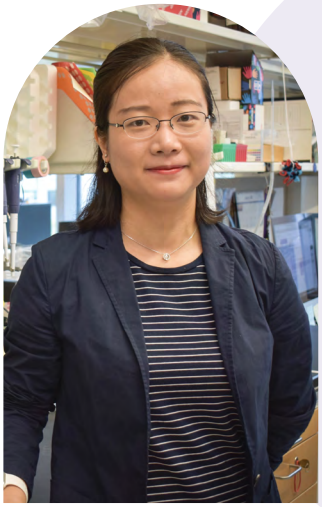
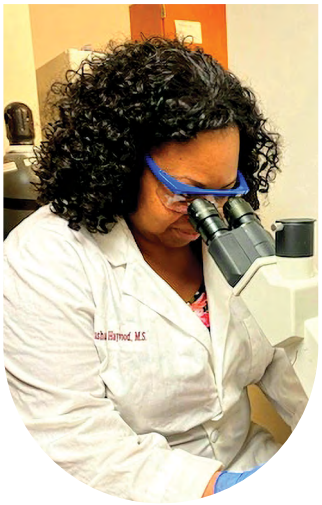
2023 Annual Report





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Mission

The PhRMA Foundation fosters biopharmaceutical innovation and value-driven health care by investing in the frontiers of research. The Foundation catalyzes the careers of promising researchers through competitive, peer-reviewed grants and fellowships.

Vision

A healthier world where all people have access to innovative, life-changing medicines.

Values

- **INTEGRITY**
  We strive to be scientifically independent and evidence-based in our decision-making.
- **INNOVATION**
  We invest in cutting-edge research and ideas that will improve patient health.
- **COLLABORATION**
  We support collaborative research efforts that are diverse and inclusive.

Message from the

President and Chair

As the leaders of the PhRMA Foundation, we are committed to championing promising researchers and their innovative ideas to foster biopharmaceutical innovation and value-driven health care. By investing in today's early career researchers, we are setting the stage for tomorrow's medical breakthroughs that could improve patients' lives. The Foundation's ability to support these researchers hinges on the generous support of the pharmaceutical companies that fund our programs and volunteer their employees' time to advance our vision of a healthier world where all people have access to innovative, life-changing medicines.

PROVIDING CRUCIAL EARLY-CAREER SUPPORT

Through a peer-reviewed, competitive process, the PhRMA Foundation awards grants and fellowships to researchers pursuing novel science in the fields of drug discovery, drug delivery, translational medicine, and value assessment and health outcomes research. In 2023, we provided 53 awards totaling over $4.7 million. This represents an increase in the funding and number of researchers awarded compared with 2022 and is mainly due to three special opportunities we offered in 2023, which are described in more detail below.

For predoctoral fellows and postdoctoral trainees, our stipend support allows them to dedicate their time and attention to specific research projects. For early-career faculty awardees, the Foundation grants enable them to launch their independent careers and work toward acquiring their first large National Institutes of Health (NIH) grant. By funding up-and-coming scientists across the United States, the PhRMA Foundation helps to build and train a workforce to support the ever-changing needs of the biopharmaceutical sector.

Foundation award recipients are conducting exciting research on drug delivery methods and treatments for conditions such as opioid use disorder, tuberculosis, chronic pain, and many types of cancer. Awardees are also studying new ways to incorporate patient preferences and health equity considerations into value assessments of health care interventions.

ADDRESSING PRESSING INDUSTRY ISSUES

The PhRMA Foundation is flexible and adaptable, and we adjust course to meet changing scientific needs. In 2023, we offered three special funding opportunities to address important topics of interest.

Harnessing Digital Health Tools for Inclusive Regulatory Decision-Making

In October 2022, the Food and Drug Administration (FDA) released a report outlining important research areas to advance the digital health landscape. To help focus these research efforts, the PhRMA Foundation is funding research aimed at answering FDA's questions regarding the use of digital health technologies in populations typically underrepresented in research.

Real-World Application of Value Assessment Frameworks

While a variety of value assessment frameworks exist, some have yet to be applied in a real-world context or evaluated using empirical studies. The PhRMA Foundation awarded $1 million for research applying previously theoretical frameworks to evaluate whether they can reliably guide value assessment, incorporate relevant diverse elements of value, and identify appropriate patient-centered outcomes.

