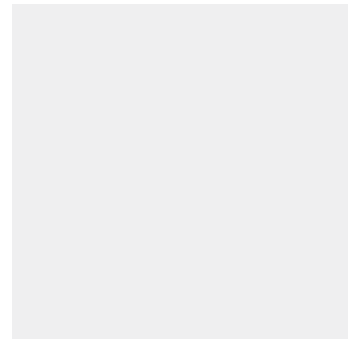
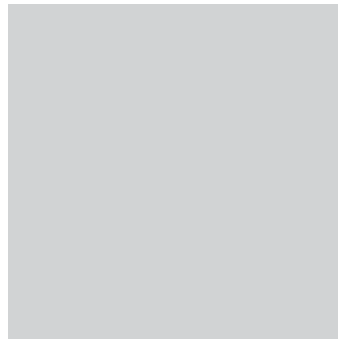
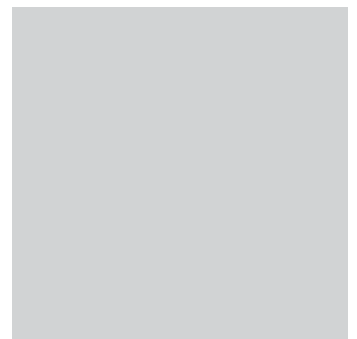
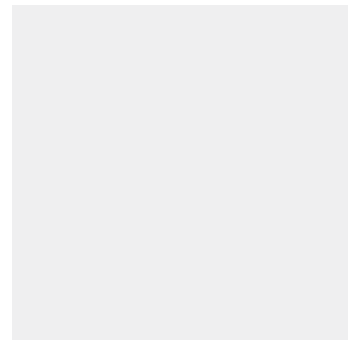


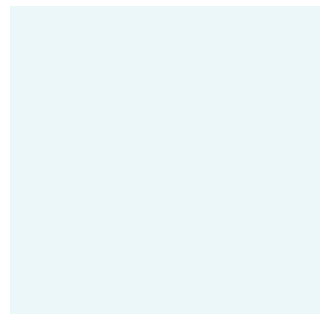
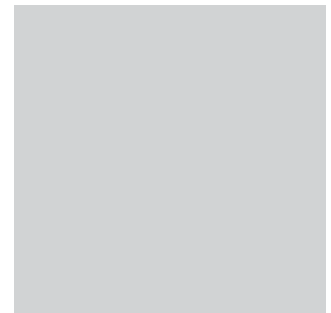


2020 ANNUAL REPORT



2020 Benefactors

Amgen Inc.
Astellas Pharma
Biogen
Bristol Myers Squibb Company
Daiichi Sankyo, Inc.
Eisai Inc.
Eli Lilly and Company
Genentech Inc.
Johnson & Johnson
Merck & Co., Inc.
Novartis Pharmaceuticals Corporation
Pfizer Inc.
PhRMA
Sanofi
Takeda Pharmaceuticals USA, Inc.
UCB S.A.



Mission Statement

The PhRMA Foundation works to improve public health by proactively investing in innovative research, education and value-driven health care.

We achieve our mission by:

- Remaining scientifically independent and nimble in an ever-evolving health care ecosystem.
- Investing in the patient perspective, including patient-centered value assessment, to empower patients and improve outcomes and efficiencies.
- Supporting and encouraging young scientists to pursue novel projects to advance innovative and transformative research efforts.
- Using data, sound methodologies and advanced technology to inform decisions.
- Supporting collaborative efforts that promote innovative research, support emerging data science and drug discovery, and build frameworks that accurately characterize the value of outcomes for a wide variety of stakeholders.





Table of Contents

Message from the Chairman	6
Message from the President	7
Using Technology and Data in Health Care to Increase Diversity in Clinical Trials	8
Value Assessment 2020 Research Awards	12
Value Assessment 2020 Challenge Awards	15
Fellowships and Grants Section	18
Health Outcomes	19
Translational Medicine	25
Pharmacology/Toxicology	29
Informatics	40
Pharmaceutics	47
Board of Directors	52
Treasurer's Report	54
Statement of Activities	55
Advisory Committees	56
PhRMA Foundation Programs for 2021	60
PhRMA Foundation Staff	62

Message from the Chairman



As the Foundation enters its 56th year of operation, we remain committed to funding research projects that answer the most perplexing healthcare questions with viable solutions.

The PhRMA Foundation's Value Assessment Initiative (VAI) is entering its fifth year, and we are seeing the real time impact of the changes we sought

to make. As we raise awareness about how the VAI is working to advance value in healthcare and reduce costs, the challenge remains not only knowing what patients value, but how that is reflected in economic evaluations.

Over the past year, our funded researchers from world-renowned universities and institutions have made numerous promising developments in this area. For example, the PedsUtil, a tool that incorporates children's voices in decisions made about their care was developed. Further, the health economics professors who received our first-place 2020 Challenge Award developed the GRACE model to fill gaps in traditional cost-effectiveness analysis by making patient perspectives a feasible metric.

Our grants and fellowships continue to support multidisciplinary projects that span the complex spectrum of drug development. The Foundation's new mission and restructured core programs, homing in on Drug Discovery, Drug Delivery, Translational Medicine, and Health Outcomes Research, have heightened our vision for healthcare systems that prioritize patient preferences. We will build on our momentum in value assessment to foster accountability throughout the care continuum, enrich evidence-based practice with literature that captures the patient voice, and build frameworks to inform decision makers.

Further, we must continue to recognize the need to address health disparities and accommodate the needs of diverse patient populations in order to build culturally competent healthcare. This year, the Foundation offered a new award program soliciting strategies to better represent patients who have long been underserved in the healthcare community. We look forward to the successful launch of a new predoctoral fellowship program to support young scientists from underrepresented communities in the pharmaceutical and biomedical sciences.

The COVID-19 pandemic has accelerated the growth of new healthcare technologies — from telehealth to wearables — but the health care sector has yet to fully leverage them, particularly when it comes to addressing the lack of diversity in clinical trials. The Foundation's new Technology and Data program aims to identify, develop, evaluate, and implement new technology-driven tools to transform research processes in a way that will help us better understand and address the root causes of racial and ethnic underrepresentation in these trials, as well as, provide a path forward to a solution.

PhRMA Foundation supported science is behind some of our most promising strategies for expanding access to care, breaking down barriers to health equity, and reducing healthcare spending systemically. This research is paving the way for innovations that can be applied and scaled where resources are most strained. Affordable, effective, and equitable health care is within reach, and with each passing year, we get another step closer to attaining it.

A handwritten signature in black ink that reads "Alfred Sandrock". The signature is fluid and cursive.

Alfred W. Sandrock, MD, PhD
Chairman, PhRMA Foundation



Message from the President



We are in the midst of a sea of change in health care – driven by the twin storms of the COVID–19 pandemic and a nationwide reckoning with systemic racism that has magnified the vast disparities among the country’s most vulnerable patients. We all have a responsibility to act to correct these imbalances and build a world in which health care is a right of all Americans.

The PhRMA Foundation is answering the call by investing more resources towards diversifying the field of value assessment research, expanding opportunities for underrepresented communities in the pharmaceutical and biomedical sciences, and leveraging data and technology to make the drug development process more inclusive.

Addressing inequality in healthcare starts with the patient perspective. This year, we announced the recipients of our new Challenge Award that prioritizes diversity and inclusion in value assessment research. The four winners of the 2021 Valuing Diversity: Addressing Health Disparities Challenge Award all identified important methods for building value frameworks that are representative of all patients. These awards are just the beginning of a broader shift towards rethinking value assessment in a way that more accurately identifies and addresses everyday drivers of health inequities.

As we endeavor to reduce disparities for critically underserved populations, we also have a responsibility to cultivate a health sciences workforce as diverse as the patients it serves. The Foundation’s new Fellowship for Underrepresented Populations in Biomedical Sciences aims to support a robust pipeline of students and early-career scientists from underrepresented populations as they pursue their careers. This initiative is part of our effort to make the biomedical and pharmaceutical sciences more representative of the country and communities it aims to serve.

We believe addressing health inequity needs to happen early in the drug development process. Our newest program seeks to leverage healthcare technology and data in innovative ways to better understand factors driving low participation of underrepresented groups in clinical trials and increase participation. These technologies will help us collect and translate data into practical guidance for research and development, ideally improving treatment safety and efficacy for minority and underrepresented populations.

Lastly, I have a bittersweet announcement to make: after more than two decades, this will be my last year with the Foundation. I have seen the organization grow and evolve, moving drug discovery and development in profound and promising ways. There has been bold, sweeping change, and with it, extraordinary progress. Weaving value into each and every one of our programs has been a significant part of my role as president. I envision value assessment taking a central space in health care and eventually becoming an inextricable part of health decision making.

Over the past 20 years, I have worked with so many wonderful people; the committee members, the Board, my staff, colleagues, and the award recipients, to name a few. Becoming passionately invested in the success of our grantees comes with the job. To this day, there is nothing more rewarding than the news of a Foundation fellow making waves in the field. I’m so proud to have been a part of the career journeys of nearly 900 scientists during my tenure. To be there at the start, when concepts are young and fragile, and later, as a budding theory gains traction, I have had the pleasure of watching our researchers build confidence in spades. They are incredible professionals and people, who I know will bring us to a new era of equitable, effective, and empowering health care.

A handwritten signature in black ink that reads "Eileen Cannon". The signature is fluid and cursive.

Eileen Cannon
President, PhRMA Foundation

Using Technology and Data in Health Care to Increase Diversity in Clinical Trials

An innovative program from the PhRMA Foundation aims to address a longstanding issue in health care, by better leveraging information technology

Achieving appropriate racial and ethnic diversity in clinical trials is a key challenge in health care, with significant challenges and consequences for patients, researchers, physicians, health care delivery systems and society.

Clinical trials are essential in establishing the efficacy and safety of new therapies, but to help deliver more informative data, they should include a pool of participants that reflects the diversity of the population who will benefit from the new therapy.

Studies show that members of diverse racial or ethnic groups may respond very differently to the therapies being tested. Yet, many minority groups — particularly Black, Asian, and Latino Americans — are all too often underrepresented in clinical trials. Use of results from trials with appropriate racial and ethnic diversity can help better inform the understanding of benefits and risk.

Organizations involved in the development and administration of clinical trials are in search of new strategies to address these issues. Among the resources that could potentially help are new, digitally based tools — ranging from social media to wearable technology — that can dramatically improve the collection and use of data. In fact, the efforts of biopharmaceutical companies and stakeholders over the past year to invest in digital health technologies have helped lay the foundation for potential long-term changes to the clinical trial process that could lead to a more streamlined approach to developing new medicines.

The health sector has the opportunity to apply these technologies in a way that is translatable to the highly complex patient-data landscape. There is enormous potential in the use of digital health technologies to help us better understand patient needs and behaviors.



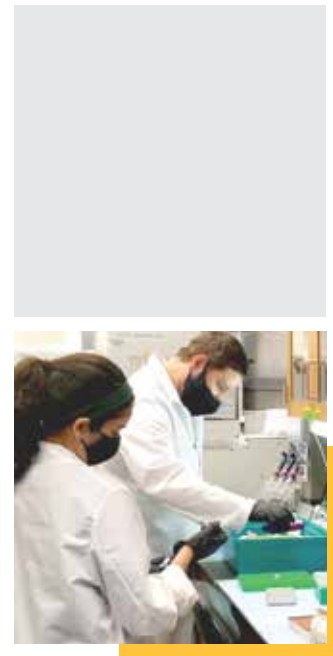
The PhRMA Foundation believes that by better leveraging the power of these promising data-yielding technologies, the health care sector can more effectively increase diversity in clinical trials — which is important to the Foundation’s overall mission of fostering transformative medical research.

With this in mind, the Foundation is developing a new program, aimed at identifying innovative and novel approaches in the use of technology and data resources to aid in understanding and addressing the root causes and complexities of racial and ethnic underrepresentation in clinical trials — and to help, ultimately, increase diversity among trial participants. The new **Technology and Data program** is expected to launch in early 2022.

“If the last year has taught us anything, it is that issues of health equity and racial, ethnic and cultural disparities have a huge impact on health care in the United States,” said PhRMA Foundation President Eileen Cannon. “The medical research sector is no exception, and those of us with roles to play in advancing research must step forward in response.”

By better leveraging technology and data platforms, more effective strategies for increasing diversity in clinical trials can be developed, while at the same time improving the evaluation and documentation of trial benefits and maximizing trial scalability and transportability across diseases and settings. These approaches may have broader application in underserved populations, rare diseases and precision medicine, and they may ultimately help address the universal difficulties in achieving recruitment targets in clinical trials.

“The promising work of the talented scientists the Foundation supports can only be fully realized with a strong and effective clinical testing environment in place — a key step in the drug discovery and drug delivery process,” Cannon said. “We want their discoveries and scientific innovations to have an impact, and clinical trials are often a key destination point as their ideas move forward toward becoming patient therapies. That’s why we believe this new program is well-aligned with, and fundamental to, our overall mission of improving public health.”





In addition to serving the PhRMA Foundation’s mission of improving public health by proactively investing in innovative research, education and value-driven health care, the program also mirrors industry trends and needs: Biopharmaceutical companies are currently making substantial investments in health care information technology, and are focused on capturing and using information in a multitude of ways — informing drug development, learning from post market surveillance, engaging with patients to guide therapies, and, crucially, guiding clinical trials.

Broader acceptance of telehealth and digital health tools during the pandemic has made the conduct of decentralized clinical trials possible. These developments mean that clinical trial-related procedures can be performed at locations remote from the study site — enabling greater opportunities for participation among those who otherwise couldn’t or who found participation inconvenient.

“We believe these developments make our program proposal timely, extremely relevant and useful as the pharmaceutical industry pursues its goals in serving all patients,” Cannon said.

The structure and operational details of the Technology and Data program will continue to be developed during 2021. The Foundation uses a broad array of programming models to achieve its mission and goals — ranging from funding individual grants and targeted research awards to supporting innovation centers — and it is likely to take the same approach with this new program. A variety of structural models are possible, and the Foundation will seek input and ideas from a wide range of stakeholders as it continues conceptual development during the year.

