SUMMIT

Personalized Medicine and the Patient

November 14 – 15, 2022

HOTEL COMMONWEALTH
500 COMMONWEALTH AVENUE, BOSTON, MA 02215

PMC PERSONALIZED MEDICINE COALITION

Contents

President's Message

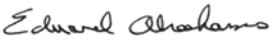
On behalf of the Personalized Medicine Coalition (PMC), I am pleased to welcome you to the *Personalized Medicine and the Patient* summit, where patients and health care professionals will come together to exchange views on the past, present, and future of personalized medicine two decades after the completion of the Human Genome Project. This summit is designed to meet what could be a pivotal moment in the history of personalized medicine.

Twenty years ago, one of PMC's chief challenges was to inspire industry leaders to invest in developing the tests and treatments necessary to tailor prevention and treatment strategies more closely to patients' biological characteristics, circumstances, and values. We have made tremendous progress on this front. Today, there are over 75,000 genetic tests and more than 286 personalized medicines on the market.

But these diagnostics and therapies are having an uneven impact. PMC research shows, for example, that only 22 percent of provider institutions are maximizing the benefits of personalized medicine. To meet patients' demands for personalized medicine, we must encourage more coordination among academia, industry, and clinicians.

The *Personalized Medicine and the Patient* summit will explore best practices and policies that will help expand patient access to personalized medicine. The summit sessions are being live streamed for maximum effect.

Sincerely yours,



Edward Abrahams, Ph.D.
President
Personalized Medicine Coalition

Personalized medicine is an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history, circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients.

WELCOME RECEPTION

IN THE FENWAY FOYER

November 14, 2022 • 6:00 p.m. ET

HOTEL COMMONWEALTH • 500 COMMONWEALTH AVENUE | BOSTON, MA 02215

Agenda

November 15, 2022

7:30 am **Registration and Breakfast**

8:15 am **Opening Remarks**

Edward Abrahams, Ph.D., President, Personalized Medicine Coalition

8:20 am **Pursuing 'the Science of the One:' A Keynote Address on the Status and Outlook for Personalized Medicine in a Post-Pandemic World**

With entire nations in the throes of the Covid-19 pandemic in 2020, Siddhartha Mukherjee urged researchers to count viral spread "within people" as well as "across peoples." The "science of aggregation," he explained in an essay published by *The New Yorker* on April 6, 2020, is most effective when it moves in step with "the science of the one." During this opening keynote presentation, the globally renowned author of *The Emperor of All Maladies* and *The Gene: An Intimate History* will provide an update on his thinking about the status and outlook for personalized medicine in a post-pandemic world.

INTRODUCTION | **Raju Kucherlapati**, Ph.D., Paul C. Cabot Professor of Genetics, Harvard Medical School

KEYNOTE SPEAKER | **Siddhartha Mukherjee**, M.D., D.Phil., Assistant Professor of Medicine, Columbia University

9:00 am **Foretelling the 'Dreaded Disease:' A Panel Discussion About Communicating with Patients in the Era of Pre-Screening and Early Cancer Detection**

Game-changing early detection. Pre-screening for genetic risk factors. In the era of personalized medicine, biomarker-based cancer diagnostics are captivating the public imagination based on their potential to help physicians get ahead of the second-leading cause of death in the United States. Against this backdrop, test developers, a patient advocate, a representative from a leading cancer center, and a medical geneticist will discuss strategies for communicating with patients about the complexities of the "dreaded disease."

MODERATOR | **William Dalton**, Ph.D., M.D., Founder, Senior Advisor, M2Gen

David Bakelman, CEO, National Pancreas Foundation

Kevin Conroy, Chairman, CEO, Exact Sciences

Robert C. Green, M.D., Professor of Medicine, Mass General Brigham and Harvard Medical School

Joshua Ofman, M.D., President, GRAIL

Sapna Syngal, M.D., Strategic Planning Director, Prevention and Early Cancer Detection, Dana Farber Cancer Institute

10:00 am **Networking Break**

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10:30 am **Biology and Beyond: A Panel Discussion About the Importance of Accounting for Biological, Environmental, and Social Factors in Personalized Medicine**

In 2021, a steering committee of patients and patient advocates came together to support the launch of a Personalized Medicine Coalition educational platform that encourages patients to advocate for health care tailored not only to their biological characteristics, but also to their circumstances and values. The online resource promises to broaden public understanding of what it means to personalize health care. During this panel discussion, the nationally recognized patient advocates leading the *More Than a Number* initiative will discuss the importance of enhancing patient-provider interactions with attention to the full scope of biological, environmental, and social factors that impact the health and well-being of every individual.