Inflation Reduction Act Implementation

How can the Centers for Medicare & Medicaid Services (CMS) enable the effective implementation of Medicare drug price negotiation under the Inflation Reduction Act? The PhRMA Foundation partnered with the Journal of Managed Care & Specialty Pharmacy (JMCP) on a call for manuscripts examining this question. Three winning papers received $25,000 and were published in JMCP to provide CMS with important considerations.

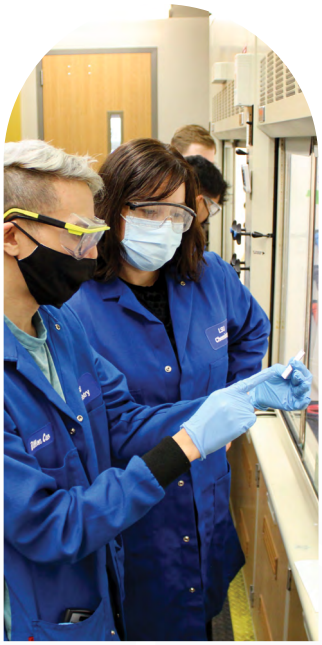
More detailed information on each funding opportunity can be found in the Grants & Fellowships section of the report.

THANKS TO OUR SUPPORTERS

Thanks to consistent support from pharmaceutical companies, Board members, individual volunteers, and partner organizations, the PhRMA Foundation continues our legacy of investment in the next generation of scientists. This support is vital to our ability to steadfastly maintain our core award programs and allowed us to grow our programs in 2023 to include special opportunities focused on research important to both industry and patients. We look forward to working with the PhRMA Foundation's supporters to keep this momentum going in 2024.

Amy M. Miller, PhD
President, PhRMA Foundation

Andrew Plump, MD, PhD
Chair, PhRMA Foundation



2023

Year in Review

53 AWARDS | TOTALING $4.7 MILLION

Award Demographics

- 55% Women
- 45% Men
- 59% White
- 30% Asian
- 10% Hispanic/ Latino
- 2% American Indian/ Alaska Native
- 8% Black

20 Predoctoral Students

11 Postdoctoral Fellows

18 Faculty Members

15 Challenge Award Authors

7 in Digital Health Technologies

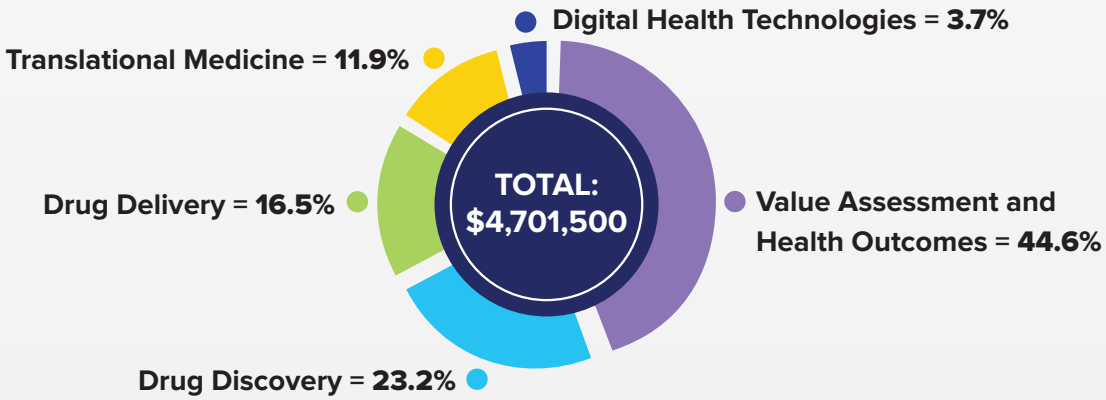
16 in Value Assessment and Health Outcomes

15 in Drug Discovery

10 in Drug Delivery

5 in Translational Medicine

Funding by Research Area



Highlights from

The Year

A NEW LOOK AND WEBSITE

At the PhRMA Foundation, we are passionate about supporting promising researchers with innovative ideas that have the power to change health care. This year, we launched a refreshed brand that better reflects this passion. The Foundation invests in early-career researchers, and our new brand matches the vitality and excitement of these researchers. Our new logo features an elegant, contemporary serif font that both modernizes our look and honors our legacy brand. It also features an elliptical orbit of spheres symbolizing the spark of a new idea. With a new brand comes a new website, and our updated web design and navigation make it easier to quickly find what you need. We also created a searchable awardee database that helps us better promote our award recipients and their research. We invite you to explore our new website to learn about the impact of the PhRMA Foundation's investment in the next generation of researchers.

