2021 ANNUAL REPORT

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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.

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Message from the Chair

For decades, the PhRMA Foundation helped catalyze new fields with ardent support for emerging scientific disciplines overlooked from a funding perspective, such as translational medicine, clinical pharmacology, toxicology, informatics, and pharmaceutical sciences. These disciplines are critical to drug development, but often neglected by basic research funding agencies.

Since joining the PhRMA Foundation's Board in 2015, I've come to see immense value in the Foundation's efforts to fill these gaps, helping to advance research in these and other research frontiers vital to the development of tomorrow's new medicines. Hand-in-hand with filling that scientific gap is the Foundation's work to facilitate research more broadly into topics relevant to health care policy, including health outcomes research and value assessment.

As I think about the pharmaceutical industry's needs in the coming decades, I'm reminded of a quote from Mahatma Gandhi: "The future depends on what we do in the present." The ways in which the PhRMA Foundation funds progress today will help our industry more effectively serve global populations in the future.

Advancing the Frontiers of Science, Social Science, and Big Data

The PhRMA Foundation has always focused its resources on advancing cutting-edge research in the quest to identify new tools and methods for drug discovery. But equally important is the Foundation's work to marry basic science with data science and social sciences to appropriately value new medicines and health care delivery models.

Digital technologies can serve as a bridge connecting experiments at the laboratory bench with data generated in the clinic. The PhRMA Foundation supports multiple efforts that use these digital tools in innovative ways to develop new quantitative and qualitative approaches to assess the value of our medicines and, ultimately, the benefits they bring to patients. This work cannot be done by industry alone — it cuts across companies, academia, regulators, and policymakers — and the Foundation is in a great position to enable the entire ecosystem.

Fostering Inclusion and Diversity

Diversity is an important area for further exploration in the pharmaceutical industry. To serve the health care needs of the diverse patient populations around the globe, we must be inclusive in our ways of thinking and working, recognizing that what works for one population (from either a medical perspective or a psychological one) may not work for all.

I've been inspired by the PhRMA Foundation's value assessment program. In particular, its ability to recognize the needs of populations historically underrepresented in research by helping to develop new methods for patient-centered outcomes research that benefit diverse patient groups. Seeking and considering diverse perspectives is an important first step to addressing drivers of health disparities, which have an important impact on overall health outcomes.

Charting a Path for the Future

The Foundation has successfully evolved its focus into relevant areas of value, data, digital health, and health equity, as well as undergone significant leadership transitions. Eileen Cannon retired after 22 years of dedicated service, passing the reins to Amy M. Miller, who joins as the Foundation's new president with a great passion for and deep experience in health care advocacy and leadership. Iris Loew-Friedrich, chief medical officer at UCB, is stepping in as Foundation treasurer as I move into the role of Board chair, succeeding Alfred Sandrock, now chief executive officer of Voyager Therapeutics, who led the Board for the past six years.

We look forward to working with our dedicated Board members, executive team, volunteers, and other key stakeholders as we look to 2022 and beyond.

Andrew Plump, MD, PhD
Chair, PhRMA Foundation

Message from the President

When I took the helm of the PhRMA Foundation in 2021, I was awed by its impressive 57-year history of funding novel research and inspired by the opportunity to build on this strong foundation during a time of rapid innovation in the pharmaceutical industry. 2021 saw new treatment modalities like mRNA vaccines widely deployed for the first time, the approval of cutting-edge therapies for unmet health needs from opioid overdose to rare cancers, and a swirling debate about patient access to new medicines.

Against this backdrop, the Foundation continued to fund innovative research and grow a diverse talent pipeline for academia, government, and industry by catalyzing the careers of promising young researchers focused on solving big problems.

Supporting Meaningful Research

In 2021, out of 315 applicants, the Foundation funded 42 researchers conducting work in drug delivery, drug discovery, translational medicine, health outcomes, and value assessment research, awarding a total of $3 million.

These researchers are working on studies to explore patients' use of and willingness to pay for high- and low-value treatments, better understand patients' medication adherence behavior patterns, develop broad-spectrum antivirals that would protect against SARS-CoV-2, and test new therapeutic targets for diseases such as Alzheimer's, Parkinson's, and leukemia.

The Foundation also worked to promote patient-centered approaches in value assessment through a webinar series. These workshops sought to raise awareness of the gaps in the capture and use of patient-centered outcomes and impacts in value assessment, and to promote methods in current practice that prioritize patient-centered outcomes and inclusive patient engagement in value assessment.

Building a Diverse Talent Pipeline

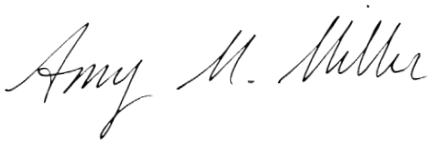
Since its inception, the PhRMA Foundation has funded more than 2,700 scientists at more than 300 institutions throughout the U.S. Many of these researchers have gone on to have illustrious careers, including Dr. Arthur Hayes, who later served as an FDA commissioner; entrepreneur Dr. J. Craig Venter, who is well-known for his genome research, and researcher Dr. Susan Band Horwitz, who discovered the mechanisms behind the anti-cancer drug Taxol.

Because the Foundation funds primarily early-career researchers — predocs, postdocs, and new tenure-track faculty — we have a unique opportunity to invest in the true frontiers of innovation. We strive to support scientists who think creatively. The penchant for tackling big questions in new ways is a thread that runs through all PhRMA Foundation grant programs.

In addition, the Foundation understands the importance of building a diverse pipeline of researchers to tackle the next big challenges in health care. Notably, in recent years, approximately half of our grantees have been women and half have been people of color.

Extending Our Reach

The Foundation's work — fostering important fields like toxicology, for example — has had an enduring impact, yet these contributions to the industry are not well-known. Looking to the future, with help from the Foundation's first-ever head of communications, we plan to expand our reach by communicating more broadly about our work and building awareness of our solutions-based approach to addressing the challenges of pharmaceutical innovation and policy. I look forward to working with the Foundation's Board, our team, our partners, and our researchers to improve public health and value-driven health care through innovative research.



Amy M. Miller, PhD
President, PhRMA Foundation

FELLOWSHIPS AND GRANTS

Value Assessment

2021 RESEARCH AWARDS IN VALUE ASSESSMENT

The PhRMA Foundation Value Assessment Initiative launched in 2017 with Challenge Awards, followed by Research Awards in 2018. Value assessment in health care comprises a broad set of methods to synthesize and evaluate the relative benefits and costs of medical interventions. The goal of value assessment is to assist stakeholders — including patients, providers, and payers — in making informed decisions about the tradeoffs involved in pursuing health interventions.

The PhRMA Foundation research awards fund projects focused on assessing the true value of medicines and health care services.

Understanding Patient Cost-Sharing Thresholds for High- and Low-Value Care Towards Development of a Value-Based Formulary

"The funding from this PhRMA Foundation award has determined patient perspectives on the value of treatment options for diabetic medications compared to value assessment by a health plan. By further aligning provider, payer, and patient preferences, this study has the potential to yield improved clinical outcomes and increase cost-savings to patients, beneficiaries, and health plans."

Diana Brixner, PhD, RPh, FAMCP
University of Utah, College of Pharmacy

The process used to prefer certain products across drug classes for diabetes is generally focused on comparative effectiveness and cost. However, payers rarely tie patient preference for treatment attributes to formulary management, resulting in a misalignment of value as defined by providers, payers, and patients. Our study explores patient preferences and willingness to pay for treatment attributes of predetermined high-value and low-value medications within a health plan, in order to redefine value to incorporate patient preferences. A cross-sectional discrete choice experiment (DCE) questionnaire study design determined patient preferences for the benefit, risk, and cost attributes of type 2 diabetes treatments. A comprehensive literature review of patient preference studies in diabetes identified studied attributes, and a review of guidelines and medical literature identified clinical attributes. Patients and diabetes experts were interviewed and instructed to identify, prioritize, and comment on which attributes of diabetes medications were most important to them. From these interviews, a total of 7 attributes were selected for the dissemination for a DCE survey: 1) hemoglobin A1C reduction, 2) cardiovascular (CV) risk reduction, 3) heart failure (HF) risk reduction, 4) out-of-pocket cost, 5) route of administration, 6) dosing flexibility, and 7) gastrointestinal (GI) side effects. From the 58 health plan beneficiaries who responded to the DCE survey, patients preferred to be treated versus foregoing care. Attribute preferences such as cost, HF risk reduction, CV risk reduction, and GI side effects were found to be statistically significant. These survey results were used to calculate the willingness-to-pay thresholds to demonstrate which diabetic medications are preferred most by patients. These findings will then be compared with how the health plan ranks medications in order to compare where patients' and health plans' definitions of value are aligned and where differences arise. By aligning patient and stakeholder preferences, our study has the potential to yield improved patient outcomes and increase cost-savings to patients, beneficiaries, and health plans.