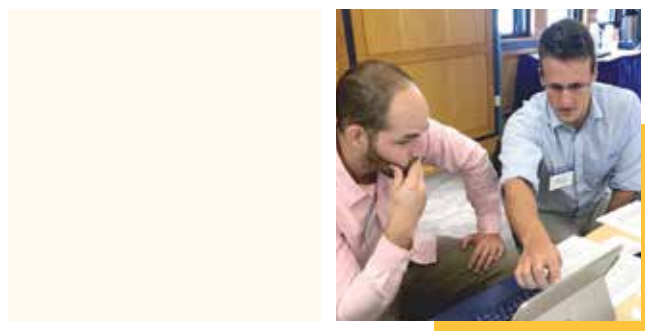
“We expect that the program’s mandate will be to identify, develop, evaluate and implement data- and technology-driven tools to improve or transform research processes,” Cannon said. “The overarching goal will be to reduce barriers, enhance recruitment and retention of more diverse patient populations, and decrease the burden of trial participation and data collection for patients and researchers.”

A wide range of innovative technologies may be useful and play a role in this effort, from passive data collection from sensors and mobile devices to chatbots, telehealth, patient-mapping, analytic and visualization tools and other emerging digital resources.

The Foundation anticipates that conceptual program models for consideration will ideally include collaborations across key stakeholders such as academia, biopharmaceutical industry, data and technology industry, regulators and payers. “It is especially important to partner with community-facing organizations who are already embedded within the underrepresented communities and have invested in building trusted relationships,” Cannon said.

A distinguishing characteristic of the program is that it will focus on vital patient-relevant data in a much broader scope than traditional sources of data and capture new elements of value that are important both for patients and the broader health care system.

“At its core, this program will help us better understand the patient journey with disease and health care through the experience of underrepresented racial, cultural and ethnic populations,” Cannon said. “By leveraging the use of promising new data and technology to aid in this understanding, we believe we can move much faster toward a more effective — and representative — environment for clinical trials.”



Value Assessment 2020 Research Awards

Value assessment in health care comprises a broad set of methods to synthesize and evaluate the relative benefits and costs of health care interventions. The goal of value assessment is to assist stakeholders, including patients, providers and payers, in making informed decisions to improve health and care efficiency. The PhRMA Foundation sought proposals to identify and address challenges in research conducted to assess the value of medicines and health care services. The following researchers were selected in 2020.

“I am truly grateful to receive the 2020 PhRMA Foundation Value Assessment Research Award. This Award has allowed our research team to develop a quality assessment tool that can quantitatively capture methodological and reporting quality for cost-effective analyses (CEA). We hope that our Criteria for Health Economic Quality Evaluation (CHEQUE) Tool can help advance the field’s understanding of the variability of study quality across CEAs and identify the best available economic evidence to inform value-based decisions.”

David Kim, PhD | Tufts Medical Center

CEVR, Institute of Clinical Research and Health Policy Studies



David Kim, PhD

Tufts Medical Center

CEVR, Institute of Clinical Research and Health Policy Studies

“Developing Criteria for Health Economic Quality Evaluation (CHEQUE)”

Since the 1970s, the number of published cost-effectiveness analyses (CEAs) has grown considerably, and CEAs have played an increasingly important role in clinical guidelines and coverage/reimbursement decisions. Multiple guidelines for conducting and reporting CEAs have been developed to promote quality, comparability, and transparency across CEAs. Yet, the absence of a quality assessment tool for CEAs limits the field’s ability to distinguish high- vs. low-quality studies and determine whether the increased information translates into better quality evidence. The Second Panel’s recent report on the future directions for CEA highlighted the need for improvements in quality scoring systems for CEAs to aid decision-makers considering economic evidence.

Our project aims to fill this gap by: 1) identifying key considerations for methodology and reporting standards in CEAs based on a systematic review of multiple guidelines; and 2) creating a quality scoring system to capture both methodological and reporting criteria based on their relative importance. We have undertaken a comprehensive review of current practice and Health Technology Assessment guidelines (e.g., 2nd Panel, CHEERs, ICER, NICE, CADTH) to identify key recommendations for quality assessment. Using the identified quality attributes, we have developed a best-worst scaling survey, and the survey was distributed to researchers and practitioners in the field to estimate the relative importance of these quality attributes. The

survey results will be used to develop a scoring algorithm to estimate quality weights for each methodological and reporting attribute.

Our work will help the field understand the relative importance of different attributes when evaluating the quality of economic evaluations, as well as the variation between methodological and reporting quality. Also, the numerical grading of methodological and reporting quality can help decision-makers in health care identify and use those economic evaluations most worthy of consideration. However, the quality score system is just one way to evaluate the potential usefulness of the study findings. Low-quality CEAs may still provide useful insights, so users of the scoring system should carefully judge each study depending on a set of criteria they believe to be important in evaluating quality.

“Collaborating across the United States and Australia, the PedsUtil will provide an important tool for standardizing pediatric health utility assessment and allow for easier integration of economic quality of life measurement into pediatric clinical trials.”



Lisa A. Prosser, PhD
University of Michigan



Eve Wittenberg, PhD
Harvard T.H. Chan School
of Public Health



Kim Dalziel, PhD
University of Melbourne



Ellen Kim Deluca,
University of Michigan

“Development and Validation of a Preference-Based Index for the PedsQL”

While the past 15 years has yielded key advances in valuing child health, there is still no scoring system to provide health utility scores — the numeric values that represent quality of life for economic evaluations — for children of all ages. The goal of this study is to create a health utility valuation set for the PedsQL. The PedsQL is a commonly-used pediatric quality-of-life instrument with a long tradition of use in clinical trials for pediatric interventions. A health utility scoring algorithm for this instrument will allow for economic endpoints to be estimated directly from the PedsQL without the need for additional resource-intensive data collection, providing an efficient approach to valuing health benefits for pediatric populations. Using national surveys to collect primary data on public values for pediatric health states, the resulting scoring system — the PedsUtil — will represent an important advance for measuring pediatric quality of life. The PedsUtil will ensure opportunities to incorporate health utility scores for children of all ages in health care value assessments, which will help ensure that children’s experiences with disease and treatment are accurately represented. This will allow decision makers to make informed decisions to improve health and care efficiency and address inequities.

“This funding from the PhRMA Foundation will help to shine a necessary light on the importance of capturing the cascade effects of precision medicine diagnostics in family members, thereby strengthening our ability to perform comprehensive high-quality economic evaluation of these technologies in children.”

Wendy J. Ungar, MSc, PhD | Director, Technology Assessment at Sick Kids (TASK),
Hospital for Sick Children, Toronto, Canada



Wendy J. Ungar, MSc, PhD

Director, Technology Assessment at Sick Kids (TASK),
Hospital for Sick Children, Toronto, Canada

“Family Matters: Expanding the economic value paradigm for precision medicine diagnostics to include the costs and health consequences of family members”

In economic evaluation, value is determined by measuring both costs and health consequences in individual patients, preferably over their lifetime. This definition of value is challenged by precision medicine (PM) diagnostics, which are changing the practice of medicine. Genome sequencing (GS) in particular is a powerful means to guide patient management and treatment and dosage selection. Sequencing may be particularly beneficial in children since early diagnosis, risk identification and intervention can result in lifelong benefit. Diagnostic results in children can trigger testing in parents, siblings and second-degree relatives, thereby potentially improving outcomes for many. Extending the scope of benefits beyond the individual patient to include family members enhances the value of PM diagnostics — but this added value is not captured with current methods of cost-effectiveness analysis. A novel paradigm for measuring costs and health consequences that includes patients and family members is required. The first objective involves identifying and understanding the specific challenges of incorporating the costs and consequences of family members into economic evaluation of PM diagnostics. This is being accomplished through literature reviews focused on theory and methods. Second, empirical research has been undertaken to measure family members’ cascade use of services and costs stemming from genetic testing in children with cardiomyopathies. Third, a versatile analytic model to accurately microcost trio genome sequencing (proband plus parents) in pediatric cardiology has been created. The research program funded by the PhRMA Foundation is well aligned with the Foundation’s value assessment priorities by addressing a growing gap in methods for cost-effectiveness analysis of PM diagnostics. Funding and policy decision makers grappling with how to optimize healthcare investment decisions in PM diagnostics will benefit from this work, as will researchers, clinicians and families.

Value Assessment 2020 Challenge Awards

The Challenge Awards program pursues papers that describe solutions to a pressing question related to value assessment in health care. In 2020, the PhRMA Foundation sought papers focused on how patient-centered outcomes can be better incorporated into health care decision making by asking:

What approaches are needed to consistently and reliably incorporate patient-centered outcomes in value assessment for both population- and individual-level health care decision-making?

First Place



Charles E. Phelps, PhD

University Professor and Provost
Emeritus, University of Rochester



Darius N. Lakdawalla, PhD

Quintiles Chair of Pharmaceutical Development and Regulatory Innovation
at the School of Pharmacy and the Sol Price School of Public Policy,
University of Southern California

“Generalized Risk Adjusted Cost Effectiveness (GRACE): Assuring Patient-Centered Outcomes in Health Care Decision Making”

Drs. Phelps and Lakdawalla propose using a novel approach — Generalized Risk-Adjusted Cost-Effectiveness (GRACE) — that aligns the economics of cost-effectiveness analysis with the human circumstances of patients and consider, in particular, how in standard models a given gain in life expectancy is worth less to sicker or more disabled people, because of the approach to weighting life-years gained by “quality of life.” The GRACE model reveals how traditional methods fail to account for disease severity, patient risk-aversion, and other issues and ensures that patient preferences, particularly the quintessential measure of untreated health status, reliably and consistently enter value measures used at both population and individual decision-making.

Second Place



**T. Joseph Mattingly II,
PharmD, MBA, PhD**

Associate Professor and
Vice Chair for Academic
Affairs, Pharmaceutical
Health Services Research,
The University of Maryland
School of Pharmacy



Julia F. Slejko, PhD

Associate Professor of
Pharmaceutical Health
Services Research, The
University of Maryland
School of Pharmacy



**Elisabeth M. Oehrlein,
PhD, MS**

Assistant Vice President,
Research & Programs, The
National Health Council



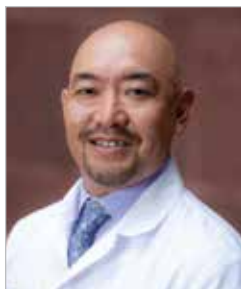
**Eleanor M. Perfetto,
PhD, MS**

Executive Vice President,
Strategic Initiatives, The
National Health Council &
Professor of Pharmaceutical
Health Services Research,
The University of Maryland
School of Pharmacy

“Nudging Health Economists: A Process for Systematic Identification of Patient-Centered Outcomes for Inclusion in Value Assessment”

The authors aim to provide practical guidance on a process for identifying and presenting patient-centered outcomes in a way that makes it easier to include them in value assessment, thereby “nudging” more economists to choose to include these outcomes in their models and not dismiss them simply as limitations. The process includes forming a multi-stakeholder, patient-centered advisory board, engaging the board in the research agenda, conducting evidence synthesis and qualitative research to ensure viewpoints are not missed, and disseminating findings to multi-stakeholder audiences. A publicly available, centralized database of identified patient-centered value elements should be created to increase the likelihood of their uptake in value assessment.

Third Place - Tie



Jonathan H. Watanabe, PharmD, PhD

Associate Dean of Assessment and Quality, Professor of Clinical Pharmacy, University of California Irvine School of Pharmacy & Pharmaceutical Sciences



Derjung M. Tarn, MD, PhD

Professor, University of California Los Angeles School of Medicine



Jan D. Hirsch, PhD

Dean, Professor of Clinical Pharmacy, University of California Irvine School of Pharmacy & Pharmaceutical Sciences

“Evolution of Precision Medicine: Applying a Population-based Evidence Assessment Repository (PEAR) to Achieve Patient-Centered Outcomes at the Point-of-Care”

The authors propose a framework that shapes goals based on patient values and shared decision-making that is continuously refined by utilizing a population-based evidence assessment repository (PEAR) to achieve personalized care. While the framework described will be more easily implemented in an outpatient clinic for chronic disease treatment, components could be applied to inpatient settings, depending on the scenario. As more discussion and information is completed and population-derived, value assessment evidence is applied, the treatment options can be reduced to the most effective for the particular patient. Treatment options tailored towards the patient’s needs would be guided by the clinician’s acumen and the evidence. It would also allow the discussion to proceed based on population-based value endpoints that were then tuned based on the individual’s characteristics and wishes.



Brandy Fureman, PhD

Chief Outcomes Officer, Epilepsy Foundation, Epilepsy Learning Healthcare System

“Co-Production in Learning Healthcare Systems is the Key to Unlocking True Healthcare Value”

The researchers share their experience using co-production — a process where patients, care partners, and providers work together to design a health system that optimizes the health outcomes that matter most to patients. For example, evaluations of value in epilepsy must look beyond standard clinical endpoints of seizure control and address other outcomes that are important to epilepsy patients, such as motor skills, communication, learning, attention and emotional well-being.

FELLOWSHIPS AND GRANTS

Health Outcomes

The first Health Outcomes fellowships and grants were awarded in 2002.

2020 PREDOCTORAL FELLOWSHIPS IN HEALTH OUTCOMES

With a focus on health care and its effects on the well-being of patients and populations, outcomes research provides crucial information to doctors, patients, policymakers, and clinicians. The Foundation's Predoctoral Fellowships in Health Outcomes seek to increase the number of trained investigators studying all aspects of drug therapies by providing a stipend to students two years away from completing doctoral dissertations.

“I am honored and extremely grateful to be an awardee of the PhRMA Foundation Predoctoral Fellowship in Health Outcomes. With the support of this award, I was able to fully concentrate on my dissertation research, present my dissertation findings through publications and academic conferences, and equip myself to become a competitive independent researcher in my future career.”

Chao Li | Auburn University



Chao Li

Auburn University

“Assessment of Risk and Risk Factors of Fluoropyrimidine-induced Cardiotoxicity among Colorectal Cancer Survivors Using a Mixed-Methods Approach”

Although new cancer treatments have been increasingly developed, fluoropyrimidines, such as 5-fluorouracil (5-FU) and capecitabine, are widely used alone or in combination as chemotherapy to treat cancer, especially for colorectal cancer. However, these drugs are associated with serious adverse events like heart damage (cardiotoxicity), which could result in emergency department visits, hospitalizations, and even sudden death. Understanding the risk and risk factors will help physicians with early prediction of fluoropyrimidine-induced cardiotoxicity (FIC) and improvement in patient monitoring. Several attempts to identify potential risk factors for FIC have been made, however, the evidence is not compelling due to a lack of robust data and limitations of traditional methodological approach. In this project, up-to-date published evidence regarding risk factors of FIC will be reviewed and summarized to assess the risk of FIC in broad cancer patients (Aim 1). Then, a large registry-claims linked, population-level data of older colorectal cancer patients will be used to evaluate the risk of FIC by types of chemotherapy treatments (Aim 2). Last, a novel data-analysis approach will be employed to develop new criteria for predicting the adverse cardiotoxicity events among colorectal cancer patients who used fluoropyrimidine treatments (Aim 3). Findings generated from this study will have important implications for identifying cancer subgroups at high risk of FIC, which could help clinicians make better clinical decisions in treatment selection and patient monitoring.