ENSURING EQUITY WITH DIGITAL HEALTH TOOLS

The COVID-19 pandemic accelerated the adoption of digital health technologies (DHTs) such as sensors, apps, and wearables in medical research by allowing for the continuation of clinical trials while minimizing the risk of spreading disease. To ensure these new tools are being designed and implemented in ways that promote health equity, the PhRMA Foundation launched a program to invest in research on the use of DHTs in underrepresented populations in clinical trials. The program's goal is to help answer critical questions to advance FDA regulatory decision-making regarding DHTs for diverse populations. Seven researchers received $25,000 planning grants based on their letters of intent, and they will compete in 2024 for two $500,000 grants.

BROADENING THE VALUE ASSESSMENT CONVERSATION

In 2023, the PhRMA Foundation offered two special funding opportunities focused on pressing issues in the field of value assessment.

Frontier Award

Value assessment is becoming increasingly important as the U.S. moves toward a value-based health care system. Therefore, it is crucial that value frameworks incorporate meaningful patient engagement and outcomes. This year, the Foundation awarded two $500,000 grants for research projects applying novel approaches to patient-centered value assessment to help guide health care decision-making.

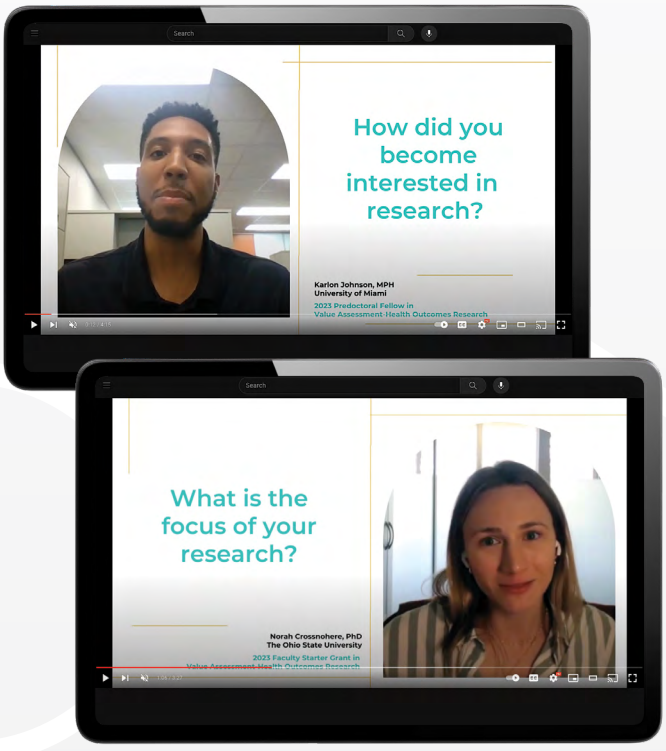
Challenge Award: Addressing Unanswered Questions in Medicare Drug Price Negotiation

The PhRMA Foundation and JMCP partnered on a competitive call for papers examining crucial challenges in the implementation of the Inflation Reduction Act by CMS. Three winning papers received $25,000 awards and were published in JMCP.

RESOURCES TO ENHANCE ENGAGEMENT AND VALUE

In addition to financial support, the PhRMA Foundation offers awardees and applicants opportunities to hone their professional skills and explore career opportunities. We partnered with UCB and Biogen on webinars about their fellowship programs, which offer early-career researchers a chance to gain experience in the biopharmaceutical industry. The Foundation also hosted two webinars focused on grant writing skills, including developing a strong letter of intent and crafting realistic research aims. To complement these popular sessions, we created blogs and infographics highlighting the speakers' excellent advice. Finally, PhF Head of Communications Emily Ortman hosted a science communications training and a Q&A with a science journalist for the 2023 awardees to sharpen their communications and media knowledge. These resources increase the Foundation's engagement with awardees and applicants and provide added value for both researchers and our industry funders.

