

# THE COST OF A CURE

CREATING SUSTAINABLE SOLUTIONS  
FOR GENE AND CELL THERAPIES



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## PANELISTS

**OPENING REMARKS:** Larry Jameson, MD, PhD, EVP and Dean of Perelman School of Medicine  
Dan Polsky, PhD, MPP, Executive Director, Leonard Davis Institute of Health Economics

### PANEL 1

#### Innovation

Development, regulation, and innovation in gene and cell therapies

#### MODERATOR

**Rachel Sachs, JD, MPH**  
*Washington University in St. Louis*

#### PANELISTS

**Stephan Grupp, MD, PhD**, *CHOP*  
**Ke Liu, MD, PhD**, *Oncology Center of Excellence, FDA*  
**Jeff Marrazzo, MBA, MPA**, *Spark Therapeutics*  
**Salveen Richter, CFA**, *Goldman Sachs*  
**Bhaven Sampat, PhD**, *Columbia University*

### PANEL 2

#### Pricing and Affordability

Current and future pricing models, cost considerations, financing mechanisms, and affordability of gene and cell therapies

#### MODERATOR

**Rena Conti, PhD**  
*Boston University*

#### PANELISTS

**Liz Barrett, MBA**, *Novartis Oncology*  
**Jennifer Malin, MD, PhD**, *UnitedHealthcare*  
**Anand Shah, MD, MPH**, *Center for Medicare and Medicaid Innovation/CMS*  
**Jacob Sherkow, JD**, *New York Law School*  
**Steve Pearson, MD, MSc**, *ICER*



### LUNCHTIME PLENARY: Scott Gottlieb, MD, FDA Commissioner

### PANEL 3

#### Access

Challenges and opportunities to provide access for gene and cell therapies for all patients

#### MODERATOR

**Stacie Dusetzina, PhD**  
*Vanderbilt University School of Medicine*

#### PANELISTS

**Peter Bach, MD**, *Memorial Sloan Kettering Cancer Center*  
**Cindy Chmielewski**, *Patient Power*  
**Mandy Cohen, MD, MPH**, *NC Department of Health and Human Services*  
**Madan Jagasia, MD, MS, MMHC**, *Vanderbilt University School of Medicine*  
**Bhuvana Sagar, MD**, *Cigna*

### PANEL 4

#### Ideation Session

Ideation session to propose solutions for innovation, affordability, and access in gene and cell therapies

#### MODERATOR

**Justin Bekelman, MD**  
*University of Pennsylvania*

#### PANELISTS

**Rachel Sachs, JD, MPH**, *Washington University in St. Louis*  
**Rena Conti, PhD**, *Boston University*  
**Stacie Dusetzina, PhD**, *Vanderbilt University School of Medicine*

## SCHEDULE & EVENTS

### THURSDAY, SEPTEMBER 27TH

7:00PM – 9:00PM

#### Networking Dinner

**Location:** Penn Museum, Egypt Upper Gallery  
3620 South Street, Philadelphia

### FRIDAY, SEPTEMBER 28TH

8:00AM – 4:15PM

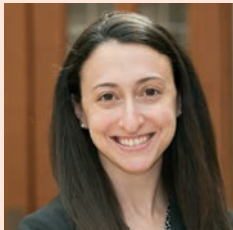
#### Symposium & Plenary

**Location:** The Study, 20 S. 33rd Street, Philadelphia

8:00AM – 8:30AM	Breakfast
8:30AM – 8:45AM	Opening Remarks
8:45AM – 10:15AM	Panel 1: Innovation
10:15AM – 10:30AM	Networking Break
10:30AM – 12:00PM	Panel 2: Pricing and Affordability
12:00PM – 1:25PM	Lunch Plenary Session
1:30PM – 3:00PM	Panel 3: Access
3:00PM – 3:15PM	Networking Break
3:15PM – 4:15PM	Panel 4: Ideation Session

# PANELIST BIOS

## INNOVATION PANEL

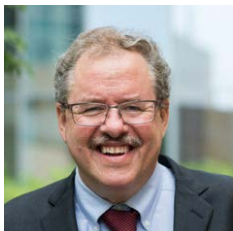


### **MODERATOR: Rachel Sachs, JD, MPH**

*Associate Professor of Law, Health Law, Washington University in St. Louis*

Professor Rachel Sachs is a scholar of innovation policy whose work explores the interaction of intellectual property law, food and drug regulation, and health law. Her work explores problems of innovation and access, considering how law helps or hinders these problems. Professor Sachs' scholarship has or will have appeared in journals that include the Michigan Law Review, the Harvard Law Review, the New England Journal of Medicine, and the Journal of the American Medical Association. Prior to joining the faculty, Professor Sachs was an Academic Fellow at the Petrie-Flom Center for Health Law Policy, Biotechnology, and Bioethics and a Lecturer in Law at Harvard Law School. She also clerked for the Hon. Richard A. Posner of the U.S. Court of Appeals for the Seventh Circuit. She received her J.D. magna cum laude from Harvard Law School and a Master of Public Health from the Harvard School of Public Health. She received her A.B. in Bioethics from Princeton University.