MODERATOR | **Faswilla Sampson**, Chief Operating Officer, Personalized Medicine Coalition

Deanna Darlington, President, Links2Equity

Candace Henley, Founder, Blue Hat Foundation; diagnosed with colorectal cancer in 2003

Sarah Krüg, Executive Director, Cancer101

11:15 am **Presentation of the 17th Annual Award for Leadership in Personalized Medicine**

The *Annual Award for Leadership in Personalized Medicine* recognizes an individual whose contributions in science, business, and policy have helped advance the frontiers of the field. This year's award goes to pioneering genomics researcher Geoffrey Ginsburg. Long recognized by his colleagues as a founder and luminary, Dr. Ginsburg's work emphasizes the importance of translating emerging discoveries in genomics and personalized medicine into improved health outcomes for all patients. In November of 2021, the National Institutes of Health selected Dr. Ginsburg as the Chief Medical and Scientific Officer of the *All of Us* Research Program, a flagship initiative in personalized medicine distinguished for its emphasis on diversity and inclusion.

PRESENTER | **Jay G. Wohlgemuth**, M.D., Chief Medical Officer, Senior Vice President, Quest Diagnostics

AWARDEE | **Geoffrey Ginsburg**, M.D., Ph.D., Chief Medical and Scientific Officer, *All of Us* Research Program, U.S. National Institutes of Health

12:00 pm **Lunch Break**

1:00 pm **Diversity, Equity, and Inclusion in Personalized Medicine: A Panel Discussion on How Personalized Medicine Can Improve Care for Diverse Patient Populations**

Personalized medicine calls for a more diverse, equitable, and inclusive biomedical research enterprise that can provide reliable evidence about the ways in which various health care interventions may affect subsets of heterogenous patient populations. It also calls for clinical interactions tailored more closely to patients' circumstances, preferences, and cultural backgrounds. During this panel discussion, a diverse group of health care leaders will explain how a focus on personalized medicine will improve patient care.

MODERATOR | **James W. Lillard**, Ph.D., Senior Associate Dean, Morehouse School of Medicine

Donna R. Cryer, J.D., President, CEO, Global Liver Institute; 27-year liver transplant recipient

Omar A. Escontrías, Dr.P.H., Vice President, Research, Education, and Programs, National Health Council

Pari Johnston, Vice President, Policy and Public Affairs, Genome Canada

Richard Knight, President, American Association of Kidney Patients; former hemodialysis patient

1:45 pm **Mila's Story and the Future of 'N-of-One' Therapies: A Fireside Chat About Lessons Learned from the Development of a Therapy for a Single Patient**

In October of 2019, Dr. Timothy Yu of Boston Children's Hospital co-authored a landmark paper in *The New England Journal of Medicine* reporting the successful administration of an "n-of-one" therapy specially designed for Mila Makovec, an eight-year-old patient with an ultra-rare neurological disorder. The development of the therapy points to the rapidly expanding possibilities in personalized medicine. It also raises questions about how to regulate and pay for n-of-one therapies in the future. During this fireside chat, Mila's mother will join Dr. Yu to reflect on their experiences and consider the future of personalized medicine for patients with rare diseases.

MODERATOR | **Walter Kowtoniuk**, Ph.D., Venture Partner, Third Rock Ventures

Julia Vitarello, Mila's mom; CEO, Mila's Miracle Foundation; Co-Founder, N=1 Collaborative

Timothy Yu, M.D., Ph.D., Staff Physician & Investigator, Division of Genetics and Genomics, Boston Children's Hospital; Co-Founder, N=1 Collaborative

2:30 pm **Networking Break**

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3:00 pm Streamlining the Patient Experience: A Panel Discussion on Strategies for Ensuring Patient Access to Tailored Health Care

Prior authorization. Informed consent. And, too often, a diagnostic odyssey. The complexities of the health care system can be exasperating. During this panel discussion, a caregiver, a payer, a pharmaceutical industry representative, and a health system administrator will discuss strategies for ensuring patient access to high-quality health care tailored to each patient's needs.

MODERATOR | **Alan Balch**, Ph.D., CEO, National Patient Advocate Foundation

J. Michael Graglia, Co-Founder, Managing Director, SynGAP Research Fund; caregiver to a son with a rare neurological disease

Tammy McAllister, Operations Administrator, Center for Individualized Medicine, Mayo Clinic

John M. O'Brien, Pharm.D., President, CEO, National Pharmaceutical Council

Susan Perry, Head of Strategy and Operations, Office of the Chief Medical Officer, Point32Health

3:45 pm Purpose and Progress in Pharmacogenomics: A Patient Narrative, a Pilot Program, and the Evolving Prospects for Genetically Based Prescribing Practices

During this panel discussion on the future of pharmacogenomics, a patient harmed by severe side effects from an antibiotic will reflect on the shortcomings of one-size-fits-all prescribing practices; a testing provider will review data from a pilot program showing the clinical and economic advantages of selecting and dosing drugs based on each patient's genetic characteristics; and the executive director of precision health at a leading health system will identify the obstacles still slowing the uptake of pharmacogenetic testing in clinical settings.