BUILDING OUR VISIBILITY BY PROMOTING AWARDEES



The PhRMA Foundation's award recipients are our best resource for demonstrating our impact and spreading the word about our programs. We started a new effort in 2023 to interview as many of our awardees as possible. We featured 32 of our 2023 award recipients in video Q&As and blog posts. Awardees talked about their personal research journey, their funded project, how the Foundation award impacted them, and their future career aspirations. For many of these early-career researchers, this was their first time being interviewed and served as a great opportunity to practice their communications skills. Communicating research to the public benefits both scientists and society by building trust in science and providing valuable knowledge for health care decision-making. These video interviews can be found on our website and YouTube page.

Grants and Fellowships

DIGITAL HEALTH TECHNOLOGIES

The COVID-19 pandemic accelerated the adoption of digital health technologies (DHTs) such as sensors, apps, and wearables in medical research. DHTs allowed for the continuation of health care and clinical trials while minimizing the risk of spreading COVID-19. These circumstances helped demonstrate the value and feasibility of using DHTs in clinical trials.

Nir Pillar, MD, PhD, University of California, Los Angeles

EMPOWERING HEALTH CARE EQUITY: HARNESSING DIGITAL HEALTH TOOLS FOR INCLUSIVE REGULATORY DECISION-MAKING

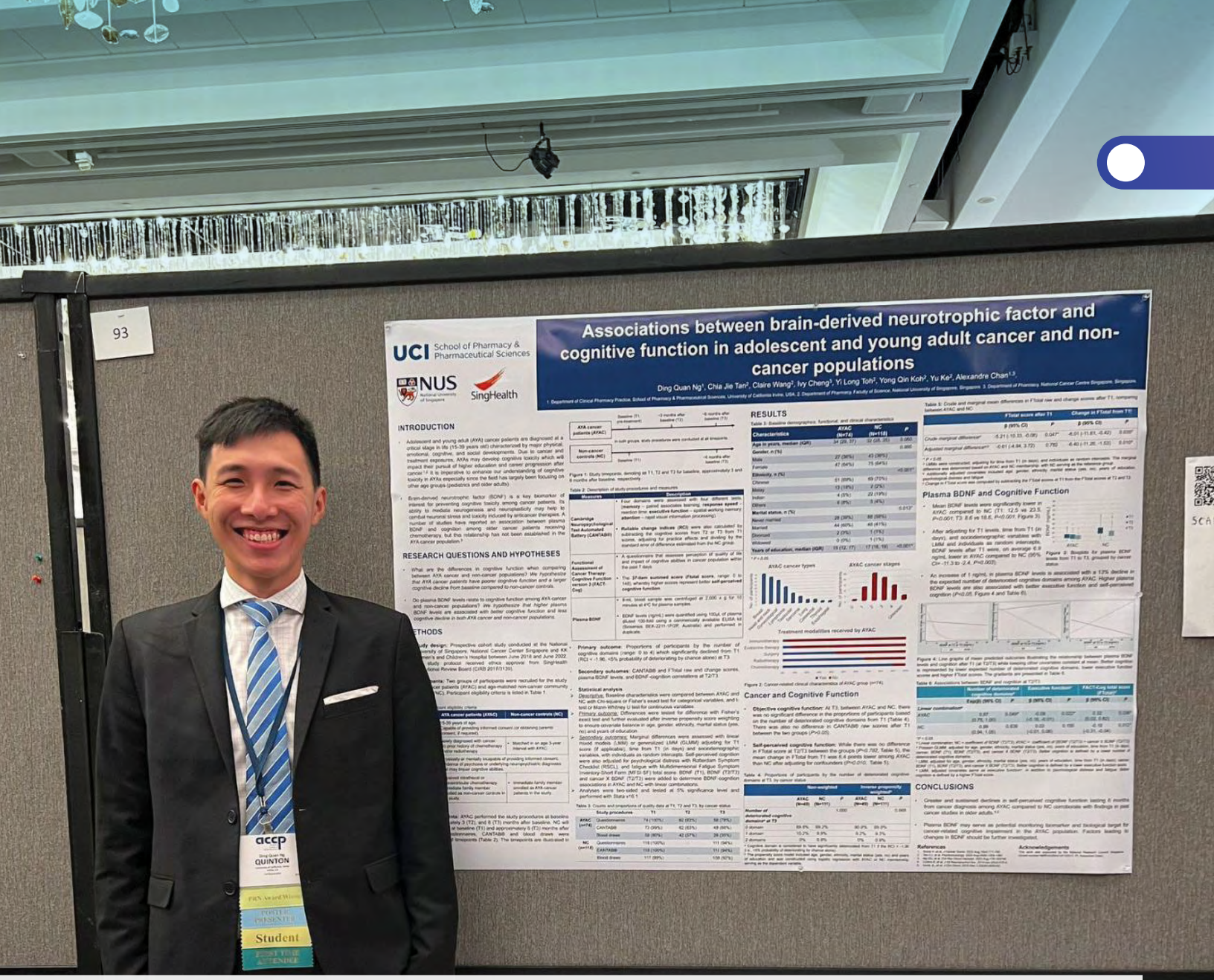
In October 2022, the FDA released a report outlining important research areas for advancing the digital health landscape. To help focus these research efforts, the PhRMA Foundation issued a call for research on the use of DHTs in underrepresented populations in clinical trials to advance FDA regulatory decision-making.