“The PhRMA Foundation Predoctoral Fellowship in Health Outcomes has provided me with the opportunity to immerse myself in my dissertation research. Without the award, I would not have had protected time to pursue additional research training in statistical theory and techniques related to my dissertation. These skills will undoubtedly contribute to my future success as an independent investigator in the field of comparative effectiveness research.”

Carly Rodriguez, MPH | Boston University School of Public Health



Carly Rodriguez, MPH

Boston University School of Public Health

“Comparative Effectiveness and the Safety of Multidrug-Resistant Tuberculosis Treatment”

Approximately 10 million people globally developed tuberculosis disease in 2019. Of these, nearly 500,000 were sick with isolates resistant to at least isoniazid and/or rifampin, referred to as multidrug-resistant TB (MDR-TB). Conventional treatment for MDR-TB is long in duration, often results in debilitating side effects and cures only half of patients. However, after nearly four decades of stagnancy in the TB drug pipeline, several promising drugs and regimens have recently gained regulatory approval by stringent authorities such as the United States Food and Drug Administration. These developments have outpaced the ability for clinical trials in MDR-TB — which can take five to ten years from inception to reporting of results — to produce timely evidence. Global guidance for the treatment of MDR-TB, issued by the World Health Organization, increasingly relies on observational data from patients treated under real-life conditions. Thus, comparative effectiveness and safety research from observational cohorts using appropriate statistical methods is required in order to inform global policy. This research will use observational data from the endTB Project. The observational study of the endTB Project is the largest study cohort of MDR-TB patients receiving treatment with bedaquiline and delamanid to date. Robust causal inference methods will be applied in the data to identify whether adding delamanid to MDR-TB regimens containing a background regimen comprised of three drugs likely to be effective improves culture conversion. Additionally, the optimal adverse event management strategy that maximizes safety and effectiveness for patients receiving linezolid will be evaluated. Lastly, the potential for bias when failing to account for MDR-TB treatment regimen changes will be assessed.

“The PhRMA Foundation’s Predoctoral Fellowship in Health Outcomes research is a great honor for me! It recognized the merit of my PhD dissertation, especially its value to the health system, pharmaceutical industry and research community. It boosted my passion and confidence in pursuing further research and practice opportunities in this field. This award also helps me identify fellow investigators and practitioners in the health outcomes research community.”

Lin Wang, ScM, MD | Johns Hopkins University Bloomberg School of Public Health



Lin Wang, ScM, MD

Johns Hopkins University Bloomberg School of Public Health

“Clinical and Cost-Effectiveness of Advanced Prostate Cancer Treatments”

Prostate cancer is the most common cancer and the second leading cause of cancer death among men in the United States. It puts a tremendous burden on the health system, with 3.7+ million living cases, 248,530 new cases, and 34,130 new deaths expected in 2021. Most prostate cancer deaths were due to metastases. Progress in research has led to several chemohormonal therapies that can delay disease progression. The availability of these therapies has improved prostate cancer survival. However, owing to the lack of head-to-head comparison in clinical trials, little is known about the optimal choice weighing effectiveness and safety. As a result, clinical guideline committees hesitate to recommend one drug over others. Meanwhile, costs vary widely across therapies, ranging from hundreds of U.S. dollars (USD) to hundreds of thousands of USD, for a patient to complete the standard treatment courses. Identifying the value of the therapies is critical to inform clinical practice and insurance policy. This study compared the efficacy, safety, and cost-effectiveness of competing treatments. Bibliographic databases, trial registries, and regulatory documents were systematically searched to identify a comprehensive body of clinical trial evidence. Relative efficacy and safety of treatments were assessed by synthesizing clinical trial data using state-of-art evidence synthesis methods. The cost-effectiveness of treatments was assessed by advanced economic modeling. Besides answering the clinical and economic questions, this study addressed methodological challenges encountered by decision-makers in oncology and rare disease areas. That is, 20% of cancer treatments and most rare disease treatments were approved based on single-arm trials. A lack of head-to-head comparison with other treatment options precludes decision-makers from understanding the relative value of different treatments and investing limited health resources in an efficient way. This study involved a treatment that had only single-arm trial evidence and used novel statistical methods to assess its value relative to other treatments. The findings and the methodology of this study shall have a significant impact on clinical practice, health policy, and health outcomes research.

2020 POSTDOCTORAL FELLOWSHIP IN HEALTH OUTCOMES

“I am very honored and grateful to be a recipient of the PhRMA Foundation’s Postdoctoral Fellowship in Health Outcomes. This award has supported my growth as an early-career investigator by enabling me to pursue research that advances the field of geriatric pharmacoepidemiology, expand my training, and build exciting new research collaborations.”

Shahar Shmuel, PhD | University of North Carolina at Chapel Hill



Shahar Shmuel, PhD

University of North Carolina at Chapel Hill

“Longitudinal Patterns of Anticholinergic and Sedative Drug Load”

The older adult population is growing rapidly, and therefore, improved healthcare management in this population is key. Medication management in older adults is challenging, since aging alters the body’s immune response and capacity to metabolize drugs, leading to an increased sensitivity to intended and unintended drug effects (i.e., side effects). Furthermore, older adults often have multiple chronic health conditions and are prescribed multiple medications to address these health conditions. Taken together, these factors make older adults vulnerable to the use of potentially inappropriate medications, medications whose potential harms may outweigh their benefits, and to dangerous drug-drug interactions. This study was designed to describe individual patterns of use of medications with anticholinergic and sedating properties since use of these medications is associated with increased risk of serious physical (e.g., falls, fractures) and cognitive (e.g., delirium) health outcomes. By helping predict which patients will over time have a high burden of these medications, this work can guide deprescribing interventions for specific high-risk patient groups. The ultimate goal of this work is to promote safe and effective medication use that reduces risk of negative health outcomes and improves older adults’ quality of life.

2020 RESEARCH STARTER GRANTS IN HEALTH OUTCOMES

“I am grateful for the opportunity the PhRMA Foundation Research Starter Grant in Health Outcomes Research provides for me at the outset of my research career. This award allows me to apply my expertise with analyzing large administrative datasets to conduct publishable research for the benefit of patients’ recovery after a cardiovascular event. The award is an appreciated catalyst in reaching my career goal of becoming an independent principal investigator in academic medicine.”

Montika Bush, PhD | University of North Carolina at Chapel Hill



Montika Bush, PhD

University of North Carolina at Chapel Hill

“Maintenance with Guideline Recommended Risk Reduction Therapies after a Heart Attack”

Over 800,000 Americans have a heart attack each year. While most people do not die from their heart attack, they are more likely to have future heart-related health problems. After a heart attack, patients may be prescribed up to four different medications and referred to cardiac rehabilitation (CR) programs to reduce the risk of future heart attacks. CR programs include supervised physical activity, education and counseling. Prior research has described medication use and CR participation separately with summary values within a few months of a patient’s heart attack. A more detailed description of how people start and continue to use these prevention activities jointly in the years after a heart attack is needed. This study will use information from Medicare insurance records to produce unique flow diagrams to create a picture of medication use and CR participation over the two years following a heart attack. Diagrams will also denote when people die or are hospitalized during follow-up. Diagrams for specific groups (e.g., men and women) will also be produced to illustrate differences in heart attack recovery activities. Diagrams from this study will establish a foundation for future research to improve health after a heart attack. This work could also help health care teams offer useful and well-timed treatment adjustments for those who have had a heart attack.

“I am very grateful to the PhRMA Foundation for the opportunity to receive funding through the Research Starter Grant in Health Outcomes. This will allow me to start building my research portfolio as an independent researcher and give me the chance to conduct a meaningful research study. This will build my career by connecting me with research collaborators and opening the door to future research opportunities.”

Natalie Hohmann, PharmD, PhD | Auburn University



Natalie Hohmann, PharmD, PhD

Auburn University

“Psychosocial Factors Affecting Genetic Testing Decisions in Cancer”

About 40% of adults in the United States will be told they have cancer at some point in their lives. But, with improved treatments for cancer, the number of cancer survivors is growing. New and improved cancer treatments include ways to fight a person's cancer with fewer side effects. Using genetic tests, doctors can tell which of these treatments will work best for someone. However, these genetic tests can be expensive, and insurance may not cover all the costs. Also, the results of the tests might not always change which treatment the doctor would recommend. It is also unclear how someone's personal values might affect whether they decide to have the genetic testing done. For example, friends' or family members' opinions, value of hope, peace of mind from getting the test, fear of the test, and what other people have chosen to do, might all play a role in someone's decision to get the genetic test done or not. To help, the goal of this project is to look at which personal values might affect people's decisions to get genetic testing done for cancer treatment, and how these personal values change in different people. First, 80 interviews will be done to ask people about their experiences, beliefs and values that may affect decisions to get genetic testing done for cancer treatment. Interview participants will be recruited from across the United States and be diverse in terms of age, gender, and ethnicity. Second, a survey of 2,400 people from across the United States will be used to look at which personal values are most and least important when making this genetic testing decision. The survey results will reveal how different people “trade-off” between different personal values and testing cost when they make this testing decision. The results of this study can be used to design educational materials to help people think through their personal values when they are faced with making the decision about getting genetic testing for cancer treatment. This can help people to be prepared when talking with their doctor about what matters most to them when making these important testing and treatment decisions and can help people to feel more confident and satisfied with the decisions that are made. In the end, these better decisions can lead to better cancer treatment results and better quality of life for people receiving cancer treatment.

Translational Medicine

The first Translational Medicine fellowships and grants were awarded in 2013.

2020 POSTDOCTORAL FELLOWSHIPS IN TRANSLATIONAL MEDICINE

The PhRMA Foundation Postdoctoral Program in Translational Medicine provides stipend support for individuals engaged in multidisciplinary/collaborative research training programs that will create or extend their credentials in this evolving area. The intent of this program is to support postdoctoral career development activities of individuals preparing to engage in research that will bridge the gap between discoveries using experimental and computational technologies and in the research laboratory and their application in clinical research and the clinic. A key component of postdoctoral training in this area involves collaborative programs that span the non-clinical and clinical domains, potentially involving multiple laboratories, advisers and/or institutions.

“The PhRMA Foundation’s support has been crucial to my career as a physician scientist. Receiving the Postdoctoral Fellowship in Translational Medicine allowed me to launch an independent research career dedicated to improving treatments for cancer. I am honored and grateful for the Foundation’s support.”

Sylvan Baca, MD, PhD | Dana-Farber Cancer Institute



Sylvan Baca, MD, PhD

Dana-Farber Cancer Institute

“Detecting Mutational Signatures from Circulating Prostate Cancer DNA”

Prostate cancer claims the lives of roughly 33,000 men in the United States every year. PARP inhibitors (PARPis) and immune checkpoint inhibitors (ICIs) are promising treatments for prostate cancer. These medicines can be very effective against certain prostate cancers that cannot properly repair damage to their DNA. Unfortunately, to determine if these medicines are likely to help a given patient, it is often necessary to biopsy prostate cancer that has spread to the bones. These biopsies can be technically challenging and uncomfortable for patients. To address these difficulties, this project will develop a blood test to predict whether a patient’s prostate cancer is likely to respond to PARPis and ICIs. It will take advantage of a promising new technology that can detect trace amounts of cancer DNA in the blood. By looking for specific patterns of mutations in cancer DNA present in blood, the test aims to identify cancers with defective DNA repair that may respond to treatment with PARPis or ICIs. This advance could identify thousands of patients who could benefit from PARPis or ICIs, while sparing those who will not respond from treatment side effects.

“I am very grateful to the PhRMA Foundation for supporting my career goal to work as a tissue engineer to improve treatments for patients. With this award, I will have the opportunity to develop new therapeutic solutions to overcome organ transplantation rejection by designing 3-D printed bioengineered organs that can evade rejection. I am very excited to make a real contribution to public health that will eventually lead to significant advances in medical practice.”

Tânia Baltazar, PhD | Yale University

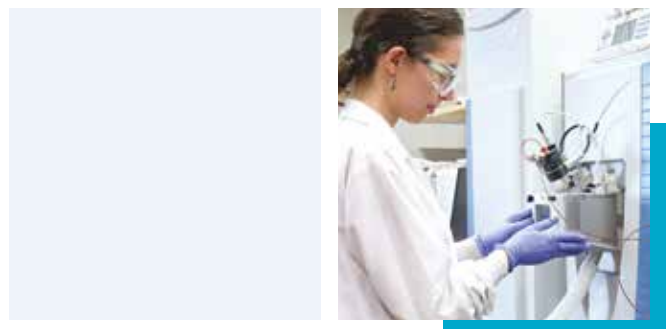


Tânia Baltazar, PhD

Yale University

“Human Immune Recognition of 3-D Printed Vascularized Skin Grafts”

Organ transplantation is the most effective therapy for end-stage organ failure but its use is limited by a lack of available organs. 3-D bioprinting technology has the potential to solve this problem by creating tissue-engineered organs. Successful engraftment will require the incorporation of perfused micro-vessel networks to provide nutrients and oxygen to these organs. Human endothelial cells (ECs) from different donor sources to the graft recipient can be used to successfully generate 3-D bio-printed micro-vessels but are capable of initiating rejection after transplantation. Using 3-D printed skin as a model tissue, this project will determine if ECs that have been genetically modified to evade the host immune system can still provide perfusion without initiating rejection. The study proposes to first determine if a model human-engineered tissue (3-D printed skin) containing a human microvasculature that can engraft and become perfused when implanted on an immunodeficient mouse host will be rejected by elements of human immune system (T cells and/or antibody) from another donor. Moreover, the project will investigate if genetically modifications of human ECs that eliminate the ability to activate the human immune system can be used to create an “off-the-shelf” 3-D printed skin graft that evades rejection.



2020 RESEARCH STARTER GRANTS IN TRANSLATIONAL MEDICINE

The Research Starter Grant Program in Translational Medicine aims to support individuals beginning independent research careers in academia or research institutions at the faculty level and where long-term training of students and/or scientists is an expected outcome in conjunction with their research. This program focuses on supporting the career development of scientists engaged in bridging research and discoveries using experimental and computational technologies to their application in clinical research and the clinic. The program is not focused on supporting the application of standard technologies to experimental biology or medicine but specifically to explore innovative and collaborative projects that bridge the non-clinical: clinical interface.

“I am very grateful and honored by this support from the PhRMA Foundation. It is enabling me to perform critical work that will lead to improved clinical outcomes for Chagas disease patients. On a more personal note, this support has been essential to establish my independent research career and develop my research group.”

