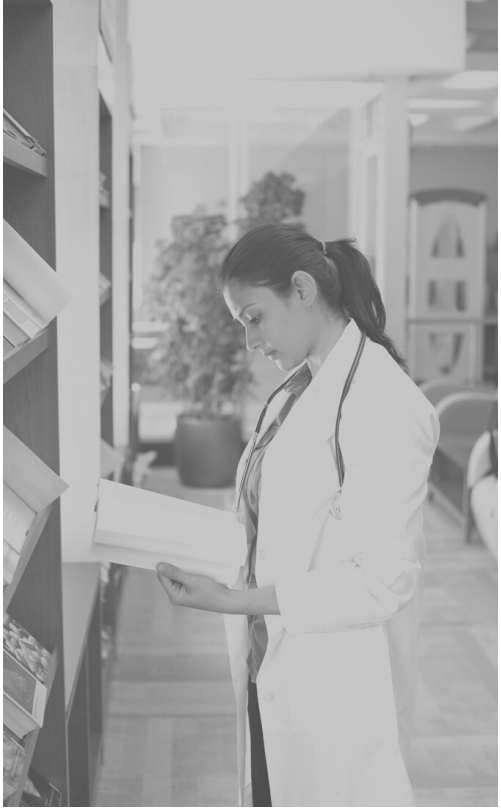

Emerging Issues in Market Access

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 Emerging Issues in Market Access:

TAYSHA GENE THERAPIES

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WHO WE ARE

Gene therapies have proven to deliver transformational benefits to patients who suffer from devastating diseases. Our mission at Taysha Gene Therapies is to build upon these advancements to eradicate monogenic central nervous system (CNS) diseases for the thousands affected. Our gene therapy strategy and unrivaled partnership with UT Southwestern help fuel our development engine.

NEXT-GENERATION PLATFORM TECHNOLOGIES

We are developing next-generation technologies to optimize key components of our AAV-based gene therapies, including redosing, transgene regulation, and capsid development.

To learn more, visit us at **Taysha Gene Therapies** and read about our upcoming **R&D Day in June 2021**

OUR APPROACH TO GENE THERAPY

The fundamental components of our approach are based on recent success in gene therapy development and commercialization: an adeno-associated virus serotype 9 (AAV9) capsid, intrathecal delivery, and an efficient manufacturing process.

AAV9 CAPSID

We use an AAV9 capsid to deliver therapeutic genes engineered to replace a mutated gene, decrease the expression of a gene, or enhance the expression of a silenced gene. AAV9 has a unique ability to cross the blood-brain barrier, making it an ideal vector for gene therapies in the CNS, and since its discovery more than 50 years ago, AAV has been one of the most well-studied vehicles for the delivery of gene therapies.

INTRATHECAL DELIVERY

We use intrathecal administration, which directly delivers our gene therapies to the cerebrospinal fluid, to facilitate optimal biodistribution and cell transduction within the CNS. The procedure is routinely performed in an outpatient setting, and in comparison to intravenous administration, it allows for a lower dose of the therapy.

EFFICIENT MANUFACTURING

Taysha's manufacturing strategy will enable consistent production of commercial- and clinical-grade AAV gene therapies. Our flexible and productive manufacturing processes allow us to produce our gene therapy product candidates efficiently at scale. Through our partnerships with UT Southwestern and Catalent, we have access to clinical and commercial-ready production capacity that utilizes a suspension HEK293 process to produce AAV9. Our internal manufacturing facility designed to produce both clinical and commercial product is expected to be online in 2023.