> **These tools hold incredible promise for advancing research, but we must ensure that we are thoughtfully designing and implementing them to promote health equity and not exacerbating existing disparities in the health care ecosystem.**

Amy M. Miller, PhD
PhRMA Foundation President

Based on letters of intent, seven researchers received $25,000 planning grants and will compete in 2024 for two $500,000 grants:

- **David Armstrong, MD, PhD**, University of Southern California
- **Andres Duarte-Rojo, MD, PhD**, Northwestern Memorial Foundation
- **Nino Isakadze, MD**, Johns Hopkins University
- **Leticia Moczygemba, PharmD, PhD**, University of Texas at Austin
- **Willi Tarver, DrPH**, The Ohio State University
- **Frank Tsai, MD**, HonorHealth Research Institute
- **Rachel Vitali, PhD**, University of Iowa



VALUE ASSESSMENT AND HEALTH OUTCOMES

Value assessment and health outcomes research provide evidence about the benefits, risks, and costs of treatments to help guide health care decision-making. The PhRMA Foundation Value Assessment and Health Outcomes Research (VA-HOR) Program funds researchers investigating challenges in evaluating the delivery, safety, effectiveness, and value of health care interventions. The Foundation has funded health outcomes research since 2002 and value assessment research since 2017. This year, we funded a special multiyear Frontier Award and a Challenge Award, along with our traditional predoctoral and postdoctoral fellowships and faculty grants.

Ding Quan (Quinton) Ng, University of California, Irvine

FRONTIER AWARD

Value assessment frameworks aim to evaluate the relative benefits and costs of health care interventions. While a variety of frameworks exist, some have yet to be applied in a real-world context. To advance the field, we need to assess whether these frameworks can reliably guide value assessment, incorporate relevant diverse elements of value, and identify appropriate patient-centered outcomes. The Frontier Award is a special multiyear funding opportunity for empirical studies that apply a patient-centered value assessment framework to determine the value of a health care intervention.

The PhRMA Foundation awarded two three-year $500,000 grants:

Surachat Ngorsuraches, PhD
Auburn University

Dr. Ngorsuraches, associate professor of health outcomes research and policy in Auburn's Harrison College of Pharmacy, will assess the value of therapies for multiple sclerosis using patient-centered multicriteria decision analysis (MCDA), with a focus on the perspectives of patients and families in the Deep South. MCDA is a decision-making tool that helps capture and weigh multiple factors important to stakeholders, including nontraditional measures of value. His team will work with the Alabama, Louisiana, and Mississippi Chapter of the National Multiple Sclerosis Society.

"We hope this study will amplify the often-overlooked patient voices in the Deep South and lead to the application of the patient-centered MCDA in the value assessments of other treatments and interventions in these states and across the country."