MODERATOR | **Cynthia A. Bens**, Senior Vice President, Public Policy, Personalized Medicine Coalition

Rachel Brummert, Communications Lead, American Society of Pharmacovigilance; patient harmed by severe side effects of antibiotic

Howard McLeod, Pharm.D., Executive Clinical Director, Precision Health, Intermountain Healthcare

Jeffrey A. Shaman, Ph.D., Chief Science Officer, Coriell Life Sciences

4:30 pm Closing Remarks

Lindsay Stephens, Program Manager, Secretary to the Board, Personalized Medicine Coalition; Diagnosed with breast cancer in 2021

Pursuing 'the Science of the One'

With entire nations in the throes of the COVID-19 pandemic in March of 2020, globally renowned author and oncologist Siddhartha Mukherjee, M.D., D.Phil., published a groundbreaking article in *The New Yorker* in which he suggested that infected patients whose bodies contain higher concentrations of the SARS-CoV-2 virus may be more likely to develop life-threatening cases of disease. If this were true, he posited, health care decision-makers might be able to use quantitative measurements of viral concentrations in blood or mucus samples to get more intensive interventions to the sickest patients, sooner.

This is not Dr. Mukherjee's only foray into personalized medicine. The Pulitzer Prize-winning author of *The Emperor of All Maladies: A Biography of Cancer*, *The Gene: An Intimate History*, and his latest book, *The Song of the Cell: An Exploration of Medicine and the New Human*, which hit the shelves on October 25, also published an essay in December in *The Wall Street Journal* that underlines the importance of ensuring that genetically based pre-screening and biomarker-based early detection blood tests do not transform the cancer care landscape into "an all-encompassing kingdom of the ill."

The *Wall Street Journal* article recognizes the promise of detecting cancers at earlier stages when they may be easier and less expensive to treat. It also alludes to the importance of carefully articulating the risks and benefits of multi-cancer early detection tests to patients. In this new era, Dr. Mukherjee observes, physicians and test developers have a renewed obligation to help patients "find a balance between curative therapies and the condition of being mortal."

The Personalized Medicine Coalition has invited Dr. Mukherjee to share with us his thinking about the future of personalized medicine. Dr. Mukherjee will be introduced by Harvard Medical School Professor and PMC co-founder Raju Kucherlapati, Ph.D.

Speakers

Raju Kucherlapati, Ph.D.

Paul C. Cabot Professor of Genetics, Harvard Medical School

Introduction

Raju Kucherlapati, Ph.D., is the Paul C. Cabot Professor in the Harvard Medical School Department of Genetics. He is also a Professor in the Department of Medicine at Brigham and Women's Hospital. Dr. Kucherlapati was the first Scientific Director of the Harvard Medical School-Partners Healthcare Center for Genetics and Genomics. His research focuses on gene mapping, gene modification, and cloning disease genes. From 1989 – 2001, Dr. Kucherlapati was the Lola and Saul Kramer Professor of Molecular Genetics and Chairman of the Department of Molecular Genetics at the Albert Einstein College of Medicine in New York.

Siddhartha Mukherjee, M.D., D.Phil.

Assistant Professor of Medicine, Columbia University

Author, *The Emperor of All Maladies: A Biography of Cancer; The Gene: An Intimate History; The Song of the Cell: An Exploration of Medicine and the New Human*

Keynote Speaker

Dr. Siddhartha Mukherjee's Pulitzer Prize-winning book, *The Emperor of All Maladies: A Biography of Cancer*, tells the story of cancer from its first description in an ancient Egyptian scroll to the gleaming laboratories of modern research institutions. A three-part documentary series based on the book, directed by Barak Goodman and executive produced by Ken Burns, aired on *PBS* stations in 2015. The film interweaves a sweeping historical narrative with intimate stories about contemporary patients and an investigation into the latest scientific breakthroughs.

Foretelling the 'Dreaded Disease'

On June 10, 2022, *The New York Times* told the stories of Jim Ford and Susan Iorio Bell.

After an experimental biomarker-based blood test detected Mr. Ford's asymptomatic pancreatic cancer at an early stage, physicians used surgery, chemotherapy, and radiation to destroy it, likely saving his life.

A similar test found a cancer-associated protein circulating in Ms. Bell's blood. But imaging tests revealed no signs of a tumor. Ms. Bell remains shaken by her prospects of developing a disease that has already afflicted both her parents.

Mr. Ford and Ms. Bell's experiences spotlight both the promise and the potential pitfalls of genetic pre-screening and blood-based early detection tests in cancer care. By identifying people whose bodies express cancer-related biological signals sooner, these tools could help physicians and patients get ahead of the second-leading cause of death in the United States. But widespread clinical adoption may also leave some patients with positive test results of uncertain significance, raising unanswered questions about if and when the "dreaded disease" may take hold. Patient-provider communication will play a key role in maximizing medical opportunities while mitigating patient anxieties in this new era of cancer care.

A patient will share views and questions about the risks and benefits of pre-screening and early detection in cancer care. Test developers and medical experts will respond.

Speakers

William Dalton, Ph.D., M.D.