UNPARALLELED GENE THERAPY PIPELINE FOCUSED EXCLUSIVELY ON MONOGENIC CNS DISORDERS

PROGRAM		INDICATION	DISCOVERY	PRECLINICAL	PHASE 1/2	PIVOTAL
NEUROGENERATIVE DISEASES						
TSHA-120	*GRT*	Giant Axonal Neuropathy				Regulatory Guidance YE 2021
TSHA-101	*GRT*	GM2 Gangliosidosis				Currently Open CTA
TSHA-118	*GRT*	CLN1 Disease				Currently Open IND
TSHA-119	*GRT*	GM2 AB Variant				
TSHA-104	*GRT*	SURF1-Associated Leigh Syndrome				IND/CTA Submission 2H 2021
TSHA-112	*miRNA*	APBD				
TSHA-111-LAFORIN	*miRNA*	Lafora Disease				
TSHA-111-MALIN	*miRNA*	Lafora Disease				
TSHA-113	*miRNA*	Tauopathies				
Visit Taysha's website to view 3 more programs in this franchise						
NEURODEVELOPMENTAL DISORDERS						
TSHA-102	*Regulated GRT*	Rett Syndrome				IND/CTA Submission 2H 2021
TSHA-106	*shRNA*	Angelman Syndrome				
Visit Taysha's website to view 8 more programs in this franchise						
GENETIC EPILEPSY						
TSHA-103	*GRT*	SLC6A1 Haploinsufficiency Disorder				
TSHA-105	*GRT*	SLC13A5 Deficiency				
TSHA-110	*mini-gene*	KCNQ2				
Visit Taysha's website to view 1 more program in this franchise						

Emerging Issues in Market Access

Co-Chairs: Francesca Cook and Tim Hunt, J.D.

Session 1: Gene Therapy Investment and Capital

3 – 3:15 p.m.

Keynote: Attracting Capital and Building a Company in the Gene Therapy Space

R.A. Session II, Taysha Gene Therapies

3:15 – 4:10 p.m.

Investor State of Play: A Panel Discussion

Moderator: Josh Schimmer, M.D., Evercore ISI
Michael Gladstone, Atlas Venture
Ritu Baral, Cowen
Craig Gordon, M.D., Capital Group
Aaron Schwimmer, Barclays Capital

Session 2: What's New in Federal Health Policy

4:10 – 4:30 p.m.

Congressional Policy Updates

Conor Sheehy, Office of Senator Tim Scott
Greg Mathis Jr., Office of Senator Mark Warner

4:30 – 4:50 p.m.

Panel Discussion

Moderator: Tim Hunt, J.D., formerly Editas Medicine

Emerging Issues in Market Access

Co-Chairs: Francesca Cook and Tim Hunt, J.D.

Session 3: How the Payer Community is Adapting to Support Gene & Cell Therapy Access

5:20 – 5:30 p.m.
Payor Reimbursement Trends for Cell & Gene Therapy
Dave McLean, Ph.D., Emerging Therapies Solutions

5:30 – 5:40 p.m.
Big Payers Paying Big Bills: Perspectives on Gene & Cell Therapies
Matthew Fickie, M.D., Highmark Inc.

5:40 – 5:50 p.m.
Self-Insured Employer Solutions
Bruce Pyenson, Milliman

5:50 – 6 p.m.
Warranties and Risk Transfer Vehicles for Cell and Gene Therapies
Brooks Wildasin, CareMetx LLC

6 – 6:30 p.m.
Panel Discussion
Moderator: Francesca Cook, REGENXBIO

Session 4: Tales From the Trenches: Navigating Coverage Challenges for Gene Therapy

6:30 – 6:45 p.m.
A Patient Perspective: Fighting for Zolgensma Coverage
Katee & Jason DellaMaggiora, parents of Lincoln

6:45 – 7 p.m.
Panel Discussion
Moderator: Max Bronstein, MGB Consulting

Ritu Baral
Cowen

Ritu Baral joined Cowen in August 2014 as a senior biotechnology analyst and managing director. Ms. Baral has more than eleven years of experience in biotechnology finance, including over seven years in biotechnology equity research. Her coverage has focused on rare diseases and neurology. From August 2006 until June 2014, she held a series of positions in biotechnology equity research at Canaccord Genuity, including senior analyst and managing director. Before that, Ms. Baral was an equity research associate at JMP Securities and a senior associate at the Trout Group. Previously, she was a research associate at Columbia University's Department of Medicine, where she participated in neuroendocrine research focused on appetite and metabolism regulation, and completed graduate coursework in immunology. Ms. Baral graduated with a B.A. in biological sciences from Barnard College. She is involved in a number of rare disease patient advocacy organizations, including as a board member of the Everylife Foundation for Rare Disease and the Industry Advisory Board of the National Tay-Sachs and Allied Diseases Foundation. She was previously on the board of directors of the Pulmonary Fibrosis Foundation.