Laura-Isobel McCall, PhD | University of Oklahoma



Laura-Isobel McCall, PhD
University of Oklahoma

“Meeting the Translational Need for Biomarkers of Chagas Disease Cure”

Trypanosoma cruzi parasites cause Chagas disease, a major form of heart disease. In the United States, at least 300,000 people are infected with *T. cruzi*. Worldwide, over 5 million people are infected. Treating Chagas disease is difficult. Existing drugs cause serious side effects, and more than 20% of patients who take these drugs are still not successfully cured of their infection. Unfortunately, there is no current method to quickly test whether a patient was cured or not. Existing tests either require over ten years of patient follow-up or are unable to pick up all the failed treatments. This is a problem for patient treatment, and for clinical trials to evaluate new drugs for Chagas disease. Clinicians have stated that identifying new indicators (“biomarkers”) of successful treatment is a key priority for the Chagas disease research field. The goal of this proposal is to meet this clinical need by proving that small molecules can be used to determine whether a patient was successfully cured or not. Overall, this project’s results will have a major translational impact on how patients are monitored and on how clinical trials for new Chagas disease drugs are performed.

“I am grateful and honored to have received the PhRMA Foundation Research Starter Grant in Translational Medicine. My laboratory focuses on enteric infections and vaccines, and one of our goals is to improve oral vaccine responses in low- and middle-income countries with high enteric infectious disease burden. This grant helps us establish a pre-clinical pipeline involving organoid cultures and animal models that will allow translation of laboratory findings into interventions that improve child health.”

Sasirekha Ramani, PhD | Baylor College of Medicine



Sasirekha Ramani, PhD

Baylor College of Medicine

“Harnessing the Potential of the Microbiome to Improve Rotavirus Vaccine Response”

Rotavirus is a leading cause of life-threatening diarrhea and vomiting in children under the age of 5 years. Live, attenuated vaccines to prevent severe rotavirus disease were first licensed in 2006 and are now used in over 100 countries. Since the introduction of vaccines, the number of rotavirus associated deaths has significantly decreased in high-income countries. However, the same vaccines are far less effective in low- and middle-income countries, where millions of diarrheal hospitalizations due to rotavirus continue to occur each year. It is important to develop targeted interventions that will improve the response to rotavirus vaccines in these settings. This project focuses on developing interventions based on gut microbiome of vaccine responders. The intestinal microbiome is an important player in human health and disease. Studies in some low-income countries have shown that the pre-vaccination microbiome of rotavirus vaccine responders is distinct from that of non-responders within the same population. The goal of this project is to identify and test factors produced by “responder bacteria” for their effects on improving rotavirus vaccine response. These evidence-based microbial interventions will first be evaluated for safety and effectiveness in biologically-relevant laboratory model systems. The long-term goal of this work is to translate these microbiome-based interventions into products that can be co-administered with vaccines in order to improve immune responses in children.

Pharmacology/Toxicology

The first Pharmacology/Toxicology fellowships and grants were awarded in 1978 with predoctoral fellowships.

2020 PREDOCTORAL FELLOWSHIPS IN PHARMACOLOGY/TOXICOLOGY

Predoctoral Fellowships in Pharmacology/Toxicology have helped expand the nation's pool of highly trained pharmaceutical researchers. The PhRMA Foundation provides awardees with a two-year stipend as they move toward completion of their research for pharmacology and toxicology doctoral dissertations.

“It is a privilege to be a recipient of the Predoctoral Fellowship in Pharmacology/Toxicology from the PhRMA Foundation. This funding provided the opportunity for me to test novel therapies for metabolic diseases and has been integral in helping me establish a track-record of funding. I’m extremely grateful for this award!”

Joshua Barton | Thomas Jefferson University



Joshua Barton

Thomas Jefferson University

“Silencing the Uroguanylin-GUCY2C Gut-Brain Axis Mediates Leptin Resistance in Obesity”

Leptin is a potent anorexigenic hormone and compelling candidate to treat diet-induced obesity (DIO). However, DIO induces leptin resistance by downregulating leptin receptors (LepRs) on hypothalamic neurons, rendering leptin therapy ineffective. Overcoming leptin resistance has become a promising goal for obesity therapy. This project discovered that the guanylyl cyclase C (GUCY2C) receptor is specifically expressed in hypothalamic LepR(+) neurons. Hypothalamic GUCY2C induces satiety, but is silenced in DIO by loss of its hormone, uroguanylin, produced in the intestine. Importantly, reconstitution of GUCY2C signaling induces weight loss and leptin sensitivity in DIO mice. These data suggest that loss of uroguanylin in obesity silences hypothalamic GUCY2C, downregulating LepRs on neurons, while reconstituting GUCY2C signaling by ligand replacement should reverse downregulation of LepRs, overcoming leptin resistance. Here, the pathophysiologic aim will demonstrate that the uroguanylin-GUCY2C axis regulates leptin sensitivity. The mechanistic aim will establish that GUCY2C signaling regulates neuronal surface LepRs. Finally, the therapeutic aim will define the ability of GUCY2C ligands to overcome leptin resistance in DIO mice. These studies will reveal new mechanisms in obesity and novel solutions to overcome leptin resistance. The potential to translate these results into patients is underscored by approval of GUCY2C ligands to treat constipation.

“It is a great honor to have been awarded this fellowship from the PhRMA Foundation. With it I have been able to pursue several novel experiments that not only provide valuable research data but have also been instrumental in allowing me to grow as a scientist. Furthermore, these successes are instrumental in the continual pursuit of my goal of becoming an independent investigator leading a translational research laboratory.”

Yu-Chun (Roy) Chang | The Ohio State University College of Medicine,
Center for Regenerative Medicine at Nationwide Children's Hospital



Yu-Chun (Roy) Chang

The Ohio State University College of Medicine, Center for Regenerative Medicine at Nationwide Children's Hospital

“Controlling Tissue Engineered Vascular Graft Development with Bisphosphonates”

The tissue-engineering revolution has brought about several significant advancements in the field of congenital heart disease. In the treatment of hypoplastic left heart syndrome, surgical intervention is required to redirect blood flow in an infant. Current approaches utilize a synthetic vascular graft, one that is unable to grow with the child and prone to complications, such as infection and calcification. Over the past 15 years, the Breuer group has created and improved upon tissue-engineered vascular grafts (TEVGs), conduits that result in the formation of near autologous vessels; however, as it stands, the major barrier to the widespread adoption of these grafts is that of excessive tissue growth leading to narrowing of the conduit (i.e. stenosis). Numerous studies have since shown that TEVG development is largely mediated by host monocyte and macrophage response. These immune cells infiltrate the TEVGs and induce a signaling cascade that leads to development of new tissue. It is when this process is taken to the extreme that results in narrowing of the vessel. Thus, the key is to balance the degree of macrophage response to ensure proper graft development, while mitigating the creation of stenosis. This project seeks to utilize an already FDA-approved class of drugs to achieve this balance and allow for more rapid adoption of TEVGs.

“I am honored and humbled to be a recipient of the PhRMA Foundation Predoctoral Fellowship in Pharmacology/Toxicology. This award has allowed me the opportunity to broaden the information we have on ENDS products and contribute to the knowledge we have on the effects that ENDS flavors have on nicotine addiction. Furthermore, this award has provided me with the resources, networking connections, and travel opportunities to succeed as a research-scientist.”

Skylar Cooper | Marshall University, Joan C. Edwards School of Medicine

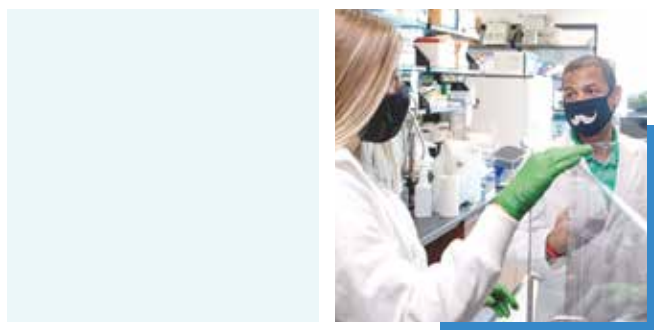


Skylar Cooper

Marshall University, Joan C. Edwards School of Medicine

“Testing Electronic Nicotine Delivery System Flavors for Their Ability to Enhance Nicotine Addiction”

Over the past two decades, combustible cigarette smoking has decreased by approximately 11% in the United States. However, the use of electronic nicotine delivery systems (ENDS) has increased by 78% among high school students in the past year alone. Although ENDS products were initially intended to be a smoking cessation aid for lifelong smokers, these products have become a new craze among the adolescent population for the popularity of flavored products. Currently there are over 15,000 ENDS flavors to choose from and many users prefer zero-nicotine flavored e-liquids. With the current knowledge on the effects adolescent nicotine exposure has on the developing brain and recent findings that ENDS flavors may enhance nicotine's actions while exhibiting addictive properties on their own, it is important to determine the pharmacology of ENDS flavorants on nicotinic acetylcholine receptors (nAChRs) in the addiction-related circuitry of the brain (midbrain). The goal of this research is to investigate the pharmacology of ENDS flavors on nAChRs to further understand the pharmacological changes that trigger enhancements in nicotine addiction.”



“Receiving a Predoctoral Fellowship in Pharmacology/Toxicology from the PhRMA Foundation is a great honor that has had a profound impact on my current and future career prospects. This funding has allowed me to pursue novel hypotheses that can expand the current knowledge surrounding both ferroptosis and Huntington’s disease and could have a profound impact on individuals with Huntington’s disease. I am sincerely grateful to the PhRMA Foundation for its support in these efforts.”

Jacob Daniels | Columbia University



Jacob Daniels

Columbia University

“Evaluation of Ferroptosis Inhibitors as Therapeutic Agents for Huntington’s Disease”

Huntington’s disease (HD) is an autosomal neurodegenerative disease caused by the expansion of CAG repeats in the Huntingtin gene, resulting in the production of pathogenic protein with an extended polyglutamine tract that is prone to aggregation. Current therapeutic options for HD are limited, focusing solely on symptom management, with the anti-choreic drugs tetrabenazine and deutetrabenazine the only FDA-approved therapeutics for HD. As such, there is an unmet demand for therapeutics for HD. Ferroptosis is an iron-dependent, non-apoptotic, regulated form of cell death characterized by the lethal accumulation of lipid peroxides and lipid peroxide byproducts. HD is associated with glutamate toxicity and neurotoxicity, dysregulated polyunsaturated fatty acid metabolism, elevated iron, and lipid peroxidation, all of which are drivers of ferroptosis. The goal of the present study was to evaluate and screen novel ferroptosis-specific inhibitors as therapeutic agents in a mouse model of Huntington’s disease. These efforts were directed into two aims: the first, evaluating the mechanism by which the cardiovascular drug probucol likely inhibits ferroptosis; and second, testing analogs of the canonical ferroptosis inhibitor ferrostatin-1 (fer-1) in vitro and in vivo for their pharmacokinetic and pharmacodynamic profiles. This research has identified a potential new mechanism by which probucol inhibits ferroptosis that is independent of fer-1. Ongoing efforts have been focused on elucidating the components of the inhibitory pathway, providing a potential new area of therapeutic development for ferroptosis inhibitors. This research has also identified new, potent analogs of fer-1 that are stable in vivo and preferentially accumulate in the brain of mice. These results lay the groundwork to evaluate the efficacy of ferroptosis inhibitors in mouse models of Huntington’s disease to determine whether ferroptosis inhibition is a therapeutic strategy for HD.

“I am honored to receive the PhRMA Foundation Predoctoral Fellowship in Pharmacology/ Toxicology. It has given me a unique opportunity to showcase my research through publications and presentations and boost my confidence to pursue my career as an independent researcher. I express my gratitude to the PhRMA Foundation for supporting international graduate students like me during their early scientific careers.”

Priyanka Das Pinky, MBBS | University of California, Irvine
Institute for Memory Impairment and Neurological Disorders (MIND)



Priyanka Das Pinky, MBBS

University of California, Irvine

Institute for Memory Impairment and Neurological Disorders (MIND)

“Mechanism of Prenatal Cannabinoid Exposure Mediated Learning and Memory Deficits and Identification of Therapeutic Target”

Cannabis is currently the most common illicitly used drug during pregnancy. Although public opinion is that marijuana is safe to use, cannabinoids can easily cross the placenta and reach the fetal brain, affecting its neural growth and development. These effects are long-lasting and can influence various aspects of adult behavior, including learning and memory, anxiety, attention span, etc. However, there are currently no therapeutic options to treat the cognitive deficits associated with prenatal cannabinoid exposure. The goal of this study was to elucidate the mechanism by which prenatal cannabinoid exposure causes learning and memory deficits in rodent offspring and to identify therapeutic targets that can correct memory deficits. In this study, rats were treated throughout pregnancy with a synthetic cannabinoid. The offspring were then examined during their adolescence by performing various behavioral experiments, i.e., Morris water maze, Y maze, and Contextual fear conditioning, and were found to have memory deficits. Electrophysiological recording on the brain slice revealed alterations in synaptic plasticity within the hippocampus, a key area for learning and memory, that likely contributed to the observed memory deficits. Synaptic plasticity, which gives an idea about memory formation, can be defined as the ability of connections between neurons to strengthen or weaken in response to neuronal activity. Long-term potentiation (LTP) and long-term depression (LTD) are two important parameters to measure synaptic plasticity and both were impaired in these offspring. An alteration in the hippocampal glutamatergic neurotransmission has also been observed. Glutamate is the major excitatory neurotransmitter in the brain and is important for facilitating learning and formation and maintenance of memory. A major modulator of glutamatergic neurotransmission is the neural cell adhesion molecule (NCAM) that helps in neurite outgrowth, neural pathfinding, and neurogenesis. NCAM and its active form Polysialylated NCAM both were reduced in the hippocampus of prenatally cannabinoid exposed offspring. Most importantly, preliminary studies indicated that modulation of PSA-NCAM could restore the observed synaptic plasticity deficits in prenatally cannabinoid exposed offspring. The data from this study not only points toward a specific mechanism responsible for prenatal cannabinoid mediated deficits but will also comprehensively assess the different roles played by synaptic molecules responsible for plasticity mechanisms closely associated with cognition. Moreover, this will serve as an excellent premise for future interventions to restore memory functioning in individuals exposed to cannabinoids.

“Receiving a PhRMA Foundation Predoctoral Fellowship in Pharmacology/Toxicology is an honor and it supports my research as a graduate student. I am lucky to have the chance to partake in research that could improve the health and well-being of many people. This fellowship helps fuel my desire to continue such research and advance toward my future career.”