## PANELISTS



### **Stephan Grupp, MD, PhD**

*Director, Cancer Immunotherapy Program, CHOP*

*Director of Translational Research for the Center for Childhood Cancer Research, CHOP*

*Medical Director, Stem Cell Laboratory, CHOP*

Stephan Grupp, MD PhD, is the Chief of the Cellular Therapy and Transplant Section, Director of the Cancer Immunotherapy Program, and Director of Translational Research in the Center for Childhood Cancer Research at the Children's Hospital of Philadelphia (CHOP), and the Yetta Dietch Novotny Professor of Pediatrics at the University of Pennsylvania. A bone marrow transplant physician, he is a member of the Division of Oncology and the Medical Director of the Cell and Gene Therapy Lab. Dr. Grupp graduated from the University of Cincinnati after completing the MD/PhD program with a PhD in Immunology. He completed pediatric residency at the Boston Children's Hospital, followed by a fellowship in Pediatric Hematology/Oncology at the Dana Farber Cancer Institute and postdoctoral work in Immunology at Harvard University. He then joined the faculty at Harvard University until 1996, when he came to CHOP.

Dr. Grupp's primary area of clinical research is the use of CAR T and other engineered cell therapies in relapsed pediatric cancers. He led all of the pediatric ALL trials of CTL019 (now approved as Kymriah), including the largest and most successful engineered T cell therapy clinical trial conducted to date (1, 2), as well as the global registration trial for CTL019 (3). As a result of this work, he presented the Clinical Perspective at the July 2018 FDA ODAC meeting, at which reviewers voted 10-0 for recommendation of approval for Kymriah in pediatric ALL. His primary laboratory interest is the development of new cell therapy treatments for pediatric cancers. Dr. Grupp is a reviewer for several journals and the author of over 180 peer-reviewed journal articles, as well as numerous abstracts and book chapters.



## Ke Liu, MD, PhD

*Acting Associate Director for Oncology Cell and Gene Therapy, Oncology Center of Excellence (OCE), Food and Drug Administration*

Dr. Liu is the Associate Director of Cell and Gene Therapy, Oncology Center of Excellence, U.S. FDA. He is also the Chief of Oncology in the Office of Tissues and Advanced Therapies (OTAT, previously known as Office of Cellular, Tissue and Gene Therapies (OCTGT)) in the U.S. FDA's Center for Biologics Evaluation and Research (CBER).

Liu is a medical oncologist and internist, certified by the American Board of Internal Medicine (ABIM). He is also an attending medical oncologist in Washington Veterans Administration Medical Center. He received his M.D. from Henan Medical University in China and his PhD in Molecular Biology from Cornell University Graduate School of Medical Sciences in New York City, NY. In 2003, Dr. Liu joined U.S. FDA's CBER as a Medical Officer / Clinical Reviewer and later was selected as a Lead Medical Officer. From 2008 to 2011, he served as a Lead Medical Officer in U.S. FDA's Center for Drugs Evaluation and Research (CDER). In 2017, he was selected to serve as the acting associate director of oncology cell and gene therapy for the newly created FDA's Oncology Center of Excellence (OCE).

Dr. Liu has received multiple awards at the FDA and Center levels for his contributions to the regulatory science / research / policy, product review and approval, and staff mentoring. He has published more than 20 original articles, 10 abstracts and one book chapter. In addition, he has been representing FDA making public presentations on many occasions.



## Jeff Marrasso, MBA, MPA

*CEO and Co-Founder, Spark Therapeutics*

Jeff Marrasso has led the creation and growth of Spark Therapeutics from a research center within the Children's Hospital of Philadelphia to a fully integrated, commercial gene therapy company that is challenging the inevitability of genetic disease by discovering, developing and delivering potential treatments in ways unimaginable – until now. He also serves on Spark's board of directors.

Under Jeff's leadership, Spark has received FDA approval for and launched the first gene therapy for a genetic disease in the United States, and has established human proof-of-concept of Spark's gene therapy platform in both the retina and liver. In the five years since founding Spark, Jeff has secured more than \$1 billion in capital and built an organization of more than 325 colleagues. For two years running, Spark has been named to the top 10 of MIT Technology Review's "50 Smartest Companies". It's also been recognized as one of 2018's most innovative companies in Biotech by Fast Company. During a career that has spanned the public and private sectors, Jeff has consistently championed the potential benefits of precision medicine and healthcare reform for patients.

Prior to Spark, he helped build the first genetic testing benefit management and pharmacogenomics medicines company up to and through the acquisition of a majority of the company's shares by CVS Caremark. Previously, Jeff served as an advisor to former Pennsylvania Governor Edward G. Rendell, where he led reforms in the financing and delivery of healthcare.

Jeff currently serves as a board member of the Biotechnology Innovation Organization (BIO). Jeff received a B.A. in economics and B.S.E. in systems science and engineering from the University of Pennsylvania. He also holds a dual M.B.A. / M.P.A. from The Wharton School of the University of Pennsylvania and Harvard University, a program which he founded.

Jeff can be found on Twitter @JeffMarrasso.