Max Bronstein
MGB Consulting

Max G. Bronstein is a mission-driven consultant serving the biomedical community with clientele in the non-profit and corporate sectors. Previously, Max was the Senior Director of Health Policy & Corporate Affairs at Audentes Therapeutics, leading company interactions with state and federal policymakers, while driving various coalitions to advance patient-focused policies for gene therapy and regenerative medicine. In particular, Max was a leader of legislative efforts to promote payment model innovation for gene therapy products as well as ensure the strongest possible standards in the US for diagnosis of rare diseases. Previously, Max was the Chief Advocacy & Science Policy Officer at the EveryLife Foundation for Rare Diseases where he led the Foundation's policy initiatives to close the innovation gap for rare diseases and to enhance newborn screening in America. In 2016, the EveryLife Foundation successfully advocated for the passage of the 21st Century Cures Act and played a key role in advocating for newborn screening legislation in California and Florida to help babies with rare diseases. Max has also published in STAT, Pediatrics, Forbes, Nature, and the New England Journal of Medicine on topics like patient engagement in drug development and the importance of leveraging incentives for closing the innovation gap for rare diseases. He is the former Director of Science Policy at Research!America where he advocated for increased appropriations for critical health research agencies like the National Institutes of Health (NIH) and the Centers for Disease Control and Prevention (CDC). Max has held various positions in the U.S. government, including at the National Science Foundation, the U.S. House Committee on Science & Technology, and the National Institutes of Health. In 2010, Max founded a non-profit organization, the Journal of Science Policy & Governance, to empower students and young scholars to publish their work. He holds a master's degree in public policy and a certificate in science and technology policy from the Ford School at the University of Michigan in addition to a B.A. in biology from Ithaca College, with a minor in writing. When not tormenting Members of Congress, he enjoys hiking around the San Francisco Bay Area and is an avid sailor, swimmer, and diver.

Francesca Cook
REGENXBIO

Francesca Cook, M.P.H., is the Vice President, Pricing and Market Access at REGENXBIO, a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. She has over twenty years of experience in market access and healthcare policy including coverage, coding and payment strategies; value proposition development and evidence generation; and government contracting and legislation. At REGENXBIO, Ms. Cook is responsible for global pricing, market access and HEOR strategy and activities across the company's therapeutic disease portfolio. Prior to joining REGENXBIO, Ms. Cook served as Senior Vice President, Policy & Government Affairs and Program Management at PharmAthene, Inc. a biotechnology company focused on developing biological and chemical countermeasures. Before her tenure at PharmAthene, Ms. Cook served in senior management positions overseeing policy and reimbursement at Guilford Pharmaceuticals Inc. and at Covance Health Economics and Outcomes Services, a policy and reimbursement consulting firm. Earlier in her career, she served as Legislative Assistant in the U.S. Senate and worked at the U.S. Department of Health and Human Services in the Public Health Service division. Ms. Cook holds a Master of Public Health degree from Yale University School of Medicine Department of Epidemiology and Public Health and a Bachelor of Arts degree in Biology from Mount Holyoke College.

Katee and Jason DellaMaggiora
Parent of Lincoln

Jason and Katee DellaMaggiora are parents of 2 year old Lincoln who has SMA Type 1. We also have three other children: Cole (17), Mae (15), and Landry, who lived for 47 days.

Their family lives in Northern California and spend their days helping Lincoln progress and succeed. They have become quite the in-home therapists, especially due to COVID, and work hard to keep Lincoln strong. They are very grateful for the treatments now available. Lincoln had 7 doses of Spinraza and the one-time dose of Zolgensma that we fought so hard for.

Matt Fickie, M.D.
Highmark Inc.

Dr. Fickie trained as a clinical geneticist. During his nearly 10 years of clinical practice he gained experience in the business aspects of clinical genetics and therapeutics through his work with Evicore and the ACMGG Economics Committee. For the past 3 years, Dr. Fickie has worked as a medical director at Highmark, Inc. Highmark is the 4th largest Blue Cross/Blue Shield affiliate and in conjunction with the Allegheny Health Network, is the 2nd largest integrated healthcare delivery and financing system in the United States. Dr. Fickie has oversees the precision medicine portfolio.