Eric Mosher | Johns Hopkins University School of Medicine



Eric Mosher

Johns Hopkins University School of Medicine

“Muscle-type Creatine Kinase as a Regulator of Tenofovir Disposition”

One of the newer approaches to decreasing the spread of HIV is pre-exposure prophylaxis (PrEP), wherein people can take a pill daily and decrease the chance they become infected with HIV. Tenofovir (TFV), a key component of FDA-approved PrEP regimens, is dosed as an inactive prodrug that must undergo multiple chemical reactions inside the body before it is active and effective. In colon tissue, a key site related to HIV infection, the last of these activating reactions is performed by muscle-type creatine kinase (CKM), producing the active TFV-diphosphate (TFV-DP) metabolite. While TFV-based PrEP has been shown in clinical trial and real-world settings to reduce the chance of HIV infection, it has not been 100% effective; and lack of efficacy results in lifelong infection. The goal of this research project is to study how person-to-person variation in the ability of one's body to activate TFV may contribute to these variable outcomes. Thus, assessment of the impact of genetics and age on CKM and its role in TFV activation and distribution is being performed. The effects of naturally-occurring mutations to CKM on its structure, endogenous activities, and ability to form TFV-DP in vitro are being evaluated. Mouse models will enable comparisons of how TFV-DP and CKM levels may change as a function of age. The impact of age on TFV-DP and CKM distributions in colon tissue will be studied using mass spectrometry imaging. These studies will inform us about the role of CKM in the inter-individual variability in PrEP outcomes and potentially create a path toward more personalized PrEP prescribing.

“I am a first-generation scientist in my family and I am a woman in science. This motivates me to pave my own path and be confident that I have the ability to inspire others through my hard work. The PhRMA Foundation’s Predoctoral Fellowship allowed me to focus solely on research to achieve my career goals and bolstered my confidence in my ability to grow as a scientist.”

Bomina Park | Indiana University School of Medicine



Bomina Park

Indiana University School of Medicine

“Targeting Soluble Epoxide Hydrolase to Treat Pathological Angiogenesis in Age-Related Macular Degeneration”

Neovascular “wet” age-related macular degeneration (wet AMD) is a leading cause of blindness among the elderly, affecting millions of people worldwide. Choroidal neovascularization (CNV) is a major pathological feature of wet AMD, in which abnormal new blood-vessel growth from the choroid leads to hemorrhage, detachment of retina and irreversible loss of vision. Today, the effort to treat wet AMD is hampered by resistance and refractory responses to the current standard of anti-angiogenic care, anti-vascular endothelial growth factor therapies. Thus, there is a critical need to advance our understanding of mediators involved in CNV pathophysiology and develop novel therapeutic strategies. One such target is soluble epoxide hydrolase (sEH), which is a key enzyme that hydrolyzes epoxy fatty acids into diols in the polyunsaturated fatty acid (PUFA) metabolic pathway. This project identified sEH as a target of an antiangiogenic homoisoflavonoid, SH-11037. sEH expression and activity are upregulated in the eyes of a CNV mouse model and sEH is overexpressed in human wet AMD eyes, while pharmacological inhibition of sEH using small molecule inhibitors delivered intraocularly suppressed CNV, suggesting strong relevance of sEH in CNV. However, challenges exist because the cellular localization of sEH in the retina and how these sEH-dependent lipid molecules signal under CNV remain poorly understood. To address these questions, this project defined localization and cellular origin of sEH through immunohistochemical and RNAscope in situ hybridization analysis in human AMD and murine laser induced CNV (L-CNV) retinas. To test the hypothesis that sEH in the eye is required for CNV formation in vivo, local sEH knockdown in the retina was achieved through intraocular delivery of adeno associated virus mediated shRNA targeting Ephx2 and followed by lipid profiling and gene expression analysis. In addition, sex differences in ocular sEH expression and sex differential pharmacological response to sEH inhibitors will be examined. The effect of tissue specific knockdown of sEH on CNV, which had not been examined before, will provide additional evidence validating sEH as a key player of CNV.

“Receiving a PhRMA Foundation Predoctoral Fellowship in Pharmacology/Toxicology is a great honor. In addition to allowing me to pursue my research project on anticancer drug delivery to the brain, this fellowship has helped propel my research career forward and made me more competitive in the job market. I am very grateful for this opportunity. Thank you, PhRMA Foundation, for supporting early-career scientists, particularly graduate students like me, launching their careers!”

Julia A. Schulz | University of Kentucky, College of Pharmacy



Julia A. Schulz

University of Kentucky, College of Pharmacy

“Dual PI3K/Akt Inhibition to Overcome Blood-Brain Barrier P-glycoprotein and Breast Cancer Resistance Protein: A New Strategy to Improve Glioblastoma Therapy”

Glioblastoma is the most common and most devastating human brain cancer. On average, patients survive for only one year after diagnosis. Therefore, glioblastoma is also called “the terminator.” One reason for the treatment failure of anticancer drugs is the blood-brain barrier that protects the brain by impeding xenobiotic uptake from the blood. To this end, efflux transporters at the blood-brain barrier, such as P-glycoprotein and Breast Cancer Resistance Protein, prevent a myriad of compounds, including anticancer drugs, from entering the brain. Thus far, approaches to deliver anticancer drugs across the blood-brain barrier have been unsuccessful in clinical trials. Therefore, novel therapeutic strategies to overcome the blood-brain barrier to improve glioblastoma treatment are urgently needed. This project hypothesizes that downregulating the efflux transporters P-glycoprotein and Breast Cancer Resistance Protein at the blood-brain barrier through dual PI3K/Akt inhibition opens a “window-in-time” that allows anticancer drugs to enter the brain. This research will determine the mechanism through which PI3K/Akt regulates efflux transporters at the blood-brain barrier. Additionally, this therapeutic approach will be tested in a mouse glioblastoma model. It is expected that PI3K/Akt inhibition will lead to increased anticancer drug brain levels, which will result in reduced tumor size and prolonged survival in a mouse glioblastoma model. This study will serve as a first step toward developing a new therapeutic strategy for glioblastoma and is expected to positively impact the treatment, survival and well-being of glioblastoma patients.

2020 POSTDOCTORAL FELLOWSHIPS IN PHARMACOLOGY/TOXICOLOGY

The PhRMA Foundation has been awarding Postdoctoral Fellowships in Pharmacology/Toxicology since 2002. This fellowship provides a two-year stipend to scientists who seek to gain new skills in areas relevant to pharmacology. Eligible candidates are actively pursuing a multidisciplinary research training program to enhance their expertise and education, or embarking on a research project that aims to integrate information on a drug's molecular or cellular mechanisms of action with the agent's effects on the intact organism.

“I am very grateful for the support of the PhRMA Foundation for my Postdoctoral Fellowship in Pharmacology/Toxicology. The support has given me the opportunity to pursue my passion in research and work towards my goal of becoming an independent investigator in academia.”

Christian L. Egly, PharmD | Vanderbilt University Medical Center



Christian L. Egly, PharmD

Vanderbilt University Medical Center

“A High-throughput, Fluorescence Assay to Identify Drugs that Rescue Trafficking-Deficient KV11.1 Variants”

The human Ether-a-go-go-Related Gene (hERG) encodes one of the major repolarizing potassium channels in the heart (KV11.1). Pathogenic variants of the channel are responsible for causing long QT syndrome subtype 2 (LQT2), a disorder that prolongs ventricular contraction and action potential duration. KV11.1 variants are the second leading cause of congenital long QT syndrome and comprise nearly 30% of clinical cases. Severe variants in KV11.1 can predispose individuals to a fatal ventricular arrhythmia, torsades de pointes, so restoring the function of the channel has clinical importance. Currently, the only treatment modalities for LQT2 include beta-blockers or implantable-cardiac defibrillators. Previous reports estimate that ~90% of KV11.1 variants are trafficking deficient and studies showed that treatment with E4031, a KV11.1 specific inhibitor, restored the trafficking for some channel variants. Remarkably, after washout the drug functionally restored KV11.1 related current (IKr) and led to the discovery that KV11.1 channel variants can be chaperoned by drug molecules to the plasma membrane. Unfortunately, to date there are no new drug molecules with a clinical indication to rescue KV11.1 trafficking deficient variants, so there remains a need for high-throughput assays. Thallium (Tl⁺) flux is a standard screening tool for monovalent ion channels used widely in industry and academia. This research utilizes the high-throughput, thallium-based, fluorescent assay to identify drugs that rescue KV11.1 trafficking after 24-hour drug exposure. The results from screening close to 1200 FDA-approved drugs are promising. The goal is to identify novel drugs and drug targets for trafficking KV11.1 variants. There is possibility of transferring knowledge from KV11.1 trafficking to other ion channel disorders.

“The PhRMA Foundation Postdoctoral Fellowship has allowed me to ask, test, and answer novel questions that are fundamental to how man-made environmental toxicants cause heritable diseases. This work would not have been possible without the support of the PhRMA Foundation and I am honored to have been selected for this fellowship.”

Ross Gillette, PhD | The University of Texas at Austin



Ross Gillette, PhD

The University of Texas at Austin

“Environmental Contaminants and Their Role on Heritable Disease and Traumatic Stress”

Man-made environmental toxicants have permanently and irreversibly contaminated the environment. Certain chemicals created for the industrial and agricultural industries are classified as endocrine disrupting chemicals (EDCs) and cause a broad range of hormone-sensitive diseases like cancer, reproductive abnormalities, and behavioral disorders. Two ubiquitous EDCs, polychlorinated biphenyls (PCBs - industrial) and Vinclozolin (agricultural), cause heritable disease that is not due to DNA mutation. The mechanisms that allow EDCs to cause heritable disease are not fully understood and it is unclear why such a mechanism exists. Traumatic stress is the only other life event known to be heritable and is suspected to act through a conserved evolutionary mechanism meant to provide future generations with information essential for survival. The novel hypothesis proposed here is that EDCs hijack or interact with the same mechanisms by which traumatic stress informs future generations. To test this hypothesis, male rats will be exposed to either PCBs or Vinclozolin from fetal development to adulthood and then exposed to traumatic stress paired with a strong odor. If heritable, their sons, who never experienced EDCs or traumatic stress, are predicted to display a fear response when presented with the same odor, an effect that is predicted to diminish due to EDC exposure. The brain, blood, and sperm of rat fathers and sons will be used to isolate extracellular vesicles (EVs), which are cellular components that contain material believed to be involved in the transfer of information across generation. The EV contents are used for gene sequencing, and advanced bioinformatics will be used to identify the components that are necessary for the heritability of experience. The goal of this work is to determine the mechanisms that allow heritable disease to be passed between generations and to identify potential targets for future therapeutic intervention and mitigation.

2020 RESEARCH STARTER GRANT IN PHARMACOLOGY/TOXICOLOGY

For faculty members without sufficient sources of funding, the PhRMA Foundation offers the Research Starter Grant: A one-year award to help launch independent research careers. This grant aims to assist academic scientists in pursuing studies that shed light on individualized drug therapy and optimal therapeutic options.

“This Research Starter Grant from the PhRMA Foundation has kickstarted my independent laboratory into a new avenue of research in evaluating new drug targets for pain. It has allowed us to collect preliminary data for an NIH proposal that will hopefully be funded soon. I am very grateful for these funds from the PhRMA Foundation and excited to see how this research unfolds.”

Erin N. Bobeck, PhD | Utah State University



Erin N. Bobeck, PhD

Utah State University

“Evaluation of a New Receptor, GPR171, as a Pain Therapeutic”

Opioid prescriptions for pain in the United States have skyrocketed over recent years, which has contributed to the opioid epidemic. The need for better pain therapeutics that have reduced abuse liability is essential in ending this crisis. Orphan G-protein coupled receptors could be a new avenue to investigate for the treatment of pain and a variety of other health concerns. One interesting candidate is GPR171, which recently was found to be the receptor of the highly abundant neuropeptide, BigLEN. This research is investigating the neural circuitry of this system, BigLEN-GPR171, and its ability to alleviate chronic pain. Preliminary data show that a GPR171 agonist has antinociceptive properties when combined with an opioid or in inflammatory pain. In addition, inflammation alters GPR171 expression within a key brain region, periaqueductal gray, that is involved in pain modulation. The ongoing research funded by the PhRMA Foundation uses molecular and behavioral pharmacology to investigate the long-term effects of a GPR171 agonist on inflammatory pain. In addition, the project is deciphering which brain areas are mediating these responses. Overall, this innovative research has important pain therapeutic implications, as it will enhance our understanding of the physiological functions of this novel neuropeptide system.

Informatics

The PhRMA Foundation has been awarding fellowships and grants in Informatics since 2002.

2020 PREDOCTORAL FELLOWSHIPS IN INFORMATICS

At universities throughout the country, students are researching new avenues to find, process, and translate information about human health and disease. Their work stands to advance the state of the art in informatics, improving pharmaceutical research and patient care. To support these bright young scientists as they integrate information technology with biological, chemical, and pharmacological sciences, the Foundation provides an annual stipend for up to two years.

“The PhRMA Foundation Informatics Fellowship provided the financial support for me to pursue an informatics research trajectory, which was entirely new for my lab. I was able to develop new software to probe the biology of our prior bench-work-based findings at a broader and higher-throughput scale.”

Veronica F. Busa | Johns Hopkins University School of Medicine



Veronica F. Busa

Johns Hopkins University School of Medicine

“nearBynding: Detecting RNA Structure Proximal to Protein Binding”

RNA-binding proteins (RBPs) play diverse, important roles in cellular processes and regulation. One RBP can bind and affect hundreds of RNAs and cause a cascade effect across the transcriptome. Understanding RBP binding specificity and function is therefore central in understanding human biology and diseases. RBP binding relies on the sequence and folded structure of the RNA. There are many tools to study sequence-based RBP binding preferences, but few for identifying structure-based RBP binding preferences. nearBynding is a pipeline that incorporates RBP binding sites and RNA structure information to discern local RNA structure for regions bound by an RBP. The motivation for this pipeline is three-fold: to visualize RNA structure at and proximal to RBP binding transcriptome-wide; to provide a flexible scaffold to study RBP binding preferences relative to diverse RNA structure data types; and to allow for RBP binding data analysis without extensive pre-processing. nearBynding extends the algorithm StereoGene to allow for direct estimation of correlation among pairs of continuous or interval features along the transcriptome, such as RNA structure and RBP binding. Using both sequence-based RNA structure predictions and experimental RNA annotations, nearBynding can recapitulate published RBP structural binding preferences and observe new binding profiles. nearBynding is efficient enough to run on a personal computer and is available as an easy-to-use R package on the biology software database Bioconductor.