Measuring the Value of Fear of Contagion in COVID-19 Care

"The PhRMA Foundation Value Assessment Research Award provided me an opportunity to assess a new value element. Subsequently, it has helped me achieve my career goal, which is to help patients access effective and preferred medications and health care services."

Surachat Ngorsuraches, PhD
Auburn University Harrison College of Pharmacy

The objective of this study is to obtain a quantitative measure of the value of fear of contagion in COVID-19 care. A discrete choice experiment (DCE) will be conducted. A list of important attributes of COVID-19 medical options – including the possibility of disease exposure and cost – will be obtained from literature, clinical experts, and adults from the general public. The attributes will be used to generate DCE choice sets, which are included in a self-administered, web-based survey. A total of 500 adults with and without COVID-19 infection will be asked to respond to the survey. Statistical analysis based on Random Utility Theory will be used to determine the relative importance between all study attributes and cost. The willingness-to-pay for reducing the possibility of disease exposure will be calculated. Study findings will be applied for value assessments when dealing with not only COVID-19 care but also care for other infectious diseases.

Estimating the Value of Diabetes Prevention Programs Using Real-World Data

"The Value Assessment Research Award funding has made it possible for me to explore the use of real-world data to inform value assessments in heterogeneous populations, for example, reflecting impacts of health inequality and exploring how the value of diabetes prevention may differ by race or ethnicity. On a personal level, the grant also takes me further along the path of establishing myself in the role of principal investigator and building my research portfolio as an early-career independent researcher."

Natalia Olchanski, PhD
Tufts Medical Center

Reliance on narrowly defined trial cohorts for generation of value evidence may limit the generalizability and applicability of the results to broader populations that may be treated in real-world practice. This study will provide important insights into the risk profile of a real-world population receiving an intervention, as well as how it might differ from the risk profile of the clinical trial population, using the case study of the Diabetes Prevention Program (DPP) for individuals with prediabetes. It will subsequently provide insights into how the value of DPP in real-world practice may differ from health economic estimates based on clinical trials.

There is substantial variation in intervention benefits from diabetes prevention, such as the national DPP, in individuals with prediabetes in the United States. Because these benefits drive variation in the value, or cost-effectiveness, of DPP across the population, it is important to accurately capture real-world eligible population characteristics. One approach is to use real-world data to supplement health economic assessments based on clinical trial cohorts.

We propose to use national survey data, including the National Health and Nutrition Examination Survey, to characterize the diabetes risk of individuals referred to or actually receiving DPP in the United States, using validated diabetes risk-scoring instruments. We will then use individual-level simulation modeling to estimate the individual and population level value of DPP. Finally, we will compare these distributions between the DPP clinical trial population and the real-world population to assess the impact of using real-world data on value assessment for DPP.

2021 CHALLENGE AWARDS IN VALUE ASSESSMENT

This program encourages researchers – regardless of career stage – to develop publication-worthy research papers that describe solutions to a pressing question in health care value assessment.

In 2021, the program focused on how the field of value assessment can better serve diverse populations and address the drivers of health disparities by asking the following question:

How can value assessment methods and processes better account for populations that are typically underrepresented in research and drivers of health disparities?

The four winning proposals were published in a special supplement of the ***Journal of Managed Care & Specialty Pharmacy***. Each winning team has made a substantial contribution toward making the value assessment field more inclusive and better attuned to the needs of diverse populations.

The PhRMA Foundation congratulates the award recipients, whose proposals are an important first step toward meaningfully refocusing value assessment through a health equity lens.

First Place

The Effect of Unobserved Preferences and Race on Vaccination Hesitancy for COVID-19 Vaccines: Implications for Health Disparities

The first-place team conducted an analysis of COVID-19 vaccine preferences among underrepresented populations. Using latent class analysis, the team built a model identifying key factors underlying the disparities in COVID-19 vaccination. They found that health care interventions intended to reduce health disparities that do not reflect the underlying values of individuals in underrepresented populations are unlikely to be successful.

Eline M. van den Broek-Altenburg, PhD
Larner College of Medicine, University of Vermont

Jamie S. Benson, BA
Larner College of Medicine, University of Vermont

Adam J. Atherly, PhD
Larner College of Medicine, University of Vermont

Stephane Hess, PhD
Choice Modelling Centre and Institute for Transport Studies, University of Leeds

Second Place

Using Latent Class and Quantum Models to Value Equity in Health Care: A Tale of 2 Stories

The second-place award recipient described two approaches to empirically address health equity in value assessment: using a discrete choice experiment to elicit preferences from individuals on value attributes with a latent class model to derive the value of equity and using a flexible choice model to value health equity. Both approaches aim to capture patient preferences and ensure the systematic consideration of equity in health care decision-making.

Surachat Ngorsuraches, PhD
Harrison School of Pharmacy, Auburn University

Third Place (Tie)

It's Time to Represent: Shifting the Paradigm to Improve the Quality of Inputs into Value Assessment Frameworks

A team from the Texas Center for Health Outcomes Research & Education (TxCORE) at the University of Texas at Austin proposed a two-pronged strategy to increase the diversity of populations that participate in research and address drivers of health disparities to better inform value assessment. The first part of this strategy consisted of a comprehensive national campaign to inform, create buy-in, and generate excitement for participation in research. Following this, the researchers proposed an expediting of current methodological initiatives to require a minimum set of patient-reported social determinants of health elements to be collected and reported in research, including clinical trials and observational studies, as a way to enhance the information used in value assessment frameworks.

Leticia R Moczygemba, PharmD, PhD
College of Pharmacy, University of Texas at Austin

Carolyn Brown, PhD
College of Pharmacy, University of Texas at Austin

Michael Johnsrud, PhD, RPh
College of Pharmacy, University of Texas at Austin

Third Place (Tie)

Incorporating Health Equity into Value Assessment: Frameworks, Promising Alternatives, and Future Directions

A team from the University of Florida and Florida A&M University examined emerging value assessment frameworks in the United States and presented examples where evidence on outcomes and preferences for value do not take into consideration diverse perspectives. They then identified possible solutions to improve existing value assessment methods and used a hypothetical case study to illustrate an alternative value assessment framework to evaluate prevention choices for women at high risk of developing breast cancer.

Vakaramoko Diaby, PhD
College of Pharmacy, University of Florida

Askal Ayalew Ali, PhD
College of Pharmacy and Pharmaceutical Sciences, Institute of Public Health, Florida A&M University

Aram Babcock, PharmD, MS, MBA, PhD Student
College of Pharmacy, University of Florida

Joseph Fuhr, PhD
College of Pharmacy, University of Florida

Dejana Braithwaite, PhD
Health Cancer Center, University of Florida

Health Outcomes Research

2021 PREDOCTORAL FELLOWSHIPS IN HEALTH OUTCOMES RESEARCH

This predoctoral fellowship program seeks to increase the number of trained investigators studying outcomes associated with drug therapies by providing a stipend to students two years away from completing doctoral dissertations. Health outcomes research provides crucial information to doctors, patients, policymakers, and clinicians. The Foundation began awarding health outcomes research fellowships and grants in 2002.

Investing in Clinical Trials for Older Adults: The Value and Challenges of Older Adult-Specific Clinical Trials

"The predoctoral fellowship from the PhRMA Foundation gave me both financial support as well as emotional support. The Foundation's generous assistance helped alleviate concern about locating funding sources and made it possible for me to focus on the research questions that I am tackling."