### **Salveen Richter, CFA**

*Senior Analyst, Biotechnology Equity Research, Goldman Sachs*

Salveen Richter, CFA, covers the Biotechnology sector in the Global Investment Research Division. Prior to joining Goldman Sachs as a Vice President in October 2015, Salveen was a Managing Director at SunTrust Robinson Humphrey, where she covered Biotechnology. Salveen received a B.S. in Biomedical Engineering and a minor in Entrepreneurship and Management from Johns Hopkins University



### **Bhaven Sampat, PhD**

*Associate Professor, Department of Health Policy and Management, Columbia University  
Research Associate, National Bureau of Economic Research*

Bhaven Sampat is an Associate Professor in the Department of Health Policy and Management at Columbia University and a Research Associate at the National Bureau of Economic Research. He received his B.A., M.A., M.Phil. and Ph.D. (all in economics) from Columbia.

Most of his research focuses on issues at the intersection of health policy and innovation policy. His current work includes (1) various empirical studies of drug and life science patent policy in the U.S. and developing countries (2) evaluating the validity of different approaches to measure science, innovation and science-technology linkages (3) examining whether and when science is self-correcting (4) assessing the impact of federal indirect cost recovery policy on the biomedical research enterprise.

His previous work includes research on the political economy of the NIH, patent examination and patent quality, and the roles of academic patenting in university-industry technology transfer. He was previously a Robert Wood Johnson Foundation Scholar and Health Policy Research, and recipient of a Robert Wood Johnson Foundation Investigator Award in Health Policy.

## PRICING AND AFFORDABILITY



### **MODERATOR: Rena Conti, PhD**

*Associate Research Director, BioPharma and Public Policy, Institute for Health System Innovation and Policy, Boston University*

*Associate Professor, Markets, Public Policy, and Law, Boston University*

Rena M. Conti, PhD is an Associate Research Director in BioPharma and Public Policy at the Institute for Health System Innovation and Policy at Boston University. She is also an Associate Professor of Markets, Public Policy, and Law. Dr. Conti is a 2007 graduate of the Harvard University Interfaculty Initiative in health policy and an elected member of the Conference on Research on Income and Wealth.

Dr. Conti's research is on the financing, organization and regulation of medical care. She is an expert on the demand for, supply of and pricing of prescription drugs, particularly those used to treat cancer and other "specialty" conditions. She has published several studies on what factors determine the pricing of branded and generic prescription drugs and the intended and unintended consequences of the 340B drug discount program. She is interested in using classic economic theory to guide policy making on the expansion of new drug development and patient access to existing drugs.

## PANELISTS



### **Liz Barrett, MBA**

*CEO, Novartis Oncology*

Liz Barrett joined Novartis as CEO, Novartis Oncology in February 2018. She is a member of the Executive Committee of Novartis. Liz previously served as Global President of Oncology at Pfizer Inc. While at Pfizer, she held several leadership positions, including President of Global Innovative Pharma for Europe, President of the Specialty Care business unit for North America and President of United States Oncology. Prior to Pfizer, she was Vice President and General Manager of the Oncology business unit at Cephalon Inc. and worked at Johnson & Johnson. Liz started her career at Kraft Foods Group Inc.

Liz holds a Bachelor of Science from the University of Louisiana and an MBA from Saint Joseph's University, both in the U.S.





### Jennifer Malin, MD, PhD

*Senior Medical Director, Oncology and Genetics, UnitedHealthcare*

Jennifer Malin, M.D., Ph.D., is a Senior Medical Director, Oncology and Genetics, at UnitedHealthcare. In this role, she provides clinical and strategic leadership for improving the health and outcomes of cancer and genomic medicine for United Healthcare members.

After graduating from Harvard University, Dr. Malin received her medical degree and doctorate in public health from UCLA. She is board certified in internal medicine and medical oncology. A Clinical Professor of Medicine at the UCLA David Geffen School of Medicine, she is the author of more than 100 peer-reviewed articles and is widely recognized for her research on the quality of cancer care. She has served on a number of advisory boards and national committees, including the American Society of Clinical Oncology's Quality of Care Committee and the National Quality Forum's Cancer Steering Committee. Prior to joining UnitedHealthcare, she was the architect of the cancer care quality program at Anthem.

Dr. Malin continues her clinical practice by volunteering at the Veterans Affairs Greater Los Angeles Health Care System. She lives in Santa Monica, California with her two children and three dogs.



### Steve Pearson, MD, MSc

*Founder and President, Institute for Clinical and Economic Review (ICER)*

Steven D. Pearson, MD, MSc is the Founder and President of the Institute for Clinical and Economic Review (ICER), an independent non-profit organization that evaluates the evidence on the value of medical tests, treatments, and delivery system innovations to encourage collaborative efforts to improve patient care and control costs. Prominent among its evidence reports are ICER reviews of new drugs that include full assessments of clinical and cost-effectiveness along with suggested "value-based price

benchmarks" to inform policymakers and guide price and coverage negotiation. ICER convenes public hearings to discuss its evidence reports under the auspices of the California Technology Assessment Forum (CTAF) and Comparative Effectiveness Public Advisory Councils (CEPAC) in New England and the Midwest. At these meetings independent groups of evidence experts and public representatives engage with all stakeholders to debate the strength of evidence and provide recommendations on how best to apply the best evidence to clinical practice and coverage policies. Dr. Pearson is a Lecturer in the Department of Population Medicine at Harvard Medical School and also serves as Visiting Scientist in the Department of Bioethics at the National Institutes of Health. His published work includes over 100 articles on quality of care, the role of evidence-based medicine within the health care system, and related clinical, ethical, and organizational policy challenges. His book, *No Margin, No Mission: Health Care Organizations and the Quest for Ethical Excellence*, was published by Oxford University Press.