“The PhRMA Foundation Predoctoral Fellowship in Informatics is providing me the resources necessary to pursue my research goals in pharmacomicrobiomics. With the help of this fellowship, I am growing as an independent researcher and deepening our understanding of the microbiome’s contribution to interindividual variation in drug response.”

Annamarie Bustion | University of California, San Francisco



Annamarie Bustion

University of California, San Francisco

“Identifying Bacterial Enzymes Responsible for Drug Metabolism in the Human Gut Microbiome”

Pharmacogenomics research revealed that host genetic variants lead to interindividual variation in drug response. From research in the recently minted — yet nearly century-old — field of pharmacomicrobiomics, however, we now know that the bacteria residing within human hosts also affect a therapeutic’s pharmacokinetics. So far, over 200 FDA-approved compounds are known to be altered when exposed to the human gut microbiome, but only thirty of these reactions have been linked to a characterized gut-associated bacterial enzyme. As high-throughput experimental research continues, this imbalance between drugs identified relative to their metabolite and enzyme characterizations will likely widen. This project narrows that gap by employing chemoinformatic and metagenomic methods to computationally predict the bacterial enzymes responsible for previously reported gut microbiome drug metabolism events. Via reaction fingerprinting and chemical similarity calculations, microbiome drug-metabolism events lacking a characterized enzyme are compared to all reactions in MetaCyc, a curated database of bacterial enzyme-driven reactions. Using profile Hidden Markov Models, the MetaCyc gene sequence annotations of these most similar reactions are then searched against human gut microbiome gene catalogs and metagenome repositories to yield candidate enzymes capable of the drug-metabolism query. When queried with thirty drugs metabolized by characterized gut bacterial enzymes, the pipeline accurately predicts the positive control enzymes. Furthermore, when queried with the as yet uncharacterized bacterial metabolism of methotrexate into DAMPA and glutamate, the pipeline predicts enzymes found in isolates previously shown to metabolize the anti-arthritic compound. Now, enzyme purification experiments are being employed to further validate the method’s ability to predict previously unknown gut bacterial enzymes responsible for drug metabolism. In sum, this project leverages the wealth of recent experimental evidence of drug-metabolism in the gut microbiome and expands prior work by determining the specific enzymes responsible.

“I am very grateful and honored to have received the PhRMA Foundation Predoctoral Fellowship in Informatics. The fellowship has provided me with extra support in my thesis research in human functional genomics. Furthermore, it has increased my confidence in exploring the great potential for human genomics in the future.”

Xiaoting Li | Columbia University

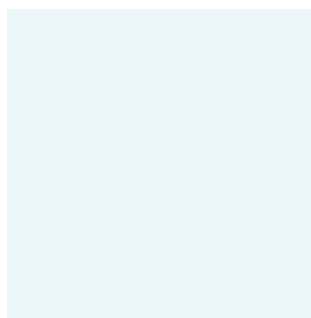
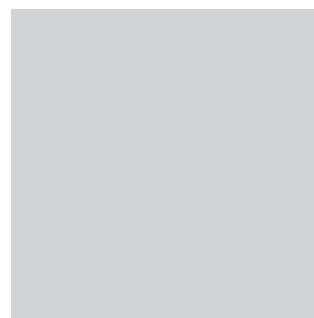


Xiaoting Li

Columbia University

“Identifying Genetic Regulatory Variants that Affect Transcription Factor Activity”

Genetic variation can impact gene expression either via cis-acting or via trans-acting mechanisms. Trans-acting loci affecting the regulatory activity of TFs have been referred to as activity quantitative trait loci (aQTLs). Mapping of trans-acting genetic variants to downstream genes is usually limited by statistical power. However, mapping the association between variants and the regulatory activity of TFs provides a complementary way to study how variants impact gene expression by modulating TF activity. The recent emergence of large collections of human functional genomics data has put human aQTL analysis within reach. Identifying aQTLs is important, both for understanding disease-causing variants and for gaining a better understanding of the cellular regulatory systems. To achieve this goal, this study has developed a generalized linear modeling (GLM) based method to systematically estimate activity levels of hundreds of human TFs in an individual-specific manner, and apply it to large human gene expression datasets. This will provide insight into how TFs regulate differential gene expression across tissues and across individuals. The inferred TF activity will be treated as a quantitative trait, and the genome-wide association mapping will be performed to identify genetic variants that are significantly associated with TF activity levels in each tissue. The identified aQTLs will provide insight into genetic determinants of TF regulatory network function.



“I am deeply honored to be supported by the PhRMA Foundation Fellowship in Informatics. The award has enabled me to devote my full attention to research on creating powerful machine learning models that shed light on the mechanisms underlying RNA splicing. Funding from the fellowship has been invaluable in advancing my career as a researcher. Thank you, PhRMA Foundation!”

Mukund Sudarshan | Courant Institute, New York University



Mukund Sudarshan

Courant Institute, New York University

“Insights Into RNA Splicing and Oligonucleotide Design From Machine Learning”

Splicing of pre-messenger RNA (pre-mRNA) is an essential process necessary for proper gene expression in eukaryotic cells, and occurs in the vast majority of human genes. During this process, the pre-mRNA is transformed into a mature messenger RNA (mRNA). This involves the recognition of intron/exon boundaries, the subsequent excision, or splicing, of the introns, followed by joining of the adjacent exons. The splicing process is governed by an array of sequence features known as the splicing code. This project seeks to significantly advance the understanding of this splicing code using machine learning models and apply this knowledge towards the development of splice-switching antisense oligonucleotides (ASOs) in particular. ASOs, such as the FDA-approved drug Nusinersen, are short chemically-modified nucleic acids that modulate the splicing process. Knowledge of this splicing process and being able to predict the exon sequence from any pre-mRNA sequence can lead to significant improvements in both the diagnosis and treatment of genetic disorders. There is a strong unmet clinical need for such knowledge in order to create efficient drugs for antisense therapy, as currently, the design of ASOs is often determined by a search through a very large space of sequences. Using state-of-the-art machine learning techniques, this project will model the splicing code in silico, and then use these models to rationally design ASOs. Preliminary work in designing machine learning models based on data from massively parallel reporter assays (MPRAs) is very encouraging and has led to high prediction accuracy, and more importantly, several mechanistic insights into the splicing code. Further insights from these machine learning models will assemble a coherent picture of the splicing code and how it interacts with ASOs.

“I am incredibly honored and grateful to have received the PhRMA Foundation Informatics Fellowship which provides invaluable support for my work in the study of how aging contributes to genome alterations observed in human cancers. On a personal level, the fellowship also enables me to have greater intellectual freedom at an early phase of my career to pursue interesting ideas that can lead to the betterment of human health.”

Kar-Tong Tan, BSc, MSc | Harvard University



Kar-Tong Tan, BSc, MSc

Harvard University

“Defining the Mechanistic Role of Aging in the Acquisition of Cancer Related Genomic Aberrations”

Aging is the strongest risk factor responsible for the development of cancers. Indeed, over 60% of people with cancer are age 65 and above. However, while chromosomal aberrations and somatic single nucleotide variants (sSNV) are widely observed and accepted as causal events responsible for tumorigenesis, how and to what extent aging causes the widespread acquisition of these genome aberrations in cancer is not known. Previous studies have highlighted how chromosomal aberrations and sSNVs that are widely observed in cancer can be induced respectively by telomere shortening, and DNA damages that one acquires as one ages. For instance, the shortening of telomeres is expected to cause telomere dysfunction and telomere fusions, which can drive the occurrence of chromosomal aberrations as demonstrated by previous studies. At the same time, the accumulation of DNA damages as one gets older can also lead to the accumulation of sSNVs when these sites are misread during DNA replication. Nonetheless, the extent to which these aging-related processes are involved in driving genome alterations in cancer patients is unclear. As such, this project seeks to establish the degree to which these processes contribute to genome alterations in cancer patients, which upon completion will highlight potential opportunities for therapeutic intervention to reduce the risk of onset of oncogenesis with aging.

2020 POSTDOCTORAL FELLOWSHIPS IN INFORMATICS

The PhRMA Foundation supports postdoctoral research activities that will enhance the expertise of informatics specialists and bridge experimental and computational approaches in genomic and biochemical studies. This award has served to provide a strong base for development of professional careers in research, both in academia and in industry.

“I am grateful to the PhRMA Foundation for supporting my research. The Postdoctoral Fellowship in Informatics has given me the opportunity to focus on my research project over the past year and provided me with the time and space to learn several key skills for analyzing modern genomic datasets. My development as a researcher has undoubtedly benefited from the experience made possible through the fellowship.”

Xiaofan Jin, PhD | J. David Gladstone Institutes



Xiaofan Jin, PhD

J. David Gladstone Institutes

“Investigating the Relationship Between Chromosomal Structure and Recombination During Meiosis”

Recombination during meiosis is the process by which parental chromosomes exchange genetic material. Errors in this process lead to negative health outcomes, and variability in recombination rate also affects genome evolution. In mammals, most crossovers occur in hotspots defined by the PRDM9 protein, though PRDM9 binding sites are not all equally hot. Meiotic chromosomes adopt distinct 3D structural organization, suggesting that differences in 3D genome organization may be linked to variable recombination activity at hotspots. To explore this idea, this project applies an integrative bioinformatics approach combining recently published measurements of 3D chromosomal organization during meiosis (Hi-C) as well as measurements of recombination activity including double-strand break (DSB) and crossover activity at PRDM9 hotspots. By stratifying recombination hotspots by their DSB and crossover activity, patterns of chromosomal configuration favoring recombination activity can be identified. The subsequent development of statistical models will then allow for the joint assessment of the contributions of different chromosomal structure variables to recombination activity. Collectively, these findings establish which aspects of chromosomal architecture are related recombination activity.

“Thanks to the PhRMA Foundation Postdoctoral Fellowship in Informatics I have been able to pursue such an ambitious project. This fellowship gave me the freedom to lay the foundations of my research which will now allow important discoveries in neuroscience and developments in machine learning models that will translate to future medical applications.”

Tiago Marques, PhD | MIT Department of Brain and Cognitive Sciences



Tiago Marques, PhD

MIT Department of Brain and Cognitive Sciences

“Building an Understanding of Visual Processing in the Brain Via New Neural Network Models”

Potential treatments for blindness, such as neuroprosthetics, require an understanding on how cortical areas in the brain represent visual information. For over half a century, neuroscientists have been studying the primate cortical ventral stream and its role in visual object recognition; that is, the ability to quickly recognize objects in visual scenes. Despite significant progress, we still do not fully understand the sequence of computations that enable this complex visual behavior. Recently, deep artificial neural networks (ANNs) brought a leap in performance in visual object recognition tasks. In addition, ANNs have unprecedented accuracy in predicting the patterns of neuronal responses in the primate visual cortex. Despite being the most accurate model class to describe primate visual processing, the extent of their ability to explain a wide range of neuronal and behavior effects remains limited. This project takes advantage of ANNs to build a more detailed understanding of visual processing in the brain by pursuing two complementary aims: (1) a comprehensive meta-analysis of state-of-the-art models of primate vision, followed by (2) the development of more accurate and brain-mappable biologically-inspired neural networks. By making comparisons with primate neural data, it was first observed that ANNs that better match the primate primary visual cortex (V1) are also more robust to image perturbations. Inspired by this observation, the VOneNet, a new class of hybrid ANN vision models, was developed. This novel network architecture contains a fixed front-end that simulates visual processing in V1, followed by a more traditional back-end adapted from current ANN models. VOneNets are not only substantially more robust than standard ANN models to a wide range of image perturbations, but they also better predict primate neuronal responses in visual cortical areas. These results demonstrate that more precisely mimicking the primate visual system leads to new gains in challenging computer vision applications. The development of even more biologically-matched models will point the way to a deeper understanding of the mechanisms underlying visual perception. This will have important implications, laying the foundations for the development of future therapeutics for vision and enabling more robust computer-assisted medical imaging diagnostics.

Pharmaceutics

The PhRMA Foundation began funding awards in Pharmaceutics in 1972.

2020 PREDOCTORAL FELLOWSHIPS IN PHARMACEUTICS

As one of the Foundation's longest-standing programs, the Predoctoral Fellowship in Pharmaceutics assists students engaged in dissertation research on relationships among drug delivery systems, gene therapy, and clinical applications. A stipend is provided when coursework has been completed and PhD candidates begin their final research project.

“This predoctoral fellowship has given me the financial support that has allowed me to focus on my dissertation research. I sincerely appreciate the PhRMA Foundation for the opportunity to do what I love to do as a pharmaceutical scientist.”

In Heon Lee, PharmD | Rutgers, The State University of New Jersey



In Heon Lee, PharmD

Rutgers, The State University of New Jersey

“Targeted Delivery of Nanoparticles to Cancer Stem Cells in Glioblastoma”

Glioblastoma multiforme (GBM) is the most common and lethal primary brain tumor, with overall survival of less than two years. Nearly all GBMs eventually recur. One likely cause of recurrence is the presence of cancer stem cells (CSCs), a small subpopulation of cancer cells that are more resistant to radio- and chemotherapy than normal cancer cells. Thus, a new treatment modality that is more specific to CSCs and less toxic to normal cells is urgently needed. CXCR4 is a receptor that is overexpressed on the cell surface in CSCs isolated from GBM tumors. CXCR4 proteins expressed on the cell surface are internalized upon ligand binding. It has been shown that nanoparticles (NPs) carrying a receptor agonist to CXCR4 or other receptors are internalized upon receptor binding, revealing that CXCR4 can function as portals for NP binding and cellular uptake. Interestingly, allosteric ligands, which bind to different sites on receptors than orthosteric ligands, can preserve the function of CXCR4 and minimize adverse effects expected from the use of orthosteric antagonists. The main goal of this project is to develop drug-loaded, CXCR4-targeted, allosteric peptide-conjugated NPs that can utilize the functional selectivity of allosteric peptides for selective drug delivery to cancer cells with less toxicity. This project will investigate the feasibility of CXCR4-targeted delivery of nanocarriers conjugated to allosteric peptides, fabricate and deliver CXCR4-targeted, drug-loaded polymeric NPs into CXCR4-expressing cells, and evaluate the in vitro efficacy of the CXCR4-targeted NPs for the treatment of GBM.

“At the start of my PhD, I had no prospects of competing for prestigious fellowships because international graduate students are typically ineligible for fellowships and awards. By changing the eligibility criteria, the PhRMA Foundation gave me an opportunity to attain a professional achievement that makes me a strong candidate for a career in the pharmaceutical industry. I am extremely honored to be a recipient of the PhRMA Foundation Predoctoral Fellowship in Pharmaceuticals. This fellowship has provided me the financial freedom and confidence to solely focus on advancing my dissertation research and has resulted in multiple first-author research manuscripts.”