Woojung Lee, PharmD
University of Washington School of Pharmacy

Understanding predictors of the low accrual in older adult-specific clinical trials and demonstrating the value of such trials, when successful, can help overcome the underrepresentation of older adults in trials. Aim 1 of this study is to identify trial-level predictors for low accrual in older adult-specific trials. Using AACT data, I will develop prediction models for low accrual based on traditional and machine-learning approaches. Aim 2 of the study is to quantify the realized value of older adult-specific trials, the AVEX and CALGB9343 study. A retrospective analysis of Surveillance, Epidemiology, and End Results (SEER)-Medicare data will be used to estimate the change in practice after the trial publications. State transition models will be developed to estimate the long-term outcomes of treatment studied in the trials. The realized value will be calculated based on these two estimates. By assessing the feasibility and estimating the potential value of older adult-specific trials, this study will facilitate investment and design decisions by funders.

Unpacking the Complexity of Diabetes Care Through Investigating Disease Control and Therapeutic Inertia Among Patients with Type 2 Diabetes

"Receiving the PhRMA Foundation Predoctoral Fellowship has allowed me to focus my time on successfully conducting my dissertation project and continually developing the skills needed for a career in health outcomes research."

Cassidi Crosby McDaniel
Auburn University Harrison College of Pharmacy

Diabetes management is complicated and changes over time. Approximately half of the people living with diabetes do not meet A1C targets for optimal glycemic control, leaving them at higher risk for diabetes-related complications. Optimal glycemic control involves routine assessments of patients' antihyperglycemic treatment regimens and intensification or initiation of additional antihyperglycemic medications as appropriate when A1C targets are not achieved. If there is a lack of antihyperglycemic medication intensification or initiation as appropriately indicated by guidelines, therapeutic inertia occurs. Investigation of glycemic control and therapeutic inertia in patients with type 2 diabetes will shed light on diabetes management. This

dissertation seeks to understand factors influencing glycemic control, to predict therapeutic inertia occurrence in clinical practice, and to examine the subsequent health outcomes after exposure to therapeutic inertia. This dissertation will be completed using real-world data to expand the evidence base for glycemic control and therapeutic inertia in people with type 2 diabetes. The public health significance of the dissertation is to optimize patients' diabetes care and management across their lifetimes and mitigate the occurrence of subsequent diabetes-related complications.

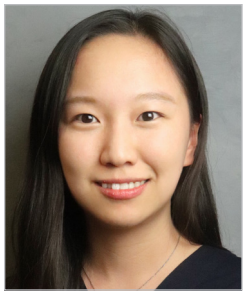
Trajectories of Medication Non-adherence with Time-Varying Predictors and Association with Clinical and Economic Outcomes: A Comparison of Classical Statistical Methods with Machine-Learning Algorithms

"This award provides me the opportunity to pursue a research project that I am truly passionate about. The contribution from the PhRMA Foundation will help identify the developmental perspective of how patients establish their medication adherence behavior patterns, while also identifying the life-changing events or time-varying predictors of medication adherence that can help health care professionals design targeted interventions."

Vasco M. Pontinha, PharmD
Virginia Commonwealth University School of Pharmacy

This study aims to 1) identify trajectories of medication adherence of chronic diseases treated with oral medications, and 2) distinguish the predisposing, enabling, need, and provider/care characteristics factors that determine trajectory membership using group-based trajectory modelling. Additionally, this study will investigate the association between adherence trajectories and economic and health outcomes. This association will be investigated by deploying two alternative predictive methods, one based on classic logistic regression and the other based on machine-learning algorithms. It is hoped that the findings of this study will elicit longitudinal medication adherence trajectories, the factors that determine trajectory membership, as well as establish optimal medication adherence trajectories based on the association with outcomes. Lastly, the conclusions of this study will allow health care professionals to identify patients at risk and payers to develop new value-based payments schemes based on medication adherence.

Neoadjuvant Versus Adjuvant Chemotherapy for Older Adults with Stage I-III Breast Cancer



"It is incredibly motivating to have received this recognition of my potential as I strive to become an independent and productive health outcomes researcher."

Hanxi "Molly" Zhang
University of Texas at Austin

Despite an increasing trend of neoadjuvant chemotherapy (NAC) use in breast cancer, evidence on benefits/risks of NAC — specifically in older women and within cancer subtypes – is limited, possibly contributing to the sizable variation in NAC use. This project aims to examine the effect of NAC versus adjuvant chemotherapy (AdC) alone on survival, health care utilization, and costs, in addition to assessing the temporal trends of NAC use and identifying factors related to NAC use among older women. The project will use the population-based Surveillance, Epidemiology, and End Results (SEER)-Medicare linked database to identify stage I-III breast-cancer patients. Subtype heterogeneity will be incorporated throughout, and propensity score matching is proposed to reduce bias. The research is expected to provide otherwise unavailable evidence on NAC in a large elderly population, which will inform patient-physician decision-making and ultimately improve clinical and economic outcomes.

2021 RESEARCH STARTER GRANTS IN HEALTH OUTCOMES RESEARCH

Scientists beginning independent research careers at the faculty level are eligible to receive funding for one year to study patient-centered outcomes, data, systems, and technologies for understanding and improving the effectiveness of pharmaceutical interventions.

The Role of Mild Cognitive Impairment and Alzheimer's Disease and Related Dementias (ADRD) in the Utilization of High- and Low-Value Health Care

"The Research Starter Grant from the PhRMA Foundation was a critical boost to important research on the value of care for people with cognitive impairment. This support has allowed me to accelerate my research program in this area and grow as a leader in studying the connection between health policies and the chronic conditions that define healthy aging."

Douglas Barthold, PhD
University of Washington School of Pharmacy

Utilization of low-value (LV) care and under-utilization of high-value (HV) care is a major problem for older adults. Cognitive impairment (CI), including both mild cognitive impairment (MCI) and Alzheimer's disease and related dementias (ADRD), is prevalent for older adults and likely affects HV and LV utilization. Patient harm and unnecessary costs of LV replacing HV care could be especially high and widespread in this vulnerable subpopulation, but these relationships have not been studied. The Centers for Medicare and Medicaid Services (CMS) has called for value promotion, but policies cannot be adequately targeted and implemented without understanding the aforementioned relationships. Therefore, we propose to close this gap by identifying the association between CI and utilization of LV and HV care. Results will depict trajectories of utilization of relevant services for individuals at different stages of CI and inform policies to improve patient welfare and promote healthy aging.

Studying the Drivers of Biosimilar Coverage by Commercial Plans and Estimating the Social Welfare Gain from Biosimilar Entry in the United States

"Receiving the support from the PhRMA Foundation has allowed me to build a research team consisting of three doctoral researchers and to obtain datasets that we would otherwise not have had access to. The award has accelerated our work on multiple fronts, which we hope will make significant contributions to our understanding of the biosimilar market in the United States."

Jakub P. Hlávka, PhD
University of Southern California Price School of Public Policy

Biosimilars are an equivalent of generic medications for infusion therapies, such as those used in oncology or neurology, and have great potential to lower prices and increase access to otherwise expensive treatments. The first aim of our research is to explore and examine key factors associated with coverage decisions regarding biosimilars that have been approved by the U.S. Food and Drug Administration (FDA) and have become available on the U.S. market, drawing on unique data provided by the Tufts Cost-Effectiveness Analysis Registry and the Specialty Drug Evidence and Coverage Database. Our second aim is to study the social welfare gain from biosimilar entry in the U.S. health care system. By using the SSR Health US Brand Rx Net Pricing data, we will estimate the additional number of patients receiving access to therapy following biosimilar entry, thus enabling us to quantify the extensive margin. We will use data on average net (post-rebate) prices by quarter to calculate the effect of biosimilar entry on the average cost of treatment (intensive margin). Our research will help clarify what drives the prices and utilization of biologics and what determines their coverage by insurance plans in the United States.

Associations Between Household Polypharmacy and Adherence to Medications for Substance Use Disorders and Health Outcomes

"The PhRMA Foundation Research Starter Grant in Health Outcomes Research has been instrumental in launching my career as an independent scientist by providing support to acquire health insurance claims data to conduct pharmacoepidemiologic studies of substance use disorder treatment."