Among his past roles, from 2005-2006 Dr. Pearson served during the Bush Administration as Special Advisor on Technology and Coverage Policy within the Coverage and Analysis Group at the Centers for Medicare and Medicaid Services. Dr. Pearson has also been a Senior Visiting Fellow at England's National Institute for Health and Care Excellence (NICE), a Board Director of HTAi, the international society of health technology assessment agencies, and the Vice Chair of the Medicare Evidence Development and Coverage Advisory Committee (MedCAC).



### **Anand Shah, MD, MPH**

*Chief Medical Officer, Center for Medicare & Medicaid Innovation, Centers for Medicare & Medicaid Services (CMS)*

Anand is the Chief Medical Officer of the Center for Medicare & Medicaid Innovation (CMMI) at CMS where he leads medical and science policy development to create market-driven and patient-centered Medicare and Medicaid payment and service delivery models. At the Innovation Center, Anand focuses on the development of consumer-directed care, physician specialty and prescription drug models, and program integrity innovation. Anand is also an attending radiation oncologist at the NIH, oncologist at FDA, and Adjunct Senior Fellow at the Leonard Davis Institute of Health Economics at the University of Pennsylvania. At FDA, designed a public-private collaborative technology incubator to harness big data and advanced analytics. Anand previously provided strategic direction to several health care startups and served as an external advisor to a U.S. investment bank.

Anand was chief resident during his radiation oncology residency at Columbia. He concurrently earned his MD from the University of Pennsylvania and an MPH in health care management and policy from the Harvard School of Public Health. Anand graduated with honors from Duke with a degree in economics. He also served as a Canada-U.S. Fulbright Scholar at the University of Toronto.



### **Jacob Sherkow, JD, MA**

*Professor of Law, Innovation Center for Law and Technology, New York Law School*

Jacob S. Sherkow is a Professor of Law at the Innovation Center for Law and Technology, New York Law School. His research focuses on the intersection of advanced biotechnologies and intellectual property and regulation. Prof. Sherkow is the author of over 30 articles on these and related topics in both traditional law reviews and scientific journals, including *Science*, *Nature*, the *Yale Law Journal*, and the *Stanford Law Review*. He is also the winner of the 2018 Otto L. Walter Distinguished Writing Award and the Class of 2017 Teaching Award. In 2018, Prof. Sherkow was appointed to the National Academy of Medicine for a three-year term as part of the Academy's Emerging Leaders Forum. On matters pertaining to biotechnology and patent law, Prof. Sherkow has advised both the U.S. Senate Committee on the Judiciary and a committee of France's National Assembly.

Aside from his appointment at New York Law School, Prof. Sherkow is a Permanent Visiting Professor at the Center for Advanced Studies in Biomedical Innovation Law at the University of Copenhagen Faculty of Law and a community member of the New York Genome Center. Previously, Prof. Sherkow has held research positions at Stanford Law School and Columbia University's Mailman School of Public Health. Prof. Sherkow graduated cum laude from the University of Michigan Law School, where he was an editor of the *Michigan Law Review*. He also holds an M.A. in biotechnology from Columbia University and a B.Sc. from McGill University in molecular biology.

### KEYNOTE

#### Scott Gottlieb, MD

*Commissioner, Food and Drug Administration*

Dr. Scott Gottlieb was sworn in as the 23rd Commissioner of Food and Drugs on May 10, 2017. Dr. Gottlieb is a physician, medical policy expert, and public health advocate who previously served as the FDA's Deputy Commissioner for Medical and Scientific Affairs and before that, as a senior advisor to the FDA Commissioner.

He also worked on implementation of the Medicare drug benefit as a Senior Adviser to the Administrator of the Centers for Medicare and Medicaid Services, where he supported policy work on quality improvement and the agency's coverage process, particularly as it related to new medical technologies.

In 2013 Dr. Gottlieb was appointed by the Senate to serve on the Federal Health Information Technology Policy Committee, which advises the Department of Health and Human Services on healthcare information technology.

Dr. Gottlieb was previously a Resident Fellow at the American Enterprise Institute, and a Clinical Assistant Professor at the New York University School of Medicine in Manhattan, where he also practiced medicine as a hospitalist physician.

He completed a residency in internal medicine at the Mount Sinai Medical Center in New York, New York and is a graduate of the Mount Sinai School of Medicine and of Wesleyan University, in Middletown, Connecticut, where he studied Economics.



## ACCESS



### **MODERATOR: Stacie Dusetzina, PhD**

*Associate Professor, Health Policy, Vanderbilt University Medical Center*

*Ingram Associate Professor of Cancer Research, Vanderbilt University Medical Center*

Dr. Dusetzina is an associate professor in the Department of Health Policy and an Ingram associate professor of cancer research at Vanderbilt. She is a health services researcher whose work focuses on measuring and evaluating population-level use and costs of medications in the United States. Dr. Dusetzina's work has contributed to the evidence base for the role of drug costs on patient access to care

and policy changes that might improve patient access to high-priced drugs.