Founder and Senior Advisor, M2Gen

Moderator

Dr. William "Bill" Dalton is the Founder of M2Gen, a national biotechnology subsidiary of the Moffitt Cancer Center. Dr. Dalton is also the past President, CEO, and Center Director of the Moffitt Cancer Center, a National Cancer Institute-designated Comprehensive Cancer Center in Tampa, Florida. In 2014, the Moffitt Cancer Center and the James Cancer Center at the Ohio State University founded the cancer center alliance called ORIEN (Oncology Research Information and Exchange Network) with the goal of accelerating cancer research discovery by sharing information and promoting collaborative learning through partnerships.

David Bakelman

CEO, National Pancreas Foundation

Panelist

David Bakelman is the CEO of the National Pancreas Foundation. Bakelman has a rich history in growing nonprofits through his senior-level leadership over the past 15 years. Most recently, David served as CEO of The Pap Corps — Champions of Cancer Research and raised funds for cancer research at the Sylvester Comprehensive Cancer Center — University of Miami.

Kevin Conroy

Chairman, CEO, Exact Sciences

Panelist

Kevin Conroy is the Chairman and Chief Executive Officer of Exact Sciences. Mr. Conroy led Exact Sciences through the development, clinical study, regulatory approval, and commercialization of its non-invasive colorectal cancer screening test, Cologuard®. In 2019, Exact Sciences' acquisition of Genomic Health united two of the industry's strongest brands, Cologuard and Oncotype DX®, and established the company's position as a global leader in advanced cancer diagnostics. The company is working to improve screening, early detection, and treatment guidance throughout the cancer continuum.

Robert C. Green, M.D.

Professor of Medicine, Mass General Brigham and Harvard Medical School

Panelist

Robert Green, M.D., M.P.H., is a board-certified medical geneticist and Professor of Medicine at Harvard Medical School who directs the Genomes2People Research Program at Mass General Brigham, Ariadne Labs, and the Broad Institute. Dr's empirical research and policy development is accelerating the implementation of genomic and precision medicine. His work has established the safety and feasibility of disclosing various forms of genetic risk information, assessed the impact of whole genome sequencing in primary care, created the concept of aggregate penetrance of genomic variants in a prospective population cohort, and provided early data on the clinical utility and cost-effectiveness of genomic sequencing in healthy adults.

Joshua Ofman, M.D.

President, GRAIL

Panelist

Joshua Ofman, M.D., M.S.H.S., is President at GRAIL. Josh also serves on the Board of Directors of Cell BT, an immuno-therapy company focused on the discovery and development of innovative cancer therapeutics. Previously, Josh spent more than 15 years at Amgen, where he most recently held the role of Senior Vice President, Global Value, Access and Policy. Prior to that, Josh was a faculty member in the Department of Medicine and Health Services Research at the University of California, Los Angeles (UCLA) School of Medicine.

Sapna Syngal, M.D.

Strategic Planning Director, Prevention and Early Detection, Dana Farber Cancer Institute

Panelist

Dr. Syngal is a practicing gastroenterologist at the Dana Farber Cancer Institute. As the Institute's Strategic Planning Director for Prevention and Early Detection, she is also spearheading the next steps for one of the first clinical cancer genetics and prevention programs in the world. Dr. Syngal received her M.D. from McGill University in 1990 and completed her clinical training in internal medicine and gastroenterology at Brigham and Women's Hospital. She received her M.P.H. from Harvard School of Public Health and completed a research fellowship at the Harvard Education Program in Cancer Prevention.

Biology and Beyond

On October 28, 2021, a steering committee of patients and patient advocates came together to support the launch of a Personalized Medicine Coalition educational platform that encourages patients to advocate for health care that is tailored to their biological characteristics, circumstances, and values. In an opinion essay published on November 16, 2021, in the *Boston Globe*'s health care affiliate, *STAT*, PMC Senior Vice President for Public Affairs Christopher J. Wells elaborated on the rationale for embracing a holistic definition of personalized medicine. With reference to the pandemic's tragically uneven effects across racial, ethnic, and socioeconomically defined lines, Wells writes that "genetically guided prevention and treatment should be understood as part of a broader movement toward personalized health care that also accounts for the wide-ranging needs and preferences of diverse patient populations."

What can patients do to help health care providers consider all the variables that may influence how each patient experiences an encounter with the health system and any subsequent medical interventions?

The nationally recognized patient advocates leading the *More Than A Number* initiative will reflect on this timeless challenge.

Speakers

Faswilla Sampson

**Chief Operating Officer,
Personalized Medicine Coalition**

Moderator

As the Chief Operating Officer of the Personalized Medicine Coalition and the director of PMC's *More Than A Number* initiative, Faswilla Sampson optimizes the Coalition's systems, processes and personnel while spearheading its efforts to empower patients to ask questions about personalized medicine prevention and treatment options. Sampson also oversees strategic planning and evaluates results to ensure that departmental and organizational objectives are met and are in line with the needs and mission of the organization.