Bhawana “Suruchi” Shrestha | The University of North Carolina at Chapel Hill



Bhawana “Suruchi” Shrestha

The University of North Carolina at Chapel Hill

“Engineering Ultra-Potent Sperm-Binding IgG Antibodies for Effective Non-Hormonal Female Contraception”

Nearly half of all pregnancies in the United States are unintended. Millions of women avoid using available contraceptives and risk unintended pregnancies every year, due to perceived and/or real side-effects associated with the use of exogenous hormones. This indicates a strong unmet need for alternative, non-hormonal contraceptives. Direct vaginal delivery of sperm-binding monoclonal antibodies (mAb) can provide the much-needed non-hormonal contraception. To address the unmet need, this project developed a panel of ultra-potent sperm-binding IgGs possessing 6-10 Fabs against a unique surface antigen universally present on human sperm. These highly multivalent IgGs (HM-IgGs) are at least 10- to 16-fold more potent and faster at agglutinating sperm than the parent IgG, while preserving Fc-mediated trapping of individual spermatozoa in vaginal mucus. The increased potencies translate to effective (>99.9%) reduction of progressively motile sperm in the sheep vagina using just 33 micrograms of the 10-Fab HM-IgG. HM-IgGs produce at comparable yields and possess identical thermal stability to the parent IgG, with greater homogeneity. HM-IgGs represent not only promising biologics for non-hormonal contraception but also a promising platform for generating potent agglutinating mAb for diverse medical applications.

2020 POSTDOCTORAL FELLOWSHIP IN PHARMACEUTICS

Postdoctoral Fellowships in Pharmaceuticals support scientists seeking to further develop and refine their pharmaceuticals research skills through formal postdoctoral training. The program was initiated to encourage more qualified graduates to obtain the postdoctoral research training so vitally needed in the area of Pharmaceuticals. The PhRMA Foundation recognizes the critical need for such well-trained scientific investigators.

“I am forever indebted to the PhRMA Foundation for the support it has provided me in my postdoctoral training. This award has given me the freedom to explore new horizons in my research while receiving world-class mentorship and development from my advisors. Thanks to the PhRMA Foundation I have been able to pursue new collaborations and project ideas, which will unequivocally set me apart for the next stage in my career.”

Danny Griffin, PhD | University of Kansas



Danny Griffin, PhD

University of Kansas

“Antigen-Fc Conjugates as Bioinspired Immunotherapeutics”

Current immunotherapies for treating autoimmune diseases like Type 1 Diabetes act nonspecifically to suppress the immune system. Such broad approaches may impede autoreactive tissue damage, but they also impose significant risks by compromising healthy immune functions as well. A significant pharmaceuticals opportunity exists for developing next-generation immunotherapies that can selectively target disease-causing cells to maximize potency and safety. We are developing a bioinspired, B cell-targeted strategy in which antigen-Fc conjugates are designed to recapitulate the native antibody-antigen complexes that are formed during the propagation of immune responses. These complexes typically home to germinal centers in the lymphatics, targeting B cell surface receptors to elicit potent effects across many arms of immunity. We have reproduced an inhibitory Fc protein, Fc(V12), that selectively binds with FcγRIIb, a natural negative feedback mechanism. We are coupling Fc(V12) to insulin antigen in bifunctional and multivalent configurations to colocalize B cell antigen receptors with FcγRIIb and anergize autoreactive populations for conferring antigen-specific tolerance. We are characterizing these conjugates to ensure retained functionality after modification, and we are assessing the targeted delivery of these constructs by evaluating their distribution and potency *in vivo*.

2020 RESEARCH STARTER GRANTS IN PHARMACEUTICS

A grant can do more than facilitate research. It can also motivate scientists who have no other viable funding sources and lay the groundwork for successful academic careers. The Research Starter Grant in Pharmaceuticals supports scientists who are beginning their academic research careers at the faculty level, and ensures the promising work of these researchers continues.

“I am incredibly grateful for the support from the PhRMA Foundation, especially through the events of 2020. Receipt of the Research Starter Grant has been critical to ensuring and sustaining the successful launch of an important area of my research program, allowing us to pursue a high risk-high reward research project that will not only yield long-term scientific progress, but also has a significant personal impact in supporting my overall career goals.”

Catherine Fromen, PhD | University of Delaware



Catherine Fromen, PhD

University of Delaware

“Tuning Degradation Rates of Dry Powder Hydrogel Nanoparticle Formulations to Drive Antigen-Specific Immune Responses in the Lung”

If the events of 2020 have shown us one thing, it is the dire need to develop new therapeutic and prophylactic approaches for respiratory diseases. Development of efficient inhalable immunotherapies remains a far-off goal, challenged in part by the paucity of knowledge surrounding the physiochemical properties which dictate desirable interactions with lung antigen presenting cells (APCs). There remains a critical need to develop particulate formulations specifically for lung administration and identify the physiochemical features that lead to optimized lung immune responses. Recent preliminary lab data has suggested that APC longevity is intimately controlled by the amount and frequency of nanoparticle (NP) internalization, as well as the rate of the particle intracellular degradation. Building from this preliminary data, this project’s central hypothesis is that slowly degrading particulate systems provide enhanced adjuvant-like effects that sustain APC viability and activation for extended antigen-specific adaptive immune responses. To test this hypothesis, the Fromen lab has engineered a library of antigen-loaded NPs for dry powder administration with tunable degradation rate and surface charge. The role of NP degradation rate and surface charge has been quantified in murine models of prophylactic lung vaccination, with ongoing efforts in a model of tolerogenic lung allergy. The proposed studies will broadly improve fundamental understanding of APC interactions with NP therapeutics in the lung, advancing development of inhalable NP immunoengineering while simultaneously catalyzing the PI’s research portfolio in pharmaceutical design of respiratory therapeutics.

“As a newly independent researcher, the PhRMA Foundation Research Starter Grant in Pharmaceuticals made a world of difference in my career path. It enabled me to work on a project that could play a crucial role in the enhancement of my academic career. I have been working on nucleic acid delivery since 2009, and the nanoparticles evaluated in this study can change the trajectory of my research career, since it afforded us the opportunity to deliver siRNA safely and efficiently into the targeted cells. I cannot thank the PhRMA Foundation enough for believing in me and this project.”

Hamidreza Montazeri – Pharm D, PhD | Chapman University



Hamidreza Montazeri – Pharm D, PhD

Chapman University

“Peptide/Lipid-Associated Nucleic Acids (PLANAs) for In Vivo Delivery of Nucleic Acids”

The full potential of RNA interference via small interfering RNA (siRNA) is yet to be realized due to challenges involved in efficient delivery of siRNA, which are also faced for delivering CRISPR/CAS9. This project will provide an efficient delivery system for nucleic acids to be used for investigational and therapeutic purposes. A library of lipid modified cationic peptides has been created that form complexes with nucleic acids via inter-ionic interaction. The preliminary data show effective cellular internalization of siRNA. The hypothesis for this project is that these peptides can be incorporated into a multicomponent nucleic acid delivery system, called Peptide/Lipid-Associated Nucleic Acid (PLANA). The immediate aim of this project is to optimize PLANAs for efficient and safe in vivo siRNA delivery. The long-term objective is to use this approach for targeted delivery to cancer cells to silence proteins involved in tumor growth. In the designed study, the PLANAs would be modified for targeted delivery to evaluate the in vivo efficiency in a tumor bearing animal model. Development of a reliable siRNA carrier affords researchers the opportunity to explore new possibilities by silencing virtually any protein for exploratory research or therapeutic purposes. With the multicomponent nature of PLANAs, small adjustments could create carriers tailored for delivering different nucleic acids.



BOARD OF DIRECTORS



Alfred W. Sandrock, MD, PhD
Chairman
Executive Vice President, Head
of Research and Development
 Biogen
 Cambridge, Massachusetts



Andrew Plump, MD, PhD
Treasurer
President, R&D
 Takeda Pharmaceutical Co., Ltd.
 Cambridge, Massachusetts



Mikael Dolsten, MD, PhD
Chief Scientific Officer and President,
Worldwide Research, Development
and Medical
 Pfizer Inc.
 New York, New York



Prof. Dr. med. Iris Loew-Friedrich
Chief Medical Officer, Executive Vice
President of Development & Solutions
 UCB S.A.
 Monheim, Germany



Aran Maree, MB BCH, MRCPI
Chief Medical Officer, Pharmaceuticals
 Janssen Pharmaceutical Companies of
 Johnson & Johnson
 New Brunswick, New Jersey



Joseph P. Miletich, MD, PhD
Senior Vice President
Discovery Research, Preclinical
Sciences & Early Development
 Merck Research Laboratories
 Rahway, New Jersey



John C. Reed, MD, PhD
Executive Vice President,
Global Head R&D
 Sanofi
 Cambridge, Massachusetts



Daniel Skovronsky, MD, PhD
President, Lilly Research Laboratories
Chief Scientific Officer,
 Eli Lilly and Company
 Indianapolis, Indiana



Rupert Vessey, MA, BM BCH, FRCP, DPhil
Executive Vice President,
Research and Early Development
 Bristol Myers Squibb
 Princeton, New Jersey



Richard A. Moscicki, MD
Executive Vice President
and Chief Medical Officer
 PhRMA
 Washington, DC

Treasurer's Report



In a challenging year of a global pandemic, we are beyond grateful for the ongoing support of our generous benefactors. Because of this support, the Foundation has continued to move forward with important new initiatives developed in alignment with the priorities of PhRMA member companies. The Foundation is dedicated to improving public health and the lives of patients by proactively investing in innovative research, education and value-driven health care and I am excited to share the details of our progress with you.

I am pleased to report the PhRMA Foundation achieved its financial goals in 2020 and is planning to make an even greater impact in 2021 with restructured core programs and the development of exciting new initiatives. Member company contributions were \$3.4 million, which is 6% higher than 2019. These contributions along with its investments are the Foundation's sole support.

The Foundation's total expenditures were down 5.7% to \$4.86 million in 2020 versus \$5.1 million in 2019. In 2020 our program expenses and services were reduced due in part to COVID-19. We conducted our meetings remotely and all related travel expenses were not incurred. We also experienced a reduction of 5.6% in the award program, reflecting a transition year with reduced spending in the former core programs and a lag time in ramping up spending on new areas of focus, such as the Value Assessment initiative, which prioritizes patient-centricity and health equity as fundamental to the Foundation's forward-looking focus.

The Value Assessment Initiative is now in its fourth year of funding awards. The program spending in this category increased 11% over the previous year. In 2020, all four Centers of Excellence (Healthcare Value Assessment, Patient Driven Values in Healthcare Evaluation, Enhanced Value Assessment and Pharmaceutical Value) were fully supported, and we funded new Research and Challenge Awards. Further, we invested in research efforts supporting the advancement of Patient-Centered Outcomes to complement and enhance the Value Assessment activities in 2020. This initiative continues to support activities that lead to the development and application of high-quality, patient-centered approaches to value assessment offering transformative solutions that reflect patient preferences and real-world clinical practice.

Net Assets on December 31 were \$24.4 million, a 4.4% increase from the prior year. The increase in net assets is attributable to the return on the Foundation's investments. Financial details are shown in the accompanying Statement of Activities.

Sincerely,

A handwritten signature in dark ink, appearing to be 'A. Plump', with a long horizontal flourish extending to the right.

Andrew Plump, MD, PhD
Treasurer, PhRMA Foundation

Statement of Activities

For the year ended December 31, 2020

REVENUE AND SUPPORT	
Contributions	\$3,397,859
Contributions – in kind from PhRMA ¹	\$49,738
Interest and Dividends	\$477,282
(Realized and Unrealized) Gains in Securities	\$1,422,282
Other Income	\$31,541
Total Revenue and Support	\$5,378,702

EXPENSES	
PROGRAMS	
Clinical Pharmacology Program	\$149,250
Health Outcomes Program	\$442,917
Informatics Program	\$323,750
Pharmaceutics Program	\$351,667
Pharmacology Program	\$588,750
Translational Medicine Program	\$445,826
Value Assessment Initiative	\$1,317,954
AFPE Fellowship Award	\$11,000
Healthcare Care Ready Grant	\$75,000
Subtotal – Grants and Awards	\$3,706,114

OTHER	
Program Expenses and Services	\$878,145
Events and Meetings	\$6,112
Total Program Services	\$4,590,371

SUPPORTING SERVICES:	
Management and General	\$268,246
Rent & Accounting Services ¹	\$49,738
Fundraising	\$752
Total Supporting Services	\$268,998

TOTAL EXPENSES	\$4,859,369
-----------------------	--------------------

¹ Rent and Accounting Services are donated by PhRMA not included in total expenses

Change in net assets	\$1,025,950
Net assets beginning of year	\$23,397,904
Net assets at end of year	\$24,423,854

Advisory Committees

SCIENTIFIC ADVISORY COMMITTEE

Richard Moscicki, MD

Chairman

Executive Vice President and
Chief Medical Officer
PhRMA

Terry L. Bowlin, PhD

President & Chief Executive Officer
Microbiotix, Inc.

Michael J. Hageman, PhD

Valentino J. Stella Distinguished Professor
Department of Pharmaceutical Chemistry
The University of Kansas

Michael N. Liebman, PhD

Managing Director
IPQ Analytics, LLC

Edmund J. Pezalla, MD, MPH

Chief Executive Officer
Enlightenment Bioconsult, LLC

Nancy C. Santanello, MD, MS, FISPE

Independent Research Consultant
Former: Vice President, Epidemiology,
Merck MSD

PHARMACOLOGY/TOXICOLOGY ADVISORY COMMITTEE

Terry L. Bowlin, PhD

Chairman

President & Chief Executive Officer
Microbiotix, Inc.

Daniel Acosta, Jr., PhD

Retired as Deputy Director for Research
FDA's National Center for Toxicological
Research

David J. Aldous, PhD

Head of Global R&D Processes and
Operations
Sanofi

Craig T. Basson, MD, PhD

Chief Medical Officer
Boston Pharmaceuticals

David G. Beer, PhD

Professor Emeritus, Active
University of Michigan

Namandjé N. Bumpus, PhD

Director, Department of Pharmacology
and Molecular Sciences
Professor of Pharmacology and
Molecular Sciences
Johns Hopkins University

Kenneth Dretchen, PhD

Professor Emeritus
Department of Pharmacology and Physiology
Georgetown University Medical Center
Vice President and Chief Science Officer
Mesa Science Associates Inc.