She has been recognized for her work at a national level, including being an invited participant for two working group meetings on "Patient Access to Affordable Cancer Drugs," hosted by the President's Cancer Panel, and being selected to co-author a National Academies of Sciences, Engineering and Medicine report on the same topic. Dr. Dusetzina's research has also been broadly covered by NPR, Reuters, The Washington Post, STAT News, ABC News and The Wall Street Journal.

In addition to her work on drug pricing, Dr. Dusetzina is a population health scientist and pharmacoepidemiologist specializing in large data informatics. She has authored or co-authored over 85 peer reviewed applied studies using Medicaid, Medicare, and commercial insurance claims data, and contributed several methods papers to the field.

## PANELISTS



### **Peter B. Bach, MD, MAPP**

*Director of Center for Health Policy and Outcomes, Memorial Sloan Kettering Cancer Center*

Peter B. Bach, Director of Memorial Sloan Kettering's Center for Health Policy and Outcomes, is a physician, epidemiologist, researcher, and respected healthcare policy expert whose work focuses on the cost and value of anticancer drugs. Dr. Bach is leading efforts to increase understanding of the US drug development process and develop new models for drug pricing that include value to patients. Dr. Bach described a 100-fold increase in cancer drug prices since 1965 after adjusting for inflation, and that the cost

of an additional year of life from a cancer treatment increases by \$8,500 each year.

In 2012, he and other physicians at MSK drew attention to the high price of a newly approved cancer drug and announced his hospital's unprecedented move not to offer it to patients because of its high price tag with no notable improved clinical outcomes. The drug price was later cut in half by the manufacturer.

Dr. Bach's work in lung cancer screening has led to the development of several lung cancer screening guidelines and one of the first-ever risk-prediction models for this disease. He has also proposed a number of strategies for Medicare to link payment to the value of healthcare services delivered.

Dr. Bach has been inducted into the National Academy of Medicine, American Society of Clinical Investigators and the Johns Hopkins University Society of Scholars. He served as a Senior Advisor for Cancer Policy at the Center for Medicare and Medicaid Services in 2005 and 2006. Dr. Bach has published more than 100 peer-reviewed articles and editorials in scientific journals such as the *New England Journal of Medicine* and the *Journal of the American Medical Association*. He has also written numerous healthcare-related op-eds and been featured in mainstream media outlets such as the *New York Times*, *New York* magazine, the *Wall Street Journal*, *Forbes*, *NPR*, and *60 Minutes*.

Dr. Bach completed his undergraduate studies at Harvard University and his medical studies at the University of Minnesota and the University of Chicago Harris School. He completed a residency in internal medicine at Johns Hopkins University followed by a fellowship in pulmonary and critical care medicine at the University of Chicago and Johns Hopkins. While at the University of Chicago, he was a Robert Wood Johnson Foundation Clinical Scholar. Dr. Bach has been a faculty member in MSK's Department of Epidemiology and Biostatistics since 1998 and a Senior Scholar at the International Agency for Research on Cancer since 2008.



## Cindy Chmielewski

*Myeloma Survivor, Advocate and Mentor- Patient Power*

Cynthia attended Rutgers University graduating with degrees in Psychology and Education. Upon graduation she secured her first teaching position and immediately fell in love with her chosen profession. Cynthia continued teaching for 28 years.

Now retired, Cynthia, a myeloma survivor, is using her passion for education to teach a new group of “students” - myeloma patients and their caregivers. She is on the advisory boards of the Patient Empowerment Network, the Myeloma Crowd Research Initiative and the Philadelphia Multiple Myeloma Networking Group. Using social media to educate is her passion. Cynthia educates and advocates by tweeting at @MyelomaTeacher and sharing myeloma resources, educational opportunities and clinical trial information on her MyelomaTeacher Facebook page. Ms. Chmielewski is the co-founder of the #MMSM TweetChat and is also a regular panelist on the CureTalks Podcast. Cynthia has been an invited speaker and has presented posters on using social media in hematology at the American Society of Hematology’s (ASH), the American Association for Cancer Research’s (AACR) and the European Bone Marrow Transplant (EBMT) annual meetings

Ms. Chmielewski enjoys serving as a voting member on the IRB at the University of Pennsylvania and is the patient advocate on the Myeloma Steering Committee of the ACCRU clinical trial network. Cynthia also works for Patient Power in the area of Patient Engagement.



## Mandy Cohen, MD, MPH

*Secretary, North Carolina Department of Health and Human Services*

Dr. Mandy Cohen is the Secretary of the North Carolina Department of Health and Human Services (DHHS), an agency with 16,000 employees and an annual budget of \$20 billion, and charged with improving the health and well-being of all North Carolinians. Dr. Cohen is an internal medicine physician and was previously the Chief Operating Officer and Chief of Staff at the Centers for Medicare & Medicaid Services (CMS), responsible for implementing policies for Medicare, Medicaid, the Children’s

Health Insurance Program and the Federal Marketplace. A graduate of Cornell University, she received her medical degree from Yale School of Medicine, a Master’s in Public Health from the Harvard School of Public Health and trained in Internal Medicine at Massachusetts General Hospital.