Marissa Seamans, PhD
University of California, Los Angeles, Fielding School of Public Health

Medications for opioid use disorder (OUD) and alcohol use disorder (AUD) have the potential to improve the health and well-being of more than 2.1 million Americans with OUD and 14.1 million with AUD; however, long-term adherence to these medications is alarmingly poor. Household availability of opioids, benzodiazepines, and other medications with high risk of misuse may be risk factors for poor treatment adherence in patients with substance use disorders (SUD) but have not been explored. This research aims to 1) develop prediction models to identify patients prescribed medications for SUD at risk of suboptimal adherence, and 2) determine whether household polypharmacy is associated with poor medication adherence and substance use-related adverse events among commercial insurance and Medicaid beneficiaries. This project will fill a critical gap in our understanding of whether medication use by family members influences SUD medication utilization and outcomes, and will provide evidence to inform and tailor interventions to improve SUD medication adherence in these populations.

Translational Medicine

2021 POSTDOCTORAL FELLOWSHIPS IN TRANSLATIONAL MEDICINE

This fellowship program supports multidisciplinary research aimed at bridging the gap between experimental and computational or laboratory research and real-world clinical work. 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 Foundation awarded the first translational medicine fellowships and grants in 2013.

Hypoxia as a Driver of the Metastatic Melanoma Glycome and its Immunoevasion Capacity

"The PhRMA Foundation Postdoctoral Fellowship has allowed me to pursue a new direction in my project focused on harnessing the metastatic melanoma glycome for improving therapy response in patients."

Asmi Chakraborty, PhD
Florida International University Herbert Wertheim College of Medicine

Metastatic melanoma (MM) has a dismal 5-year survival rate of 25%. Hence, there is a dire need for biomarkers to predict metastasis and response to immune checkpoint inhibitor (ICI) therapy. Our laboratory discovered that altered MM cell surface sugar structures, glycans, formed by a loss of glycosyltransferase, GCNT2 induce MM growth. Since low tissue oxygen (hypoxia) drives MM growth and resistance to ICI therapy, we hypothesize that MM glycans are evoked by hypoxia and confer ICI resistance. Exciting preliminary data suggest that hypoxia lowers GCNT2 and increases melanoma-initiating cells and MM binding to pro-metastatic galectin (Gal)-3. Further, low GCNT2 correlates with poor MM patient survival and a reduction in ICI response. Studies in this grant are poised to identify novel treatment approaches to improve ICI therapy response based on the identification of unique hypoxia-driven MM glycans and their interaction with Gal-3. Results will reveal glycans as biomarkers of MM and implicate Gal-3 targeting to improve ICI efficacy.

Advancing the Repurposing of Lapatinib to Combat COVID-19, Future Pandemic Coronaviral, and Other Emerging Viral Infections

"The PhRMA Foundation Postdoctoral Fellowship will have a fundamental impact on my career path. As a woman scientist from a developing country, I recognize the impact of role models on empowering students to pursue careers in science. This fellowship will help me to be an independent researcher and hopefully transition into a tenure-track faculty position at an academic institution. I am very honored and grateful to the PhRMA Foundation for this support."

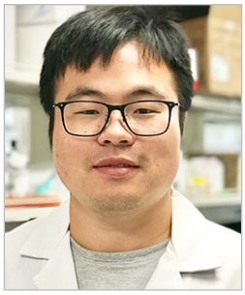
Marwah Karim, PhD
Stanford University School of Medicine

The COVID-19 pandemic caused by severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) has killed millions of people worldwide and has caused global public health emergencies. Although there are effective vaccines available to reduce COVID-19 severity, there is no effective treatment option available to treat the patients who are already infected with the virus. Most of the existing antiviral drugs target viral enzymes, typically providing a "one drug, one bug" approach that can lead to the emergence of viral resistance. Moreover, addressing viruses individually uses resources inefficiently and is not easily

scalable to meet the large unmet need. Our goal is to overcome these challenges by developing host-targeted broad-spectrum antivirals that would provide protection against SARS-CoV-2 and readiness for future coronavirus outbreaks. We have recently discovered lapatinib, an approved anti-cancer epidermal growth factor receptor (ErbB) 1/2/4 inhibitor, that suppresses replication of SARS-CoV-2 and other unrelated viruses in human cell lines as well as in unique human lung organoid models with a high barrier to resistance. ErbB1/2 are known as key regulators of acute lung injury and fibrosis, and their inhibition protects mice from lung injury and mortality. We hypothesize that Pan-ErbB inhibition is a safe strategy, which not only suppresses viral infection, but also reduces inflammation, acute lung injury, and fibrosis by inhibiting ErbB-mediated signaling.

The results of this project will contribute significantly to the knowledge of virus-host interactions critical for SARS-CoV-2 infection and pathogenesis in human lung organoid and animal models. If successful, our project will advance lapatinib, as a key therapeutic component into clinical studies in hospitalized patients with the goal of not only reducing viral replication but also preventing lung injury and long-term fibrosis.

Targeting IGF2BP2 as New Therapy in MLL-Rearranged Acute Myeloid Leukemia



"The PhRMA Foundation fellowship has provided great academic and financial support, which will help me to forge ahead as a postdoctoral researcher in the field of leukemia translational medicine. The funding greatly advanced our translational research, which is aimed at developing new therapeutic strategies for MLL-rearranged acute myeloid leukemia. I have reaped great experience, which will benefit my career."

Wei Li, PhD
Beckman Research Institute, City of Hope

Among all acute myeloid leukemia (AML) cases, mixed-lineage leukemia (MLL) rearranged AMLs are related to poor treatment outcomes overall. Thus, it is urgent to identify new therapeutic targets and then develop more effective novel therapeutics accordingly. IGF2BP2 has been reported as one of the genes that overlap in overexpression and hypomethylation in MLLr leukemia. As a newly identified N6-methyladenosine (m6A) "reader," IGF2BP2 could promote target-mRNA stability and translation. Our data showed IGF2BP2 specially overexpressed in MLLr AML, suggesting its critical oncogenic role and the therapeutic potential as a target to treat MLLr AML. Thus, we developed a potent IGF2BP2 inhibitor (CWI1-2) that exhibits high anti-leukemia efficacy. The results of this project will contribute significantly to the knowledge of MLLr AML development and maintenance, and therefore improve clinical outcomes in MLLr AML patients. If successful, our inhibitor will advance the current therapeutic strategies in treating MLLr AML in the future.

2021 RESEARCH STARTER GRANT IN TRANSLATIONAL MEDICINE

This program supports individuals beginning independent research careers at the faculty level in academia or research institutions, where long-term training is an expected outcome in conjunction with research. This program supports researchers integrating experimental and computational technologies or laboratory research with real-world clinical research outcomes. The program is focused on innovative and collaborative projects that bridge the non-clinical/clinical interface.

Role and Regulation of Cholesterol-Associated GWAS Locus EHBP1 in NASH

"The PhRMA Foundation Research Starter Grant has helped me to build an attractive study for competing for extramural funding."

Bishuang Cai, PhD
Icahn School of Medicine at Mount Sinai

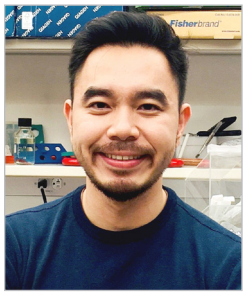
Due to the incomplete understanding of the mechanisms of nonalcoholic steatohepatitis (NASH) progression, effective treatment options for NASH are lacking. Emerging experimental and human evidence has revealed that liver cholesterol accumulation is critical for the pathogenesis of NASH. However, how cholesterol is regulated in NASH remains unknown. Human genome-wide association studies (GWAS) have determined that several single-nucleotide polymorphisms in EH domain binding protein 1 (EHBP1) are associated with cholesterol levels, and our data show that EHBP1 expression is reduced in human and mouse NASH livers, which can be reversed by the administration of pro-resolving mediators. The objective of this proposal is to explore the role of EHBP1-mediated cholesterol metabolism in NASH and the mechanism of down-regulated EHBP1 during NASH. The results of this project will contribute significantly to the knowledge of NASH progression and advance a key therapeutic component that will meet the clinical need for novel NASH therapies.