Deanna Darlington

President, Links2Equity

Panelist

Deanna Darlington is an advocacy relations professional with more than 25 years of experience in government and external affairs, patient advocacy, policy, reimbursement consulting, and related fields. She specializes in engaging patient advocacy organizations on access and policy issues, which includes a focus on health disparities for vulnerable patient populations. Deanna works closely with advocates on key policy issues that support dialogue and engagement to support patient access to quality care and value.

Candace Henley

Diagnosed with colorectal cancer in 2003
Founder, Blue Hat Foundation

Panelist

When Candace Henley was diagnosed with colorectal cancer in 2003, she was a 35-year-old single mother raising five daughters. She found herself feeling as though she was falling through the cracks in the health care system. But Henley made a full recovery and took what she learned from her arduous journey to help others. In 2010, she founded The Blue Hat Foundation for Colorectal Cancer Awareness, an organization committed to supporting underserved community members who don't have access to cancer prevention resources.

Sarah Krüg

Executive Director, Cancer101

Panelist

Driven by the passion to make an impact in patient care and family engagement, Sarah Krüg has held a variety of roles within health care across sectors. The Founder of the Health Collaboratory, a global innovation hub that paves the path for collaboration in health care by amplifying the voice of the patient, Sarah has pioneered innovative improvements in health and medicine. She envisions a world in which patients and caregivers are recognized as change agents and are empowered as active partners in their care in collaboration with their health care teams.

Presentation of the 17th Annual Award for Leadership in Personalized Medicine

In April of 2003, pathbreaking genomics researcher Geoffrey S. Ginsburg, M.D., Ph.D., was among those who hailed the first sequencing of a human genome as a revolutionary moment for science and medicine.

Driven by the belief that humanity's newfound understanding of the human genome can and should have an equitable positive effect for patients across the globe, Dr. Ginsburg dedicated the next 17 years of his life to the translation of genomic discoveries into improved patient care. As the Founding Director of Duke University's Center for Applied Genomics & Precision Medicine, Dr. Ginsburg connected researchers from different fields to ensure that complementary scientific discoveries were combined to unlock their full potential to improve patients' lives. Among other achievements, his visionary work at the Center helped catalyze the development of prototype diagnostics designed to distinguish between viral, bacterial, and fungal infections in patients with a fever. He also focused on using gene expression blood testing to replace more invasive procedures for some cardiovascular patients.

By demonstrating the potential of personalized medicine to improve patient care at a moment when the significance of new discoveries in genetics had yet to be determined, Dr. Ginsburg's pioneering efforts helped inspire the development of a new generation of genetically based diagnostic tools. Those tools have since given us unprecedented insights into the genomic signatures of pathogens including COVID-19, enabled the genetically guided treatment of certain cancers, and brought new hope for answers to patients living with suspected rare genetic diseases.

After accepting the Personalized Medicine Coalition's *17th Annual Award for Leadership in Personalized Medicine*, Dr. Ginsburg, who now serves as the Chief Medical and Scientific Officer of the U.S. National Institutes of Health's *All of Us* Research Program, will share his views on the future of personalized medicine and patient care from his new vantage point.

Speakers

Jay G. Wohlgemuth, M.D.

Chief Medical Officer, Senior Vice President, Quest Diagnostics

Board Chair, Personalized Medicine Coalition

Presenter

Personalized Medicine Coalition Board Chairman Jay G. Wohlgemuth, M.D., is Chief Medical Officer and Senior Vice President, Research & Development and Medical, for Quest Diagnostics. Based at the Quest Diagnostics Nichols Institute in San Juan Capistrano, California, Dr. Wohlgemuth is responsible for Research & Development, Medical Affairs and Medical/Laboratory Quality. He is also responsible for improving outcomes for employees who use Quest's health benefits.

Geoffrey Ginsburg, M.D., Ph.D.

Chief Medical and Scientific Officer, *All of Us* Research Program, U.S. National Institutes of Health

Awardee

Geoffrey S. Ginsburg, M.D., Ph.D., is the Chief Medical and Scientific Officer of the *All of Us* Research Program at the U.S. National Institutes of Health. He leads the Division of Medical and Scientific Research and is responsible for helping to set the scientific vision and strategy for the program. Prior to joining *All of Us*, Ginsburg was the Founding Director of the Center for Applied Genomics & Precision Medicine in the Duke University School of Medicine, where he pioneered translational genomics and the development of novel diagnostics. Throughout his career, Ginsburg has demonstrated a strong commitment to interdisciplinary science and innovation, with work spanning oncology, infectious diseases, cardiovascular disease, and metabolic disorders.

Diversity, Equity, and Inclusion in Personalized Medicine

On April 22, 2021, the Personalized Medicine Coalition convened a diverse panel of leaders from across the health care spectrum for a virtual discussion on topics related to health equity and personalized medicine. To further the cause of personalized medicine, the panelists explained, health care decision-makers must build a more diverse, equitable, and inclusive biomedical research enterprise. Doing so, they emphasized, is the only way to ensure that physicians and patients have reliable evidence about the ways in which various health care interventions may affect subsets of heterogenous patient populations. Broadening the reach of health care research will help ensure that health systems remain accountable and accessible to all.