### **Bhuvana Sagar, MD**

*National Medical Executive for Oncology, Cigna*

Bhuvana Sagar, MD, is a board-certified in medical oncologist. She has been with Cigna, since 2013, and is currently the National Medical Executive for Oncology. She leads Oncology strategy, specialty care collaborative for Oncology and coverage policy from an Oncology perspective at Cigna. Before joining Cigna, she worked in community practice, delivering oncology care.

Dr. Sagar completed her medical degree in Kilpauk Medical College in India. She completed her residency in Internal Medicine at St. Luke's Roosevelt Hospital in New York City. She completed her fellowship in medical oncology at the University of Texas Medical Branch in Galveston. Dr. Sagar is board-certified in internal medicine and medical oncology and holds an active license in the State of Texas. She is a member of American Society of Clinical Oncology.

Dr. Sagar believes in educating patients regarding their disease, best treatment options, and shared decision-making. She also strongly believes that value of therapy needs to be taken into account while providing cancer care. Dr. Sagar's experience within her own medical practice has proven invaluable at Cigna, allowing her to establish networks and ensuring the utmost in customer and provider experience.



### **Madan Jagasia is MD, MS, MMHC**

Dr. Madan Jagasia, MD; MS; MMHC is Professor of Medicine at Vanderbilt University Medical Center, Department of Medicine, Division of Hematology-Oncology. As the medical director of the Stem Cell Transplant Program (2010-2018), he helped establish the outpatient transplant unit (2003), and the Long-term transplant clinic (LTTC) (2006). His research focus is in graft-versus-host disease (GVHD), an immune mediated disorder that occurs after HCT. The LTTC program was one of the founding centers of the Chronic GVHD Consortium, a federally funded program. As a steering committee member of the

NIH Consensus Criteria for Chronic GVHD, and co-chair the working group for Diagnosis and Scoring of Chronic GVHD, he helped define the 2014 Consensus Criteria for Chronic GVHD. He leads the cellular therapy clinical trial team at the Vanderbilt-Ingram Cancer Center (VICC), a NCI designated comprehensive cancer center. The program has been a core center of the Bone Marrow Transplant Clinical Trial Network (BMT CTN) since 2014. He has published over 160 articles in peer-reviewed journals and leads multiple investigator-initiated studies. He serves as a reviewer for multiple journals including *Blood*, *Bone Marrow Transplantation*, and *Biology of Blood and Marrow Transplantation*. He is the co-leader of the Translational Research and Interventional Oncology (TRIO) program within VICC. In 2016, he was appointed as the Medical Director of the division of Hematology-Oncology, and in July 2018, he was appointed as the Chief Medical Officer of the VICC.

## CO-CHAIRS BIOS

**Abby Alpert, PhD**

*Assistant Professor of Healthcare Management, the Wharton School  
Senior Fellow, Leonard Davis Institute of Health Economics*

Abby Alpert, PhD, is an Assistant Professor of Health Care Management at The Wharton School at the University of Pennsylvania. She is also a Faculty Research Fellow at the National Bureau of Economic Research and Senior Fellow at the Leonard Davis Institute. She is a health economist with a research focus on pharmaceutical policy. Prior to joining Wharton, she was an Assistant Professor of Economics and Public Policy at The Paul Merage School of Business at the University of California in Irvine and she was an Associate Economist at the RAND Corporation. She received her Ph.D. in Economics from the University of Maryland and B.S. in Mathematics and Economics from the University of Chicago.

**Justin Bekelman, MD**

*Director, Penn Center for Cancer Care Innovation at the Abramson Cancer Center  
Associate Professor of Radiation Oncology, Perelman School of Medicine  
Associate Professor of Medical Ethics and Health Policy, Perelman School of Medicine  
Senior Fellow, Penn Center for Precision Medicine  
Senior Fellow, Leonard Davis Institute of Health Economics*

Justin E. Bekelman, M.D., is Director of the Penn Center for Cancer Care Innovation at the Abramson Cancer Center, Associate Professor in the Departments of Radiation Oncology and Medical Ethics and Health Policy at the Perelman School of Medicine, Faculty in the Center for Health Incentives and Behavioral Economics, and Senior Fellow at the Penn Center for Precision Medicine and the Leonard Davis Institute for Health Economics, all at the University of Pennsylvania. Dr. Bekelman leads research programs in delivery system and payment reform and cancer comparative effectiveness research, integrating methods from the fields of epidemiology, clinical trials, health economics and public policy. Dr. Bekelman's research has appeared in scientific journals such as the Journal of the American Medical Association and the Journal of Clinical Oncology and has been featured in the mainstream media, including the New York Times, the Wall Street Journal, National Public Radio, and news outlets in Canada and Europe. He has received research funding from the National Cancer Institute, the American Cancer Society, PCORI, and philanthropic sources. Dr. Bekelman completed his undergraduate studies at Princeton University in the Woodrow Wilson School of Public and International Affairs and his medical training at Yale University, Johns Hopkins, and Memorial Sloan Kettering Cancer Center.