Drug Discovery

2021 PREDOCTORAL FELLOWSHIPS IN DRUG DISCOVERY

These fellowships help build a pipeline of highly trained pharmaceutical researchers by providing awardees with a two-year stipend as they progress in their doctoral dissertations focused on drug discovery. The Foundation has been awarding fellowships and grants in drug discovery (which includes the fields of pharmacology and toxicology) since 1978.

Targeting Chromosomal Instability and Epigenetics to Halt Cancer Metastasis



"Receiving the PhRMA Foundation Predoctoral Fellowship has helped tremendously in my thesis research, as well as to push the envelope in my scientific approaches to decipher unique mechanisms of cancer initiation and adaptation and potentially target them."

Albert S. Agustinus
Joan & Sanford I. Weill Medical College of Cornell University

Chromosomal instability (CIN) is a hallmark of aggressive human cancers. Currently, there are no available drugs that target CIN, despite its well-documented role as a driver of cancer metastasis and therapeutic resistance. CIN provides cancer cells with genetic heterogeneity, due to constant shuffling of their genomic makeup. Another major way cancer cells resist treatment and adapt to their environment is by epigenetic reprogramming. This allows cells to alter their timing and level of gene expression, enabling them to utilize pathways used in normal tissue development to acquire stem-like properties that facilitate metastasis and therapeutic resistance. Our preliminary finding revealed, for the first time, an interconnection between CIN and epigenetic reprogramming. Chromosomally unstable cells produce micronuclei, due to chromosome mis-segregation during anaphase. We found that histone post-translational modifications (PTMs) were differentially enriched in the micronuclei of cancer cells, which have significant impact on their gene expression if the PTMs are reincorporated to primary nucleus upon micronuclei reintegration. On the other hand, we also found that chromosomally unstable cells tend to have increased stemness through epigenetic reprogramming by histone 3 lysine 27 trimethylation (H3K27me3) installed by enhancer of zeste homolog 2 (EZH2). We plan to unravel the mechanistic basis of these newfound relationships and exploit them to develop the first CIN-suppressive treatment (Aim 1). To counteract CIN, nature has developed a fail-safe mechanism by altering the dynamics of kinetochore-associated microtubules (kMTs), which are defective in cancer. We will test a panel of first-in-class small molecule compounds that have been shown to rescue this mechanism in otherwise aggressive cancer cells (Aim 2). As a whole, this project addresses an unmet need for the development of a first-in-class drug and therapeutic strategies to suppress CIN in human cancers.

Parkinson's Disease Therapies Targeting GUCY2C

"Receiving a fellowship from the Foundation has allowed me to pursue our lab's interest in the gut-brain axis within the context of Parkinson's disease. This award has opened the door to utilizing the latest techniques, equipment, and analyses to gain a holistic understanding of the molecular mechanisms driving mitochondrial dysfunction and neurodegeneration."

Lara Cheslow
Thomas Jefferson University

Parkinson's disease (PD) is the second most common cause of age-related neurodegeneration in the United States. In PD, mitochondrial dysfunction within dopaminergic (DA) neurons of the substantia nigra (SN) induces DA neuron death, causing subsequent DA depletion and motor dysfunction. Current therapies for PD patients raise DA levels to temporarily relieve motor symptoms, but do not prevent further DA neurodegeneration or slow disease progression. Thus, there is an unmet need to develop novel therapies that protect DA neurons to treat PD. Guanylyl cyclase C (GUCY2C) is the intestinal and neural receptor for uroguanylin (GUCA2B), a hormone produced primarily in the small intestine. Within the intestine, GUCY2C-GUCA2B signaling protects mitochondrial function, and disruption of this signaling axis, reflecting the loss of hormone GUCA2B, is central to gastrointestinal cancer, autoimmunity, and toxic injury. More recently, neural GUCY2C – expressed in the hypothalamus and SN – and intestinal GUCA2B have emerged as key players in novel gut-brain signaling axes. In the hypothalamus, where GUCY2C is expressed by neurons regulating appetite, we have demonstrated that GUCY2C-GUCA2B signaling promotes healthy satiety. However, the role that this gut-brain axis plays in the SN, where GUCY2C is expressed by DA neurons that are lost in PD, remains undefined. Our preliminary studies suggest that the GUCY2C-GUCA2B axis protects DA neurons against cell death through promoting mitochondrial health. Our proposed research seeks to explore this signaling axis in greater depth and to investigate the potential of medication that stimulates neural GUCY2C to protect DA neurons from further degeneration in PD patients. The potential to translate these studies into new drugs to treat and prevent PD can be appreciated by considering that the GUCY2C ligands linaclotide and plecanatide are FDA-approved to treat constipation.

Apolipoprotein Mimetic Peptides for the Treatment of Niemann-Pick Diseases

"Receiving a predoctoral fellowship from the PhRMA Foundation has been a tremendous benefit because it has connected me to other fellowship recipients and enabled me to spend more time working on experiments for my dissertation project."

Troy Halseth
University of Michigan

Niemann-Pick Disease types A (NPA) and B (NPB) (together referred to as acid sphingomyelinase deficiency [ASMD]) are rare, fatal disorders of sphingomyelin metabolism with no FDA-approved treatments. ASMD occurs when an enzyme called acid sphingomyelinase is not able to efficiently breakdown a lipid called sphingomyelin. When this lipid builds up inside cells throughout the body, it compromises the integrity of the cell membrane and the ability of our cells to respond to stress. The clinical manifestations of ASMD are quite severe, as patients experience enlargement of peripheral organs (mainly the liver and spleen) and, in the worst cases, neurological deterioration, resulting in death in early childhood. Enzyme replacement and gene therapy have been studied for the treatment of ASMD, but these strategies are unable to get into the brain and address the neurological phenotypes without direct administration to the central nervous system. Because of this, it is important to investigate additional strategies for the treatment of ASMD. Our lab has studied several therapeutics that mimic high-density lipoproteins, which are nanoparticles found naturally in the body responsible for transporting excess lipids and cholesterol from throughout the body back to the liver to be metabolized. We have tested these therapies in cells from patients with ASMD and discovered their

ability to remove excess sphingomyelin from these cells. This represents a new therapeutic strategy for addressing the sphingomyelin accumulation resulting from ASMD and the associated disease pathologies. We have also noted the ability of this treatment to mobilize sphingomyelin into circulation following administration in a mouse model of ASMD. Based on these findings, the potential of apolipoprotein mimetics as a therapeutic strategy for ASMD warrants further investigation.

G Protein Coupling and Trafficking of Dark Orphan GPCRs

"PhRMA Foundation support has been fundamental to my MD/PhD training. The Foundation's Predoctoral Fellowship in Drug Discovery has provided me an invaluable opportunity and support to explore and identify the function of understudied orphan G protein-coupled receptors, some of which may be targetable for central nervous system disorders."

Wonjo Jang
Augusta University Research Institute, Inc.

G protein-coupled receptors (GPCRs) are versatile 7-transmembrane proteins that are targets of one-third of approved drugs. However, of 360 non-sensory GPCRs, only about 100 are targeted by FDA-approved therapies. Furthermore, more than 100 are so-called orphan receptors (oGPCRs) whose endogenous ligands, and often physiological roles, are unknown. The importance of their as-yet untapped resource for drug discovery is evident by current efforts in Illuminating the Druggable Genome (IDG) project to characterize oGPCRs' molecular and physiological roles. Despite a growing number of large-scale studies revealing the GPCR-transducer coupling landscape for well-studied GPCRs, the oGPCR-G protein landscape remains unexplored. The critical barriers to studying oGPCR function include the limited availability of known ligands as well as inefficient receptor trafficking to the cell surface. The main goal of this project is to develop a robust and efficient assay platform that deorphanizes the transducer coupling profile of oGPCRs for which no ligand is available and monitors their subcellular trafficking. Our approach will capitalize on our laboratory's well-established bioluminescence resonance energy transfer (BRET)-based assays, as well as tools borrowed from structural studies, such as nucleotide depletion, engineered G protein surrogates, and mutagenesis-driven receptor activation. Mapping the subcellular distribution of oGPCRs will be carried out by assessing BRET between receptors and fluorescent protein-tagged membrane compartment markers. Successful completion of this project will advance our understanding of how oGPCRs signal and traffic, which in turn will also guide further efforts in identifying oGPCR agonists and antagonists.