Margarita L. Dubocovich, PhD, FACNP, FASPET

SUNY Distinguished Professor
Department of Pharmacology
and Toxicology
Senior Associate Dean for Diversity
and Inclusion
Jacobs School of Medicine and
Biomedical Science
University at Buffalo (SUNY)

Salvatore J. Enna, PhD

Emeritus Professor of Physiology and of
Pharmacology
University of Kansas Medical Center

Nassir Ghaemi, MD, MPH

Translational Medicine, Neuroscience
Novartis Institutes for Biomedical Research
Professor of Psychiatry
Tufts University School of Medicine

Stephen M. Lanier, PhD

Professor of Pharmacology
Vice President for Research
Wayne State University

Francis Lee, PhD

Vice President, Biology
Angex Pharmaceutical, Inc.

Harry LeVine, III, PhD

Associate Professor
Center on Aging, Dept. of Molecular &
Cellular Biochemistry
University of Kentucky

Mark S. Marshall, PhD

Co-Director Riley Precision Genomics
Department of Pediatrics
Riley Hospital for Children

Gary O. Rankin, PhD

Vice Dean for Basic Services
Professor and Chair
Department of Biomedical Sciences
Joan C. Edwards School of Medicine
Marshall University

Christopher J. Schmidt, PhD

Retired as Executive Director Science and
Technology Group, Digital Medicine
Early Clinical Development
Pfizer Inc.

Darryle D. Schoepp, PhD

Retired as Vice President & Therapeutic
Area Head Neuroscience Discovery
Preclinical and Early Development
Merck Research Laboratories

Stephanie W. Watts, PhD

Professor, Pharmacology & Toxicology
Michigan State University

Megan Yao, PhD

Retired as Vice President of Oncology
Translational Medicine
Eli Lilly and Company

HEALTH OUTCOMES RESEARCH ADVISORY COMMITTEE

Nancy C. Santanello, MD, MS, FISPE

Chairman

Independent Research Consultant

Scott Devine, MPH, PhD

Executive Director

Center for Observational and Real-world
Evidence

Merck & Co., Inc.

Duska M Franic, BPharm, PharmD, MS, PhD

Associate Professor

College of Pharmacy

University of Georgia

Geoffrey T. Makinson, PhD

Life Sciences Consultant

Carrie McAdam-Marx, PhD, RPh

Professor

Department of Pharmacy Practice & Science

University of Nebraska Medical Center

Newell McElwee, PharmD, MSPH

Vice President

Health Economics and Outcomes Research

Boehringer Ingelheim Pharmaceuticals Inc.

Eberechukwu Onukwugha, MS, PhD

Executive Director

Pharmaceutical Research Computing

Associate Professor

Department of Pharmaceutical Health

Services Research

University of Maryland School of Pharmacy

David Veenstra, PhD, PharmD

Professor

Comparative Health Outcomes, Policy &

Economics (CHOICE) Institute

Department of Pharmacy

University of Washington,

Junling Wang, PhD

Professor and Vice Chair for Research

Department of Clinical Pharmacy &

Translational Science

College of Pharmacy

University of Tennessee

Tatia C. Woodward, MPH, MS

Senior Director, Global Value Evidence

Outcomes, Oncology

GlaxoSmithKline

INFORMATICS ADVISORY COMMITTEE

Michael N. Liebman, PhD

Chairman

Managing Director

IPQ Analytics, LLC

Pankaj Agarwal, PhD

Principal Computational Biologist

Bioinfi LLC

Yana Bromberg, PhD

Associate Professor

School of Environmental & Biological
Sciences

Rutgers University

Jeremy Jenkins, PhD

U.S. Head Chemical Biology & Therapeutics

Novartis Institutes for BioMedical Research

Robert N. McBurney, PhD

Chief Executive Officer

Accelerated Cure Project for MS

Board Member, Optimal Medicine, Ltd.

Michelle M. Meyer, PhD

Associate Professor of Biology

Boston College

Peter A. Schad, PhD

Principal

BioHelix Consulting LLC

David B. Searls, PhD

Independent Consultant

PHARMACEUTICS SCIENTIFIC ADVISORY COMMITTEE

Michael J. Hageman, PhD

Chairman

Valentino J. Stella Distinguished Professor

Department of Pharmaceutical Chemistry

The University of Kansas

Cory Berkland, PhD

Solon E. Summerfield Distinguished Professor

Department of Pharmaceutical Chemistry

Department of Chemical Engineering

University of Kansas

Robin Bogner, PhD, FAAPS

Professor of Pharmaceutics

University of Connecticut

Bruno Hancock, PhD

Global Head of Materials Science

(Small Molecule Pharmaceutical Sciences)

Pfizer Inc.

Patrick J. Sinko, PhD, RPh

Distinguished Professor of Pharmaceutics

Parke Davis Professor of Drug Delivery

Ernest Mario School of Pharmacy

Rutgers, The State University of New Jersey

Kumar Soppimath, PhD

Chief Scientific Officer

Nevakar, Inc.

NJ Center of Excellence

TRANSLATIONAL MEDICINE ADVISORY COMMITTEE

Michael Liebman, PhD

Chairman

Managing Director

IPQ Analytics, LLC

Advisory Committees

Pankaj Agarwal, PhD

Principal Computational Biologist
Bioinfi LLC

Craig T. Basson, MD, PhD

Chief Medical Officer
Boston Pharmaceuticals

Anastasia M. Khoury Christianson, PhD

Vice President, R&D Operations and
Oncology IT
Janssen Pharmaceuticals
Johnson and Johnson

Donald E. Mager, PharmD, PhD

Professor and Vice Chair
Department of Pharmaceutical Sciences
University at Buffalo, SUNY

Michael Montgomery, MD

CEO & Co-Founder
Stable Solutions LLC (a medical and machine
learning consultancy)

Maria Palmisano, MD

Senior Vice President
Research and Early Development
Clinical Pharmacology and Pharmacometrics
Early Development Immunology,
Cardiovascular, and Fibrosis
Bristol Myers Squibb

Kathryn Sandberg, PhD

Professor and Vice Chair of Research,
Department of Medicine
Director, Center for the Study of Sex
Differences in Health, Aging and Disease
Georgetown University
Director, Predoc and Postdoc Training
Program in Translational Biomedical Science
Georgetown-Howard Universities Center for
Clinical and Translational Science

Peter A. Schad, PhD

Principal
BioHelix Consulting LLC

Scott Steele, PhD

Director, Regulatory Science Programs
Clinical and Translational Science Institute
Associate Professor, Public Health Sciences
University of Rochester

VALUE ASSESSMENT PROGRAM ADVISORY COMMITTEE

Edmund J. Pezalla, MD, MPH

Chairman
Chief Executive Officer
Enlightenment Bioconsult, LLC

Alan Balch, PhD

Chief Executive Officer
Patient Advocate Foundation

Charlie E. Barr, MD, MPH

Chief Science Officer
Pivotal Strategic Consulting LLC

Linda Bohannon, RN, BSN, MSM

Chief Executive Officer
Cancer Support Community

Louis P. Garrison, Jr., PhD

Professor Emeritus
The Comparative Health Outcomes, Policy,
and Economics (CHOICE) Institute
Department of Pharmacy
University of Washington

Jan Hansen, PhD

Vice President, Evidence for Access
Medical Unit,
US Medical Affairs
Genentech

Sachin Kamal-Bahl, PhD

President & Founder
COVIA Health Solutions

Edward H. Lee, PharmD (Eddie)

Head (Director),
Health Economics and Outcomes Research
US Immunology
UCB, Inc.

Peter J. Neumann, ScD

Director
Center for the Evaluation of Value and Risk
in Health at the Institute for
Clinical Research and Health Policy Studies
Tufts Medical Center;
Professor of Medicine
Tufts University School of Medicine

Shelby D. Reed, PhD, RPh

Past President, ISPOR
Professor, Depts. of Medicine and Population
Health Sciences
Director, Preference Evaluation Research
Group (PrefER)
Center for Informing Health Decisions
Duke Clinical Research Institute
Duke University

Vipan C. Sood, RPh, MBA, MRPharms

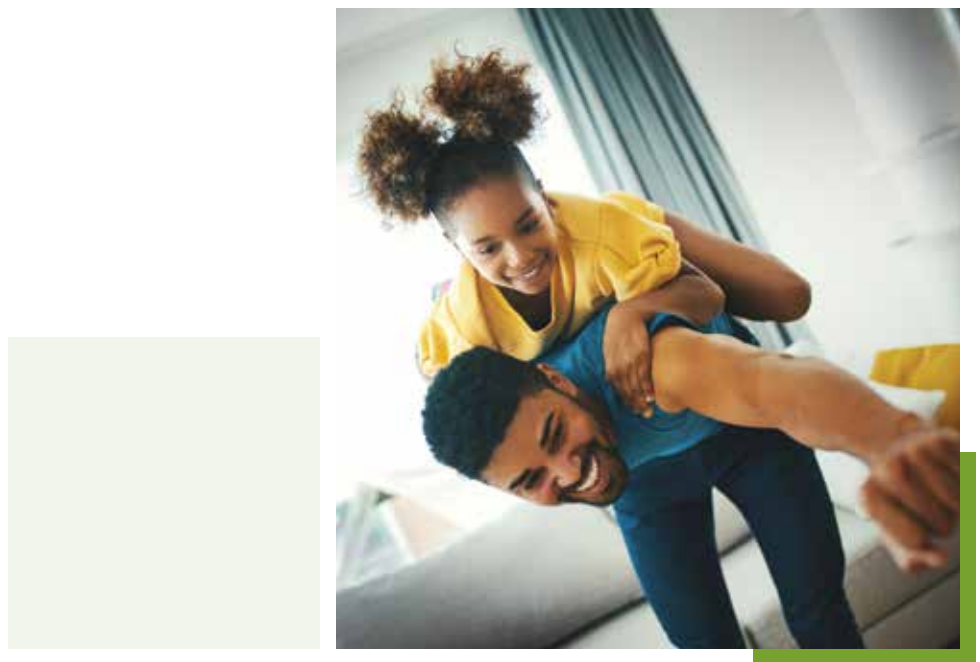
Vice President, HEOR, Specialty
AbbVie, Inc.

Jason M. M. Spangler, MD, MPH, FACPM

Executive Director,
International Health Policy &
Population Health
Global Government Affairs & Policy
Amgen, Inc.

Richard J. Willke, PhD

Chief Scientific Officer,
ISPOR



PhRMA Foundation Programs for 2021

Name of Program/ Year of First Awards	Number of Awards Budgeted Yearly/ Length of Award	Program Budget	Deadline Announcement Date Starting Time
Drug Delivery			
Predoctoral Fellowships in Pharmacology/Toxicology (1978)	4 awarded/up to 2 years	\$200,000 total \$25,000 per award per year	September 1, 2020 December 15, 2020 January 2021
Postdoctoral Fellowships in Pharmacology/Toxicology (2002)	1 awarded/2 years	\$100,000 total \$50,000 per award per year	September 1, 2020 December 15, 2020 January 2021
Research Starter Grants in Pharmacology/Toxicology (1972)	2 awarded/1 year	\$200,000 total \$100,000 per award per year	September 1, 2020 December 15, 2020 January 2021
Drug Discovery			
Predoctoral Fellowships in Pharmaceutics (1987)	5 awarded/up to 2 years	\$250,000 total \$25,000 per award per year	September 1, 2020 December 15, 2020 January 2021
Postdoctoral Fellowship in Pharmaceutics (1992)	2 awarded/2 years	\$200,000 total \$50,000 per award per year	September 1, 2020 December 15, 2020 January 2021
Research Starter Grants in Pharmaceutics (1972)	2 awarded/1 year	\$200,000 total \$100,000 per award per year	September 1, 2020 December 15, 2020 January 2021

Name of Program/ Year of First Awards	Number of Awards Budgeted Yearly/ Length of Award	Program Budget	Deadline Announcement Date Starting Time
Health Outcomes Research			
Predocutorial Fellowships in Health Outcomes (2002)	4 awarded/up to 2 years	\$137,500 total \$25,000 per award per year	February 1, 2021 April 15, 2021 July 2021
Postdoctoral Fellowship in Health Outcomes (2002)	0 awarded/2 years	\$0 total \$55,000 per award per year	February 1, 2021 April 15, 2021 July 2021
Research Starter Grants in Health Outcomes (2002)	3 awarded/1 year	\$300,000 total \$100,000 per award per year	February 1, 2021 April 15, 2021 July 2021
Translational Medicine			
Postdoctoral Fellowships in Translational Medicine (2016)	3 awarded/2 years	\$360,000 total \$60,000 per award per year	February 1, 2021 April 15, 2021 July 2021
Research Starter Grants in Translational Medicine (2016)	1 awarded/1 year	\$100,000 total \$100,000 per award per year	February 1, 2021 April 15, 2021 July 2021
Value Assessment Initiative			
Addressing Health Disparity Challenge Award (2021)	4 awards	\$5,000 - \$50,000 per award	March 2021
Challenge Awards (2018) for Value Assessment	4 awards	\$5,000 - \$50,000 per award	December 2021
Research Awards (2018) for Value Assessment	3 awarded/ 1 year	\$300,000 total \$100,000 per award per year	September 1, 2020 December 15, 2020 January 2021

All of the above programs will accept applications for research on drugs for rare diseases.

ETHICAL CONSIDERATIONS: The Scientific Advisory Committee as well as the program advisory committees of the PhRMA Foundation are dedicated to ensuring the appropriate use of animals and humans in research. In their deliberations, they consider all aspects of a proposal and may deny support for many reasons. Careful consideration is given to ensure the humane use and care of animal subjects. For human and animal research, the project review committee requires, in writing, a statement of adherence to prevailing standards of ethical research practices. Institutional Review Board approval is required before any research project may be initiated. In addition, informed consent is required before any person can participate in a research project.

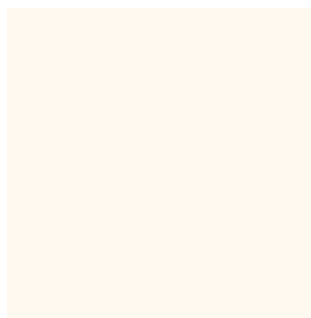
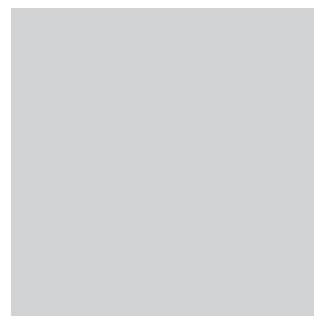
PhRMA Foundation Staff



Eileen Cannon
President



Joanne Westphal
Director of Development





950 F Street NW, Suite 300, Washington, DC 20004
202.572.7756 | foundation@phrma.org