## Steve Joffe, MD, MPH

*Emanuel and Robert Hart Professor of Medical Ethics and Health Policy, Perelman School of Medicine  
Professor of Pediatrics at the Perelman School and Children's Hospital of Philadelphia  
Senior Fellow, Leonard Davis Institute of Health Economics*

Steven Joffe, MD, MPH, is the Emanuel and Robert Hart Professor of Medical Ethics and Health Policy at the University of Pennsylvania Perelman School of Medicine. He serves as Chief of the Division of Medical Ethics, and directs the Penn Fellowship in Advanced Biomedical Ethics and the Penn Postdoctoral

Training Program in the Ethical, Legal and Social Implications of Genetics and Genomics. He is also Professor of Pediatrics at the Children's Hospital of Philadelphia.

Dr. Joffe attended Harvard College, received his medical degree from the University of California at San Francisco (UCSF), and received his public health degree from UC Berkeley. He trained in pediatrics at UCSF and in pediatric hematology/oncology at the Dana-Farber Cancer Institute and Boston Children's Hospital.

Dr. Joffe's clinical work is in stem cell transplantation in children. His research addresses the ethical challenges that arise in the conduct of clinical and translational investigation, both in pediatric oncology and other areas of medicine and science. He has been the principal investigator (PI) of NIH, PCORI and foundation-funded studies that examine the roles and responsibilities of PIs in multicenter randomized trials, accountability in the clinical research enterprise, governance of learning activities within learning healthcare systems, return of individual genetic results to participants in epidemiologic cohort studies, and the integration of genomic sequencing technologies into cancer care. He is a member of the U.S. Food and Drug Administration's (FDA) Pediatrics Ethics Subcommittee, the American Society of Clinical Oncology Ethics Committee, and the National Human Genome Research Institute's Genomics and Society Working Group.



## Dan Polsky, PhD, MPP

*Executive Director, Leonard Davis Institute of Health Economics  
Professor of Medicine, Perelman School of Medicine  
Robert D. Eilers Professor in Health Care Management and Economics, the Wharton School*

Daniel is Executive Director of the University of Pennsylvania's Leonard Davis Institute of Health Economics, is a Professor of Medicine in the Perelman School of Medicine and the Robert D. Eilers Professor of Health Care Management in the Wharton School. He currently serves on the Congressional

Budget Office's Panel of Health Advisers, the Institute of Medicine Board on Population Health and Public Health Practice, and National Academies of Sciences, Engineering, and Medicine's Health and Medicine Division Committee. He was the Senior Economist on health issues at the President's Council of Economic Advisers in 2007-08. His research was awarded AcademyHealth's Article of the Year in 2014. He received a Ph.D. in Economics from the University of Pennsylvania in May 1996 and a Master of Public Policy from the University of Michigan in 1989. His areas of interest include health care access, workforce, and economic evaluation of medical and behavioral health interventions. He is a coauthor of the book "Economic Evaluation in Clinical Trials" published by Oxford University Press.



## SYMPOSIUM STAFF



### Liz Pecan

*Symposium Director  
Analyst, Partners in Health*

Liz Pecan is currently the Symposium Director for The Cost of the Cure. She is also currently working as a data analyst for an oncology program run by Partners in Health and the Dana Farber Cancer Initiative in Haiti, where she is building and developing an analytics system to improve cancer care for patients across the country. Prior to joining Partners in Health, Liz worked as a healthcare consultant and primarily spent her time working on the launch of Keytruda and a new biosimilar portfolio at Merck Oncology. She received her BA in Medical Anthropology from the University of Pennsylvania.



### Emily Shields

*Logistics Director  
Executive Assistant, Leonard Davis Institute of Health Economics*

Emily Shields is currently the Logistics Director for The Cost of the Cure Symposium. She is also currently the Executive Assistant for the University of Pennsylvania's Leonard Davis Institute (LDI). In her role as Executive Assistant, Emily assists and represents the Executive Director by performing administrative duties and manages all LDI events. Prior to joining LDI, Emily served as the Resource Development Coordinator for the Boy & Girls Club of Chester where she worked with the Board of Directors and managed general fundraising tasks and donor cultivation. Prior to the Boys & Girls Club, Emily completed a year of service with AmeriCorps in Lowell, Massachusetts working with at-risk youth. She received her BA in Justice, Law, & Psychology from American University.

## ORGANIZING COMMITTEE & SPONSORS

### CO-CHAIRS

Abby Alpert, PhD, *University of Pennsylvania*  
 Justin Bekelman, MD, *University of Pennsylvania*  
 Steve Joffe, MD, MPH, *University of Pennsylvania*  
 Dan Polsky, PhD, MPP, *University of Pennsylvania*

### ORGANIZING COMMITTEE

Gideon Blumenthal, MD, *OHOP, FDA*  
 Rena Conti, PhD, *Boston University*  
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 Paul Melmeyer, MPP, *National Organization for Rare Diseases*  
 Rachel Sachs, JD, MPH, *Washington University in St. Louis*  
 Anand Shah, MD, MPH, *Center for Medicare and Medicaid Innovation, CMS*

### QUESTIONS?

Liz Pecan, *Symposium Director*, [epecan@wharton.upenn.edu](mailto:epecan@wharton.upenn.edu)  
 Emily Shields, *Logistics Director*, [shielde@wharton.upenn.edu](mailto:shielde@wharton.upenn.edu)