The panelists also noted that personalized medicine calls for clinical interactions tailored more closely to patients' circumstances, preferences, and cultural backgrounds.

"How we frame things has to be from the viewpoint of the person that is being served," said Jane Delgado, Ph.D., President, CEO, National Alliance for Hispanic Health. "That is why personalized medicine is the answer."

During this session, PMC will bring together a panel of health care leaders with expertise in engaging underserved communities to discuss what health care for historically underserved patient populations might look like if key decision-makers stood four-square behind this vision for the future of personalized medicine.

Speakers

James W. Lillard, Ph.D.

Senior Associate Dean,
Morehouse School of Medicine

Moderator

Dr. Lillard is a prominent immuno-biologist who develops biologics to treat chronic diseases. Most recently, his laboratory research involves dissecting the molecular mechanisms of chemokine-mediated solid tumors and heme malignancy progression using clinically annotated DNA/RNA sequencing data. His research contributions span multiple disciplines including oncology, vaccine development, biodefense, and neuro-inflammation. Dr. Lillard's cumulative peer-reviewed funding principally directed over his scientific career exceeds $30 million. He has authored more than 300 scientific communications, which have been cited over 6,000 times.

Donna R. Cryer, J.D.

27-year liver transplant recipient
President, CEO, Global Liver Institute

Panelist

Donna R. Cryer is Founder and CEO of the Global Liver Institute, the premier patient-driven liver health nonprofit organization operating with offices and partnerships across five continents. Moved by her own experience as a 27-year liver transplant recipient, Mrs. Cryer serves as a fierce advocate for the transformative potential of patient engagement in health policy, research, data, and system design. Her expertise and effectiveness in advancing the voice of patients in defining and designing equitable health care has been recognized by the United States Congress and the White House.

Omar A. Escontrías, Dr.P.H.

Vice President, Research, Education, and Programs, National Health Council

Panelist

Dr. Escontrías' expertise is centered in the areas of community and patient engagement, coalition building, health policy, and evidence-based research. Over the span of his career, Dr. Escontrías has worked with local and state governments as well as nonprofit organizations in the areas of public policy and disease prevention and health promotion to improve the health and lives of patients and communities. By the time Dr. Escontrías joined the National Health Council (NHC) in April 2022 as its Vice President of Research, Education, and Programs, he'd already spent 15 years building a career fighting systemic health care inequalities in historically underrepresented communities.

Pari Johnston

Vice President, Policy and Public Affairs, Genome Canada

Panelist

Pari Johnston leads Genome Canada's federal advocacy and policy agenda while raising awareness of the organization's mission and cross-sectoral impact among parliamentarians, senior officials, industry, community partners, and the public. Guided by the belief that genomics can change the world if responsibly and equitably applied, Pari builds strategic partnerships among diverse stakeholders to advance the role and impact of genomics in society. In this capacity, she is closely involved with Genome Canada's robust actions and commitments to Indigenous truth, reconciliation, and meaningful engagement.

Richard Knight

President, American Association of Kidney Patients

Former hemodialysis patient

Panelist

Mr. Knight is a health care professional and a former hemodialysis patient. He received a kidney transplant approximately 14 years ago. He is President of the American Association of Kidney Patients, which is the oldest and largest independent kidney patient organization in the United States.

Mila's Story and the Future of 'N-of-One' Therapies

Timothy Yu, M.D., Ph.D., co-authored a report in *The New England Journal of Medicine* for October 24, 2019, that extended the scientific and regulatory boundaries of personalized medicine. The article reviewed how Dr. Yu, a physician at Boston Children's Hospital, had developed and delivered an "n-of-one" therapy intended to treat only one person. The patient, the late Mila Makovec, was an eight-year-old girl with a genetic form of a neurological disease so rare that it had never been documented before.

Officials at the U.S. Food and Drug Administration gave Dr. Yu permission to use milasen, a custom therapy designed to block the effects of an extraneous portion of DNA whose presence was inhibiting the function of a key gene, to treat Mila's unusual form of Batten disease, a fatal condition with no cure. To finance the work, Mila's mother, Julia Vitarello, raised $3 million by creating a nonprofit research foundation and appealing to the users of the GoFundMe crowdfunding platform.

Dr. Yu and Ms. Vitarello will join moderator Walter Kowtoniuk, Ph.D., a venture capitalist focused on translating scientific breakthroughs into improved patient care, to envision a world in which physicians routinely develop n-of-one therapies. With attention to regulatory questions and strategies for ensuring the financial sustainability of the n-of-one treatment model, the conversation will explore how a race to save one girl's life has turned into a movement to push the boundaries of ultra-personalized medicine for the benefit of tens of thousands of children suffering from ultra-rare genetic diseases in the United States and around the world.

Speakers

Walter Kowtoniuk, Ph.D.