Michael Gladstone
Atlas Venture

Michael Gladstone is a partner at Atlas Ventures and focuses on building new therapeutics companies, primarily in the areas of immunology and oncology. Michael is a co-founder, acting CEO, and board member of Third Harmonic Bio, co-founder and board observer of Q32 Bio, and a board member of Day One Biopharmaceuticals. Prior to joining Atlas in 2012, Michael worked at L.E.K. Consulting, where he focused on business development and corporate strategy for biopharma clients. Michael serves as an advisor to several organizations, including as a Scientific Advisory Board member of the Institute for Protein Innovation. Michael is also on the Corporate Advisory Committee for National Tay-Sachs and Allied Diseases.

Craig Gordon, M.D.
Capital Group

Craig D. Gordon, M.D., is an equity investment analyst at Capital Group with research responsibility for U.S. and Japanese biotechnology and pharmaceutical companies. He has 13 years of investment experience and has been with Capital Group for 11 years. Prior to joining Capital, he was a biotechnology equity research analyst and associate for the Cowen Group in New York. He holds an M.B.A. from Duke University's Fuqua School of Business; a doctor of medicine (with research distinction) and Medical Resident Teaching Award from the University of Miami, Miller School of Medicine and Jackson Memorial Hospital, respectively; completed his rheumatology training at Duke Medical Center; and holds a bachelor's degree (with distinction) in economics from Cornell University. Craig is based in Los Angeles.

Tim Hunt, J.D.
formerly Editas Medicine, Inc.

Tim is an executive with more than 20 years of experience in the biotechnology field. Most recently, he served as Chief Corporate Affairs Officer at Editas Medicine, a leading gene editing company. He joined Editas Medicine in January 2016 and oversaw global policy, early-stage commercial planning & market development, government affairs, human resources, and corporate communications. Prior to joining Editas Medicine, Tim was Senior Vice President of Public Affairs for Cubist Pharmaceuticals, the world's largest developer of antibiotics to combat superbugs, until the company was acquired by Merck in 2015. At Cubist, he oversaw global policy, government affairs, and communications. Tim received his J.D. from the Columbus School of Law at the Catholic University of America and his B.A. in History and Philosophy from Boston College. He is a member of the Board of Directors of the non-profit organization Life Sciences Cares and is a member of the Strategic Advisory Board of Spero Therapeutics

Greg Mathis Jr.
Office of Senator Mark Warner

Greg is Health Policy Advisor to Senator Mark R. Warner leading the Senator's work on the Senate Finance Committee. In this role, Greg has worked on Warner-led efforts to reauthorize the Patient-Centered Outcomes Research Institute, expand the use of telehealth in Medicare to better treat mental health and substance use disorder and more. Prior to this position Greg was a Legislative Assistant for Senator Gary C. Peters and Legislative Correspondent to Representative Jesse Jackson, Jr. Greg is originally from Detroit, Michigan and an alumnus of the University of Michigan where he majored in Political Science.

Dave McLean, Ph.D.
Emerging Therapy Solutions

A 40-year veteran in health care and managed care industries, Dave McLean brings extensive experience as a successful leader and executive. Dave is the founding CEO of Emerging Therapy Solutions, Inc (ETS). ETS serves the payer industry to provide complete outsource services to manage cell and gene therapy treatments. Dave most recently was Chairman/CEO of Medication Management Systems, Inc., a pharmaceutical care management company, that he sold to Genoa HealthCare in 2017. Previously Dave was CEO of NovoLogix, a medical pharmacy technology company, that he sold to CVS in 2013. Past roles include: CEO of RxHub, an electronic prescribing company; CEO of United Resource Networks at United Health Group, that managed organ and tissue transplants for 45 million payer lives; SVP American MedCenters, an HMO management company where he managed provider contracting, reinsurance subsidiary services, and PBM services. Dave holds a B.S. in Pharmacy from Ohio State University and a Ph.D. from the University of Minnesota

Bruce Pyenson
Milliman

Bruce Pyenson is a principal and consulting actuary with the New York office of Milliman. Bruce's practice uses data science and actuarial science to address pressing issues in healthcare. In his career, he has consulted to almost every sector of healthcare, including accountable care organizations (ACOs), employers, advocacy groups, insurers, and the biotechnology industry. Many of his projects involve integrating analytics from financial, clinical, and operational models. In 2016, Bruce was appointed a Commissioner of MedPAC—the Medicare Payment Advisory Commission—serving in a position reserved for an actuary. Bruce is adjunct clinical associate professor of New York University's College of Global Public Health and a member of the Institute for Healthcare Delivery Science at the Mount Sinai Health System.