Pharmacologic Inhibition of the Glycolytic Pathway Improves Response to Immune Checkpoint Blockade

"The PhRMA Foundation has been instrumental in providing me with the resources required to push forward my research on cancer drug targets to combat resistance to immunotherapies. I'm privileged to represent a foundation focused on innovative science that directly translates to improving the lives of patients."

Svena Verma
Memorial Sloan Kettering/Weill Cornell Graduate School of Medical Sciences

Harnessing the immune system to battle cancer using immune checkpoint blockade (ICB) has revolutionized cancer treatment for many patients. However, a significant proportion of patients are resistant to these therapies, often due to a hostile tumor microenvironment. This microenvironment is unfriendly to anti-tumor immune cells, such as effector T cells, rendering immunotherapies less effective. One factor contributing to an immunosuppressive tumor microenvironment is the overconsumption of glucose and overproduction of lactic acid by tumor cells. This overreliance on glucose metabolism by tumor cells spares less glucose for effector T cells to consume, and the excess lactate is detrimental to effector T cell function. Therefore, we proposed

combining immunotherapy with an inhibitor of key enzyme lactate dehydrogenase (LDH), which catalyzes the final step of glucose metabolism, to alleviate glycolysis-mediated immunosuppression in the tumor microenvironment. Cancer patients with an elevated serum LDH level tend to have a lower probability of survival, and we observed that serum lactate and LDH levels correlate with primary tumor burden in mice. We recently demonstrated that genetic dampening of LDH in a mouse-model of breast cancer results in improved and long-lasting anti-tumor responses to CTLA-4 blockade in mice. We found that administration of a small molecule inhibitor of LDH reduces tumor lactate production and glucose consumption without inhibiting anti-tumor T-cell killing. When administered to mice, the LDH inhibitor has a slight anti-tumor effect on its own, and when combined with immune checkpoint (CTLA-4) blockade, LDH inhibition is more effective in slowing melanoma growth than immunotherapy alone. We have shown that this combination therapy enhances effector T cell function, while destabilizing the function of pro-tumorigenic immune cells in the tumor microenvironment. This study provides foundational, mechanistic research that will inform the use of metabolic inhibitors alongside immunotherapies in clinical trials to combat resistance to immunotherapy and improve cancer patient outcomes.

2021 POSTDOCTORAL FELLOWSHIPS IN DRUG DISCOVERY

This fellowship provides a two-year stipend to early-career postdoctoral scientists seeking to gain new skills in areas relevant to drug discovery. Eligible candidates are actively pursuing a multidisciplinary research training program to enhance their expertise and education or embarking on a research project focused on understanding a drug's molecular or cellular mechanisms of action with the agent's effects with the ultimate intention of improving human health.

Reducing Tau Propagation by Inhibiting Extracellular Vesicle Biogenesis

"The PhRMA Foundation Postdoctoral Fellowship uniquely enabled my growth as an independent scientist by providing the funding and support to more deeply pursue a branch of research that would otherwise be passed over. Our findings are important in the field of Alzheimer's research, and I hope that one day this work will have a meaningful impact for patients around the world."

Benjamin Bell, PhD
Johns Hopkins University School of Medicine

The personal and medical costs of Alzheimer's disease (AD) are among the highest in the world and continue to steadily rise; yet there are no effective treatments available. Results from recent clinical trials targeting the buildup of a toxic protein in the brain, amyloid-ß (Aß), have been disappointing, highlighting the need for a new approach. Our lab has turned attention to small particles that help spread another diseased protein, Tau, across the brain. We are developing a new drug, called PDDC, which prevents the production of these carrier particles, with the intention of stopping or slowing the damaging progress of Tau propagation. In this proposal, we aim to determine the efficacy of PDDC in a mouse model of AD, while simultaneously developing a new and predictive blood biomarker, a serious unmet need in the field. This research will advance our understanding of the mechanisms that underlie AD as well as aid in development of a novel treatment option.

Selection of Noncanonical D-Peptidomimetics Against Gram-Negative Pathogens

"The PhRMA Foundation Postdoctoral Fellowship in Drug Discovery has been incredibly helpful in providing me independence to explore interesting topics and method development to prepare me for an independent career."

Joseph S. Brown, PhD
Massachusetts Institute of Technology

Antibiotics are a cornerstone of modern-day medical care that continue to be challenged by bacterial resistance and exacerbated by decreased antibiotic development. Untreatable or resistant bacterial infections can become lethal, harkening back to a time before the discovery of efficacious antibiotics (e.g., penicillin). If unabated, antibiotic-resistant bacteria are projected to kill more people than cancer, heart disease, or diabetes in 2050. Thus, the development of new antibiotics is urgent. One of the most significant clinical threats comes from Gram-negative bacteria, due to their high rates of multi-drug resistance and virulence. Gram-negative antibiotic development is challenging, primarily because of the highly impenetrable cell-wall barrier, which in a variety of ways has single-handedly thwarted and limited the development of research. For this reason, Gram-negative antibiotic development must directly address the challenge of the cell-wall barrier. In our work, we will identify new therapeutics that potently antagonize outer membrane proteins on Gram-negative bacteria that are essential for their metabolism. We will kill these bacteria without needing to overcome the challenge of crossing the cell wall. Some peptide-like molecules, called peptidomimetics, have been discovered to work through this mechanism, but have only a modest binding affinity to their outer membrane target. Because of this, we hypothesize more potent peptidomimetics can be discovered and developed if they have a higher affinity. To discover these new molecules, we will use a technique called affinity selection that isolates high-affinity peptidomimetics to the outer membrane target protein from large chemically synthesized libraries containing hundreds of millions of compounds. Individual synthesis and testing of these molecules will establish the relationship binding affinity and potency and reveal their potential as new antibiotic candidates. Other outcomes of this work will potentially establish design rules for the intracellular penetration of peptidomimetics across bacterial membranes through similar discovery methods of affinity selection coupled to machine learning.

2021 RESEARCH STARTER GRANTS IN DRUG DISCOVERY

This program supports early-career faculty members with a one-year award to help launch independent research careers. This grant aims to assist academic scientists developing, exploring, or optimizing drug therapies.

Characterization of New Inhibitors of P-Tau Aggregation and Cytotoxicity

"Research Starter Grant funding allowed me to secure a tenure-track position at Purdue University and recruit a postdoc in medicinal chemistry. It also expanded my research program for the discovery of new molecules with anti-aggregation effect for Alzheimer's disease. It will enable my team to publish important results and apply to other research funding opportunities."

Jessica Fortin, DVM, PhD
Purdue University

Alzheimer's disease (AD) represents the most common form of dementia, characterized by intracellular neurofibrillary tangles (NFTs), composed of microtubule-associated tau-protein, and extracellular β-amyloid plaques, made of B-amyloid peptide fragments 1-40 and 1-42. Despite significant efforts in past decades, no disease-modifying therapy has been available to prevent or cure the progression of the disease until now. Due to the rapidly increasing aging population worldwide, there is an urgent need to identify treatment strategies that can solve this challenge. Abnormally hyperphosphorylated tau is considered an early and pivotal point in the pathogenesis of AD and other tauopathies. Compelling evidence suggests that it is a key driver in the accumulation of NFTs and can be directly correlated with the extent of dementia in AD patients. Therefore, inhibiting the abnormal tau hyperphosphorylation induced aggregation can be a viable strategy to develop newer therapeutics targeting AD. Our lab focuses on the discovery of small molecules to stop the aggregation of p-tau responsible for the NFTs in AD.

Harnessing Natural Killer Cells to Treat High-Risk B- and T-Cell Acute Lymphoblastic Leukemia

"I am grateful to the PhRMA Foundation for providing me the first source of extramural funding after I established my independent lab. The Research Starter Grant has been instrumental in enabling my young team to develop novel applications of natural killer cell-mediated immune surveillance for diagnosis and treatment of acute lymphoblastic leukemia."