Venture Partner, Third Rock Ventures

Moderator

Walter is passionate about making a difference for patients. He spends his days focused on areas where genetics and genomics bring new insight into disease biology. Walter works where science meets business and strategy, enabling insights from the laboratory to become the next generation of medicines that can change lives. He is enthusiastic about launching companies with emphatic, truly patient-centric cultures that effectively and efficiently execute drug discovery. He places priority on taking the time to listen to the patients whom Third Rock endeavors to help, recognizing that the value the company aims to create is defined by the difference it can make in patients' lives.

Julia Vitarello

Mila's mom

Founder, CEO, Mila's Miracle Foundation

Co-Founder, N=1 Collaborative

Panelist

Julia founded Mila's Miracle Foundation in 2016 upon learning that her six-year-old daughter, Mila, had Batten disease, a fatal genetic condition with no cure. In a race to save Mila, Julia's collaboration with Dr. Timothy Yu from Boston Children's Hospital led to the first ever drug tailored to just one person, affectionately named milasen. After showing great promise in the first year of treatment, Mila's disease slowly progressed. In February of 2021, Mila's big spirit left her little body. Driven by a sense of hope and responsibility, Julia is on a mission to advance individualized medicines.

Timothy Yu, M.D., Ph.D.

Staff Physician, Investigator, Boston Children's Hospital

Co-Founder, N=1 Collaborative

Panelist

Dr. Yu is a neurologist and researcher in the Division of Genetics and Genomics at Boston Children's Hospital, an Assistant Professor at Harvard Medical School, and an Associate Member of the Broad Institute of MIT and Harvard. His research group focuses on autism, neurobiology, and genomic medicine. Dr. Yu is passionate about finding ways to bring genomic tools to the bedside to help care for patients with genetic disorders. His research initiatives range from the deployment of genome sequencing in the neonatal intensive care unit to the design and delivery of genome-guided therapeutics for sick children.

Streamlining the Patient Experience

Schedule of benefits. Prior authorization. Informed consent. The complexities of the United States health care system can make it difficult for patients to access personalized medicine.

For example, although more than 70 percent of advanced non-small cell lung cancer (aNSCLC) patients are estimated to have actionable mutations that can inform personalized treatment options, many aNSCLC patients never receive genetic testing. Even among patients who do receive genetic testing, research conducted by the Personalized Medicine Coalition shows that oncologists are still prescribing one-size-fits-all treatments to as many as 40 percent of those who could have benefitted from targeted therapies. In a *Washington Post* op-ed about President Joe Biden's research-oriented Cancer Moonshot published on February 15, 2022, PMC President Edward Abrahams lamented this failure to "save lives from cancer with tools we already have."

"While it is important to pursue long-term goals, it is also critical to ensure that cancer patients today benefit from programs, tools and therapies that have already been developed," Abrahams wrote.

With attention to emerging and established tools spanning multiple disease states, a caregiver, a payer, a pharmaceutical industry representative, and a health system administrator will discuss strategies that might make it easier for patients to access high-quality health care tailored to each patient's needs.

Speakers

Alan Balch, Ph.D.

CEO, National Patient Advocate Foundation

Moderator

Alan Balch is the CEO of the Patient Advocate Foundation and the National Patient Advocate Foundation. Alan has nearly 15 years of executive leadership in the nonprofit sector, with an emphasis on consensus-building and collaboration. Prior to joining NPAF, he served as the Vice President of the Preventive Health Partnership, a national health promotion collaboration between the American Cancer Society, the American Diabetes Association, and the American Heart Association. Prior to his work with the Preventive Health Partnership, Alan was the Executive Director of Friends of Cancer Research.

J. Michael Graglia

Caregiver to son with rare neurological disease

Co-Founder, Managing Director, SynGAP Research Fund

Panelist

Mike comes from a career in public policy, international development, and strategy. Previous roles have included establishing a new program at New America, a D.C. think tank, budget & planning at both the Gates Foundation and Emerson Collective, health care consulting at BCG, developing world university support for the World Bank Group, managing a refugee program for the International Catholic Migration Commission in Zimbabwe, and teaching math in Peace Corps Namibia. Mr. Graglia has an M.B.A. from Columbia University, where he was a Bronfman Fellow, and an M.A. in Southeast Asian studies from Johns Hopkins School of Advanced International Studies.

Tammy McAllister

Operations Manager, Center for Individualized Medicine, Mayo Clinic

Panelist

Tammy McAllister is an operations administrator within Mayo Clinic's Center for Individualized Medicine. As a strategic and operational leader in the Center, she works to advance the integration of -omic medicine into the Mayo Clinic practice, overseeing an expanse of translational and clinical implementation efforts, including innovative approaches in rare disease diagnostics and therapeutics, large-scale population health projects, genomic training and education, and the development of Mayo's Omics Data Platform.

John M. O'Brien, Pharm.D.

President, CEO, National Pharmaceutical Council

Panelist

John M. O'Brien, Pharm.D., is the President and CEO of the National Pharmaceutical Council, which sponsors and participates in research on the appropriate use of pharmaceuticals and the clinical and economic value of pharmaceutical innovation. Dr. O'Brien is responsible for overseeing NPC's policy research and communications capacity, partnerships with other health care organizations, and strategic vision. Prior to joining NPC, Dr. O'Brien was Senior Advisor to the U.S. Secretary of Health and Human Services and Deputy Assistant Secretary of Planning and Evaluation. He has also held senior policy positions in the life sciences and managed care industries.