Josh Schimmer
Evercore ISI

Josh Schimmer is a Senior Managing Director on Evercore ISI's Biotech team, focusing on small, mid, and select large-cap biotechnology companies. Dr. Schimmer was ranked #1 in Institutional Investor's All America Research Team for SMID Biotech in both 2018 and 2019 and most recently 2020. He was runner up in 2015 and 2016. Dr. Schimmer has 16 years of biotechnology research experience, previously at Piper Jaffray, Lazard Capital Markets, Leerink Partners, and Cowen & Company. In addition, he spent two years on the buy side at Davidson Kempner.

Aaron Schwimmer
Barclays Capital

Aaron Schwimmer is a Managing Director of Barclays Capital within the Healthcare Group, based in New York. He leads Barclays' investment banking coverage efforts in the biotech sector and supports the firm's broader efforts across the life science industry. Mr. Schwimmer has a long track record of executing complex equity, debt and M&A mandates for leading biotech and pharmaceutical companies. Prior to Barclays, Mr. Schwimmer worked in the business development team at MedImmune until the company was sold to AstraZeneca and was an equity research analyst covering the biotech sector at Goldman Sachs. He graduated from Hamilton College with a B.A. in Biology. Mr. Schwimmer is married with three children, and is a Member, Board of Directors, Treasurer of Saint Barnabas Hospice and Palliative Care Center of West Orange, NJ.

R.A. Session

Taysha Gene Therapies

RA Session II is the President, Founder, and Chief Executive Officer (CEO) of Taysha Gene Therapies. Mr. Session has nearly 20 years' experience in the life sciences industry, primarily in business development, corporate strategy and financial roles. Previously, he served as Chief Business Officer of the gene therapy subsidiaries of BridgeBio, as well as Senior Vice President of Corporate Strategy and Business Development at AveXis.

Conor Sheehy
Office of Senator Tim Scott

Conor Sheehy serves as the Deputy Legislative Director for Senator Tim Scott. In this role, he manages the Senator's health care, labor, and retirement security policy portfolios in addition to assisting in managing the office's legislative staff. Conor has worked for Sen. Scott's office for nearly four years, having started his time in the Senate as a legislative fellow in 2017. Prior to coming to Capitol Hill, Conor taught high school in the Bronx as a Teach For America corps member during which time he earned his Master's degree. Originally from Baltimore, MD, Conor received his B.A. from the University of Virginia, where he attended as a Jefferson Scholar.

Brooks Wildasin
CareMetx LLC

Brooks Wildasin leads OutcomeRx, the cell and gene therapy division of CareMetx, LLC. OutcomeRx focuses on solving the unique market challenges introduced by gene and cell therapies. OutcomeRx drives patient access to innovative therapies using end to end patient service models, financial and insurance products, as well as technology that unites stakeholders, spreads risk, and reinvents the healthcare experience to support emerging gene and cell therapies. Brooks drives the strategic roadmap for OutcomeRx and strives to challenge the existing paradigm for therapy access across all stakeholders. Over his career, Brooks has built and launched multiple enterprise systems to support changing legislation and initiatives for both HHS and CMS. Additionally, Brooks has extensive experience developing healthcare technology and insurance products that enable access to insurance (state based healthcare exchanges, reinsurance solutions), support patient access (integrated copay programs and HUB service programs), and drive risk sharing between Manufacturers and Payers (Specialty Therapy Warranties).

Disclosures

B. Wildasin
CareMetx, LLC; Salary; Employment

F. Cook
REGENXBIO; Salary and other compensation; Employee

M. Bronstein
Ultragenyx; Consulting fees; Consultant
Taysha Gene Therapies; Consulting fees; Consultant