus, Stock; Management Position