Srividya Swaminathan, PhD
Beckman Research Institute, City of Hope

The immune system is the body's natural defense against cancer. A type of immune cell that eliminates cancer is the natural killer (NK) cell. The goal of our research is to develop NK cells as therapies for blood cancers termed B- and T-cell acute lymphoblastic leukemia (B/T-ALL). Unfortunately, about 60% of adults and about 15% of children with B/T-ALL do not respond to conventional treatments because leukemic cells abnormally express high levels of certain cancer-causing proteins that reduce NK cell number and function. In this proposal, we will test the hypothesis that administering NK cells is a viable strategy to prevent disease recurrence in patients with B/T-ALL. We will employ patient-relevant approaches: 1) identify the defects in surveillance of adult and pediatric B/T-ALL by NK cells; 2) using "humanized" mice developed by us, delineate how human ALL cells suppress human NK cells; and 3) engineer NK therapies that cannot be suppressed by leukemia cells. Our studies will inform the development of diagnosis and treatment approaches using NK cells and improve the quality of life of people with B/T-ALL.

Drug Delivery

2021 PREDOCTORAL FELLOWSHIPS IN DRUG DELIVERY

These fellowships assist students engaged in dissertation research on breakthrough drug delivery modalities, such as gene therapy, and the relationships between drug delivery systems and clinical applications. These stipends are provided once PhD candidates begin their dissertation research after they've completed their coursework. The PhRMA Foundation began funding fellowships and grants in drug delivery (formerly known as pharmaceutics) in 1972.

Cellular Networks for Enhanced Drug Retention and Accumulation at Target Site

"This award has allowed me to focus solely on my dissertation research, engineering novel and translatable drug carriers for the treatment of myocardial infarction. This network, and the funding it provides, have been invaluable in my development as an independent investigator."

Natalie Jasiewicz
University of North Carolina at Chapel Hill

Ischemic heart disease is the leading cause of death worldwide. Patients often experience dysregulated wound-healing and become at risk for long-term heart failure and poor cardiac function. Mesenchymal stem-cell-based therapies have been applied with only limited success and typically require invasive intracardiac injections. Furthermore, cell viability of these treatments is low and have poor retention at the infarct site. Therefore, the ability to administer a therapeutic that is minimally invasive but remains viable and localized at the infarct site is desperately needed. To address the inefficiencies of current therapies, we propose to develop a living drug depot that is composed of an in situ forming network of cells. When surface-decorated with heterodimerizing leucine zippers, these cells can be intravenously injected where they will crosslink to create a scaffold-free network that can remain at the infarct site, with consistent exosome release to promote cardiac tissue regeneration. This work will be the first to investigate a novel scaffold-free network of mesenchymal stem cells to create an injectable, in situ forming network with enhanced retention that can be used for the treatment of myocardial infarction.

Elucidating the Tropism of Cell-Specific RNA Nanoparticles and Consequences for Anti-Cancer Efficacy

"The PhRMA Foundation Predoctoral Fellowship support has helped fund my dissertation research and has helped me build confidence in my ability to succeed in a scientific career within the drug delivery field."

Lindsay Johnson
University of Texas Southwestern Medical Center

When the liver becomes injured or its cells are not functioning properly, it becomes inflamed and progresses towards disease and eventually cancer. A promising treatment approach is to use gene therapy, which requires the use of nanoparticles made up of chemical materials that work together to both carry and release a gene therapy to a targeted location. MicroRNA (miRNA) therapy is a promising approach that can directly inhibit oncogenes that drive cancer; however, the use of microRNAs as liver-cancer therapy has been challenged by unexpected nanoparticle-induced stimulation of immune cells, including Kupffer cells of the liver, resulting in adverse events. Clinical trial failures for miRNA therapy have illustrated the importance of understanding cell-level delivery of gene therapies. This project aims to understand cell-specific uptake

of nanoparticles for gene therapy in the liver and develop the knowledge for creation of nanoparticles that enable liver cancer therapies to reach more patients. The project will focus on approaches to elucidate to which liver cell types our laboratory's established nanoparticle formulations deliver RNA and explore anti-cancer efficacy. Currently, liver cancer patients have few options that only minimally extend survival. As cancer progresses, patients are no longer candidates for the current treatments due to their compromised liver function. If one is able to deliver the therapy to specific liver cells, this will help reduce toxicity and open up new treatment avenues. Completion of this project will help explain how the chemistry of nanoparticles affect their cell specificity within the liver and provide the necessary knowledge to advance the field of nanoparticle-based liver cancer therapy.

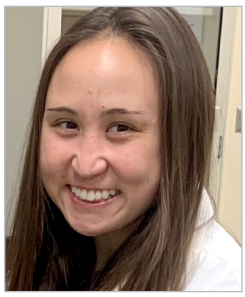
Dual Anti-CD47 and Duramycin Functionalized Carriers for Radio-Enhanced Drug Delivery

"Receiving a fellowship from the PhRMA Foundation has allowed me to continue pursuing my career goal of becoming a research scientist. Analytical techniques that strengthen my hypotheses are now accessible, and I will benefit from the PhRMA Foundation network professionally for years to come. The Foundation's support has also been a great benefit, overall, to our relatively small PhD program."

Madeleine Landry
Oregon State University

The majority of patients diagnosed with cancer undergo chemotherapy, and usually for a prolonged period with multiple cycles. Chemotherapy is toxic to healthy cells as well as cancerous cells, and is injected in the bloodstream, leading to exposure to healthy cells. This results in unbearably painful side effects for patients. Because of the toxicity, many patients must end chemotherapy prematurely before finishing the cycles. However, many clinically approved nanoformulations have decreased the side effects of traditional chemotherapy and increased the dose that reaches the cancerous cells, allowing for lowered dose of drugs in some cases. Nanoformulations can act to shield the drug from healthy cells with triggered release in the cancerous cells, have targeting moieties that increase accumulation at the tumor, can increase the circulation time in the blood, or a combination of the above. We propose encapsulating the chemotherapy in a nanoparticle surface modified with targeting moieties to increase localization to the tumor by targeting radiated tumor tissue. Radiation therapy is often prescribed alongside chemotherapy and uses a highly focused beam. X-ray radiation leads to an increase in certain cell surface markers in the radiated cells, including lipid phosphatidylethanolamine and protein CD47, that we can take advantage of to specifically localize the nanoformulations encapsulating the chemotherapy to the tumor. Our approach has the potential to decrease off-target effects that afflict patients during systemically delivered chemotherapy, making multiple treatment cycles more tolerable for them.

Non-Invasive Brain Delivery, Clearance, and Efficacy of Therapeutic Antibodies in Mice



"I am very honored to be a part of this prestigious fellowship. The funding provided by the PhRMA Foundation has supported my research efforts to enhance the delivery of antibodies to the brain."

Kelly M. Schwinghamer
University of Kansas

The blood-brain barrier (BBB) presents a formidable obstacle to the delivery of therapeutic agents to the brain. It is estimated that only 2% of current drugs can enter the brain from the systemic circulation. This renders the brain nearly inaccessible to large molecule therapeutics, such as neurotrophic factors, enzymes, and monoclonal antibodies (mAbs). An alternative for delivery of large molecules to the brain is drilling a hole into the skull to bypass the BBB. Thus, there is an urgent need to develop non-invasive methods to deliver drugs to the brain. The high binding specificity and affinity of mAbs make them attractive and promising drug candidates for treating a variety of brain diseases, such as Parkinson's and Alzheimer's disease (AD), as well as brain tumors. However, the need for non-invasive delivery of mAbs limits their use in the brain. To facilitate non-invasive transport of molecules across the BBB, the Siahaan group has designed synthetic small peptide modulators (e.g., ADTC5, HAV6) that temporarily and reversibly increase the porosity of the BBB by disrupting or modulating the "Velcro" molecules between cells of the BBB. These modulators allow mAbs to penetrate between cells of the BBB and enter the brain tissue. This project is aimed at studying the efficiency of the modulators (i.e., ADTC5 peptide) to deliver mAbs into the brains of mice. The mAb distribution and clearance from the brain and other organs over time will be evaluated to see the efficiency of our modulator to enhance delivery of mAb to the brain. Finally, the therapeutic efficacy of an mAb for AD treatment will be evaluated with the brain delivery enhancement of the mAb using ADTC5 modulator peptide compared to that of the mAb alone. The effect of mAb in the brain will be evaluated by examining the cognitive performance and brain microstructure of mice treated with mAb+ADTC5 compared to those of mAb alone, ADTC5 alone, or vehicle-treated mice as controls. It is expected that mice treated with mAb+ADTC5 will improve cognitive performance and reduce brain pathology compared to those treated with controls. The results from these studies will provide a proof-of-concept that our method can be used to improve treatments of brain diseases.