Susan Perry

Head of Strategy and Operations, Office of the Chief Medical Officer, Point32Health

Panelist

Susan Perry is the Head of Strategy and Operations at Massachusetts-based regional health insurer Point32Health. Known for innovative partnerships in personalized medicine, Point32Health is committed to providing high-quality and affordable health care, improving the health and wellness of its members, and creating healthier communities across the country.

Purpose and Progress in Pharmacogenomics

In 2006, Rachel Brummert visited her doctor with questions about a persistent cough. Her physician found nothing out of the ordinary. But she prescribed an antibiotic to alleviate the symptoms of any potentially undetected infection.

Within 18 months, Ms. Brummert had ruptured her Achilles tendons three times.

As Ms. Brummert and the U.S. Food and Drug Administration later learned, the prescribed drug, levofloxacin, can cause tendinitis and tendon ruptures in certain patients. In an article published in *The Journal of Precision Medicine* in March of 2022, Ms. Brummert, who has now undergone more than 30 surgeries to repair recurrent tendon ruptures, explains how adverse drug reactions like the ones she experienced affect patients and the health care system in the United States:

"Annually, adverse drug events account for one million emergency room visits, 2.2 million hospitalizations, 3.5 million physician visits, and a staggering $136 billion in health care costs." In one of the most compelling demonstrations of personalized medicine's real-world impact published to date, researchers from the Teachers' Retirement System of Kentucky and Coriell Life Sciences (CLS) reported on March 8, 2022, that pharmacogenetic tests designed to detect genes that may influence a patient's likelihood of experiencing adverse drug reactions helped save the health care system $37 million in direct medical charges by optimizing treatment selections to avoid downstream health care costs.

Ms. Brummert will reflect on the shortcomings of one-size-fits-all prescribing practices. CLS' Chief Science Officer will review and discuss the significance of the data from its pilot program with the Teachers' Retirement System of Kentucky. And one of the world's foremost experts in pharmacogenomics will identify the obstacles still slowing the uptake of pharmacogenetic testing in clinical settings.

Speakers

Cynthia A. Bens

Senior Vice President, Public Policy, Personalized Medicine Coalition

Moderator

Cynthia A. Bens, Senior Vice President, Public Policy, Personalized Medicine Coalition, leads the Coalition's policy development and government relations efforts and serves as its primary liaison with Congress and federal regulators. In collaboration with PMC's Senior Vice President for Science Policy Daryl Pritchard, Ph.D., Bens is responsible for implementing research, regulatory, and reimbursement policy strategies that promote the understanding and adoption of personalized medicine concepts, services and products to benefit patients and health systems.

Rachel Brummert

Patient harmed by severe side effects of antibiotic

Communications Lead, American Society of Pharmacovigilance

Panelist

In 2006, Rachel Brummert was harmed by a fluoroquinolone antibiotic. She suffered severe adverse reactions, about which she was never warned. Rachel has channeled her frustration into advocating for others who have been harmed by a pharmaceutical drug or medical device, educating others, analyzing research, and effecting policy change. Her patient safety work led her to becoming a contributor for Drug Watch and Medshadow Foundation. She also serves as a Special Government Employee for the U.S. Food and Drug Administration and as the Communications Lead for the American Society of Pharmacovigilance.

Howard McLeod, Pharm.D.

Executive Clinical Director, Precision Health, Intermountain Healthcare

Panelist

Dr. Howard McLeod is an internationally recognized expert in precision medicine, having made novel contributions at the discovery, translation, implementation, and policy levels. He is the Medical Director for Precision Medicine at the Geriatric Oncology Consortium. Dr. McLeod chaired the NHGRI eMERGE network external scientific panel for the past decade and was a recent member of both the FDA Committee on Clinical Pharmacology and the NIH Human Genome Advisory Council. Dr. McLeod has been recognized as a Fellow of both the American Society of Clinical Oncology and the American College of Clinical Pharmacy.

Jeffrey A. Shaman, Ph.D.

Chief Science Officer, Coriell Life Sciences

Panelist

Jeffrey A. Shaman, Ph.D., M.S., is the Chief Science Officer at Coriell Life Sciences, where he oversees the company's research, education, and clinical programs and leads efforts focused on bridging the gap between genetic science and clinical application. Dr. Shaman brings years of experience in advising cross-functional teams together with his scholarship in genetics, pharmacology, stem cells, and clinical laboratory operations. Along with the CEO, he forges strategic partnerships with worldwide companies, laboratories, academic institutions, public and private self-insured companies, and federal, state, and regional health care and employee systems.

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About Us

The Personalized Medicine Coalition (PMC), representing innovators, scientists, patients, providers, and payers, promotes the understanding and adoption of personalized medicine concepts, services, and products to benefit patients and health systems.

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