### THIS SYMPOSIUM MADE POSSIBLE BY:



# CELL THERAPY OVERVIEW

## CELL THERAPY OVERVIEW

Treatment Approach	Adoptive Cell Transfer (ACT): collecting and using patient or donor immune cells to treat cancer
Current Concepts	TIL (Tumor Infiltrating Lymphocytes) TCR (T-Cell Receptor) CAR (Chimeric Antigen Receptor)
Current Applications	Cancer (CAR-T)

## CURRENTLY APPROVED CELL-THERAPIES

CAR-T	<p>Patient specific T-cells are genetically engineered to produce CARs on their surface. CARs allow the T-cells to recognize specific antigens on tumor cell surfaces.</p> <p>Approved CAR-T therapies are limited to treatments for selected blood cancers.</p>
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## YESCARTA (AXICABTAGENE CILOLEUCEL)

Company	Gilead/Kite
Approval Date (US)	October 2017
Indication	YESCARTA is a CD19-directed genetically modified autologous T cell immunotherapy indicated for the treatment of adult patients with relapsed or refractory large B-cell lymphoma after two or more lines of systemic therapy, including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, primary mediastinal large B-cell lymphoma, high grade B-cell lymphoma, and DLBCL arising from follicular lymphoma.
Efficacy & Safety	<a href="#">Prescribing Information</a>
Listed WAC Price	\$373,000

## KYMRIAH (TISAGENLECLEUCEL)

Company	Novartis
Approval Date (US)	2017
Indication	<p>KYMRIAH is a CD19-directed genetically modified autologous T-cell immunotherapy indicated for the treatment of:</p> <ul style="list-style-type: none"> <li>• Patients up to 25 years of age with B-cell precursor acute lymphoblastic leukemia (ALL) that is refractory or in second or later relapse.</li> <li>• Adult patients with relapsed or refractory (r/r) large B-cell lymphoma after two or more lines of systemic therapy including diffuse large B-cell lymphoma (DLBCL) not otherwise specified, high grade B-cell lymphoma and DLBCL arising from follicular lymphoma</li> </ul>
Efficacy & Safety	<a href="#">Prescribing Information</a>
Listed WAC Price	ALL - \$475,000 B-Cell Lymphoma - \$373,000

## CURRENT PHASE 2 & 3 CELL THERAPY PIPELINES (NON-COMPREHENSIVE LIST)

Atara	P3	Epstein-Barr virus, post-transplant lymphoproliferative disorder
	P2	Epstein-Barr virus + gastric cancer
Celgene/BlueBird	P2/3	Multiple myeloma
Celgene/Juno	P3	Diffuse large B-cell lymphoma
Gilead/Kite	P3	Relapsed/refractory diffuse large B-cell lymphoma
	P2	Indolent NHL
GSK	P2	Synovial sarcoma
	P2	Multiple myeloma
	P2	Non-small cell lung cancer
Novartis	P3	Chronic Lymphocytic Leukemia
	P3	Relapsed/refractory follicular lymphoma
	P2/3	Relapsed/refractor diffuse large B-cell lymphoma in 1st relapse / + pembrolizumab

# GENE THERAPY OVERVIEW

## GENE THERAPY OVERVIEW

Treatment Approach	Adeno-Associated Virus (AAV): non-enveloped virus that can be engineered to deliver DNA to target cells
Current Mechanisms	AAV
Current Applications	Severe diseases

## CURRENTLY APPROVED GENE THERAPIES

AAV	Adeno-associated virus (AAV) is a versatile viral vector technology that can be engineered for very specific functionality in gene therapy applications.  AAV therapies are engineered to fix genetic mutations, or normalize the expression of an overactive/underactive gene. AAV is a protein shell surrounding and protecting a small, single-stranded DNA genome.
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## LUXTURNA (VORETIGENE NEPARVOVEC-RZYL)

Company	Spark Therapeutics
Approval Date (US)	December 2017
Indication	LUXTURNA is an adeno-associated virus vector-based gene therapy indicated for the treatment of patients with confirmed biallelic RPE65 mutation-associated retinal dystrophy. Patients must have viable retinal cells as determined by the treating physician(s).
Efficacy and Safety	<a href="#">Prescribing Information</a>
Listed WAC Price	\$425,000 per eye; \$850,000 for two eyes

## CURRENT PHASE 2 & 3 GENE THERAPY PIPELINES (NON-COMPREHENSIVE LIST)

Agilis Biotherapeutics	P2/3	Aromatic amino acid decarboxylase deficiency
AveXis	P3	Spinal muscular atrophy
BioMarin	P3	Hemophilia A
Bluebird Bio	P3	Beta-thalassemia
	P2/3	Cerebral adrenoleukodystrophy
GenSight	P3	Leber hereditary optic neuropathy
Nightstar Therapeutics	P3	Choroideremia
Orchard Therapeutics	P3	Adenosine deaminase severe combined immunodeficiency
Pfizer/Spark	P3	Hemophilia B
Renova Therapeutics	P3	Congestive Heart Failure
Sangamo/Pfizer	P2	Hemophilia A
Tocagen	P3	2L High-Grade Glioma