2021 POSTDOCTORAL FELLOWSHIP IN DRUG DELIVERY

These fellowships support qualified doctoral graduates seeking to further develop and refine their pharmaceutics research skills through formal training. The program encourages researchers to continue learning scientific skills in drug delivery and to build careers focused on addressing challenges in this important area.

Atomic Layer Deposition to Improve Stability of Amorphous Pharmaceutical Materials

"This award has enabled me to conduct cutting-edge research into a novel manufacturing technology while engaging in professional development opportunities, preparing me for the next stage of my career."

Dana E. Moseson, PhD
Purdue University

Product stability is required to transform a molecule into a medicine for safe and efficacious delivery to patients. Amorphous materials are advantageous to improve solubility and dissolution rate of poorly soluble compounds; however, they have a thermodynamic driving force for crystallization. Upon recrystallization, the solubility advantage is lost,

which is highly detrimental to bioavailability. Atomic layer deposition (ALD) is a layer-by-layer technique in which a nanoscale film of biocompatible metal oxide (commonly <10-100 nm) is applied to the surface of a substrate with excellent control of conformality and film thickness. The overall goal of this research is to identify the mechanistic factors required for successful implementation of ALD coatings for improved physical stability of amorphous pharmaceutical products. First, we will demonstrate that ALD coatings can improve physical stability of amorphous materials (i.e., inhibit surface crystallization). Model systems were selected to vary their chemistry to evaluate the mechanisms by which the ALD coating can improve physical stability. Next, we will examine performance attributes of ALD-coated particles and formulations: 1) dissolution performance of coated and uncoated particles and 2) physical stability following compression. Successful completion of this research will lead to enhanced understanding of ALD technology applications within pharmaceutical drug delivery to address product stability questions.

2021 RESEARCH STARTER GRANTS IN DRUG DELIVERY

These grants support scientists who are beginning their academic research careers at the faculty level without other funding sources. These grants ensure that the promising drug delivery-focused work of these researchers continues.

Stem Cell Delivery in Tissue Matrix Carrier for Spinal Cord Injury Repair

"I am honored and grateful for support from the PhRMA Foundation of my lab through the Research Starter Grant in Drug Delivery. This award has allowed my lab to initiate an exciting new line of research on developing combinatorial drug delivery platforms for spinal cord injury repair. I am excited at the prospect of making real contributions to improving health care through a clinically translatable therapeutic system."

Young Hye Song, PhD
University of Arkansas

Spinal cord injury (SCI) is a traumatic injury that results in a permanent loss of sensory and motor function of the affected people. This permanent loss of sensory and motor functions comes from the fact that spinal cord tissues create holes, which are eventually filled with scars that do not heal damaged neurons. Effective treatment options for SCI are lacking because of the complex landscape of the scar tissues formed after spinal cord injury. As such, new drug delivery strategies are needed for effective treatment to restore damaged tissues and promote functional recovery. To this end, we aim to develop a new drug delivery system for SCI repair using stem cells and polymeric carriers. Stem cells are essentially living drug depots particularly attractive for SCI repair, as they secrete various kinds of proteins that promote tissue repair and functional recovery. Currently, one of the main challenges in delivering stem cells to the sites of injured spinal cords is the low survival rate of these cells because of the inhospitable lesion environment. General practice of stem cell therapy involves delivering stem cells in saline solutions, which do not provide any protective barriers to these stem cells entering the injury sites. Our polymeric carriers will not only prolong stem cell survival and secretion of good proteins, but also provide healthy spinal cord tissue components from the carriers themselves. The ongoing research in my lab funded by PhRMA Foundation has led to optimization of polymeric carrier formulations and characterization of stem cell behavior in our carriers. Successful completion of the work will allow us to develop new treatment options for SCI that can increase clinical potential of stem cell therapy after SCI.

The First Polymeric Conjugate Vaccine for Drug Addiction

"PhRMA Foundation Research Starter Grant funding has allowed my lab to pursue a high-risk, high-reward project, yielding sufficient data to submit a patent on our technology and pursue multiyear grant funding to continue our quest to transition our technology to the clinic. In addition, the award allowed me to offer additional training opportunities and support to young scientists as they begin their scientific journey."

David Scott Wilson, PhD
Johns Hopkins University School of Medicine

Opioid addiction is a national medical crisis that is expected to worsen due to the socioeconomic fallout caused by the SARS-CoV-2 pandemic. Opioid vaccines are a promising treatment strategy for preventing opioid overdose and could enhance drug treatment when combined with existing modalities. The aim of vaccination is to generate opioid-specific antibodies that circulate in the serum to neutralize opioids, thus preventing overdose and the sensation sought by the user. However, given the inherent challenges of repeated vaccination, effective opioid vaccines must induce long-lasting, opioid-specific antibody responses. Here, we present a biomaterials-based strategy for engineering durable opioid-specific antibody responses. Our platform is designed to localize the delivery of various activating agents to the cells that produce opioid-specific antibodies, thus increasing the magnitude and durability of the opioid-specific antibody response while limiting the toxicity of these agents. If successful, our project will yield a clinically-viable therapy for preventing opioid overdose and demonstrate the efficacy of a platform that can be easily modified to treat other forms of drug addiction.

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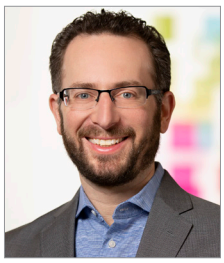

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PhRMA
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Treasurer's Report

I am pleased to take over the role of treasurer from Andrew Plump, MD, PhD, president of Research and Development at Takeda, as he steps into his new role as chair of the PhRMA Foundation Board of Directors. I am excited to share the details of the Foundation's progress with you, demonstrating the organization's ongoing commitment to enabling the next generation of biopharmaceutical advances by supporting the pipeline of top-quality researchers.

As the world continues to face challenges from the COVID-19 pandemic, in addition to political, social, and economic unrest, the PhRMA Foundation is grateful for the ongoing support of our generous supporters. The Foundation's programs provide support to a diverse array of researchers conducting work at the frontiers of drug delivery, drug discovery, translational medicine, health outcomes research, and value assessment.

The PhRMA Foundation maintains a strong financial position. Our supporting companies contributed about $2.8 million in 2021, compared with about $3 million in 2020, in line with expectations. Meanwhile, our investments garnered $2.6 million in gains in 2021. These company contributions, along with our investments, are the Foundation's sole support. The Foundation's expenditures totaled about $4.4 million in 2021, including $3 million in grants and awards and $1.4 million in program and supporting services.

In 2021, Value Assessment Initiative grants totaled $950,000, bringing us to a five-year total of $4.7 million dedicated to this program. This commitment from the Foundation has led to a more robust field, increasing the breadth and depth of value assessment efforts. The Value Assessment Initiative continues to support researchers working on the development and application of high-quality, patient-centered approaches to value assessment through research awards and challenge awards.

As of December 31, 2021, the Foundation's net assets totaled $26 million, a nearly 7% increase from the prior year. The increase in net assets is attributable to the return on the Foundation's investments, which provide a source of stability for our operations and allow us to invest in emerging and novel areas of biopharmaceutical innovation. Please find financial details in the accompanying Statement of Activities.

Through prudent financial management, the PhRMA Foundation is well positioned to execute on its mission for decades to come.

Sincerely,

Prof. Dr. med. Iris Loew-Friedrich
Treasurer

Statement of Activities

For the year ended December 31, 2021

REVENUE AND SUPPORT	
Contributions	$2,764,822
Contributions – in kind from PhRMA[1]	$74,102
Interest and Dividends	$407,496
(Realized and Unrealized) Gains in Securities	$2,644,590
Other Income	$150,683
Total Revenue and Support	**$6,041,692**

EXPENSES	
Grants and Awards	**$2,955,757**
Program Services	**$1,089,845**
Supporting Services	**$361,673**
TOTAL EXPENSES	**$4,407,275**

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

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