William Padula, PhD
University of Southern California

Dr. Padula, assistant professor of pharmaceutical and health economics at the USC Mann School of Pharmacy and Pharmaceutical Sciences, will investigate applications of Generalized Risk-Adjusted Cost-Effectiveness (GRACE) for valuing cancer therapies. GRACE aims to improve health care valuation by accounting for patients' preferences as they relate to the value of hope, insurance value, and health equity to quantify optimal cost-effectiveness thresholds that rise for more severe diseases and reduce for milder conditions. His team will work with patient support organizations CancerCare and The Breast Cancer Fundraiser.

"We aim to illustrate the utility of GRACE to improve interpretation of the value of treatments for the most common cancers affecting women and men — breast cancer and prostate cancer. This work should have important implications for federal agencies such as Centers for Medicare & Medicaid Services as they explore new methods in pharmacoeconomics to support drug price negotiation efforts under the Medicare Part D program."

ADDRESSING UNANSWERED QUESTIONS IN MEDICARE DRUG PRICE NEGOTIATIONS

The Inflation Reduction Act (IRA) authorizes Medicare to negotiate the prices of top-selling drugs with manufacturers. The PhRMA Foundation partnered with the Journal of Managed Care & Specialty Pharmacy (JMCP) to host a competitive call for papers examining crucial challenges in IRA implementation by the Centers for Medicare & Medicaid Services (CMS). We sought manuscripts that offered innovative solutions that aligned with the legislative mandate while contemplating diverse stakeholder needs.

> **As CMS moves forward with implementation of Medicare drug price negotiation, many details regarding the process remain unclear. We wanted to tap into the knowledge and expertise of the research community to provide CMS with considerations for addressing some of these challenges in ways that are scientifically rigorous, transparent, and most importantly, patient centered.**
>
> **Amy M. Miller, PhD**
> PhRMA Foundation President

Three winning submissions received a $25,000 Challenge Award:

- **Medicare Drug Price Negotiation: The Complexities of Selecting Therapeutic Alternatives for Estimating Comparative Effectivenes**
  Inmaculada Hernandez, PharmD, PhD, Emma Cousin, PharmD, Olivier J. Wouters, PhD, Nico Gabriel, MA Teresa Cameron, MS, Sean D. Sullivan, BScPharm, PhD

- **Identifying Therapeutic Alternatives in CMS Drug Negotiation: The Case of Etanercept**
  Helen Mooney, MPH, Matthew Martin, MA, Liam Bendicksen, BA, Aaron S. Kesselheim, MD, JD, MPH, Benjamin N. Rome, MD, MPH, Hussain S. Lalani, MD, MPH, MSc

- **Evidence Inventory: A Patient-Centered Methodological Framework for CMS to Assess the Clinical Benefit of Drugs to Inform Maximum Fair Price Negotiation**
  Nabin Poudel, PhD, Salome Ricci, PharmD, MS, Julia F. Slejko, PhD

These papers and others are featured in a March 2024 themed issue of JMCP focused on drug pricing policy and the Inflation Reduction Act.

PREDOCTORAL FELLOWSHIPS

$25,000 per year of stipend support for up to two years

U.S. Population Preferences for Improving Fairness in Health Policy

Christopher Cadham, MPH
University of Michigan

When determining the value of health care interventions, it is critical to consider what the public perceives as good value. My research seeks to advance methods for incorporating population preferences into evaluations of health care interventions, specifically looking at national preferences around the equitable allocation of health resources in the U.S. I will field surveys asking U.S. respondents to make a series of choices between health care programs that target different groups to understand what people consider fair outcomes. I will use this information to develop quantitative weights that reflect national preferences on the extent to which individuals are willing to give up total health gains to improve outcomes for specific groups. Finally, I will demonstrate how these weights can be incorporated into a cost-effectiveness analysis, which will assess the costs and health outcomes of using e-cigarettes to help people quit smoking. This project will show how using novel methods of cost-effectiveness analysis can improve resource allocation and policymaking by promoting population preferences.

Understanding the Impact of Financial Toxicity on Cancer Patients

Ruiqi Jin, RN, MSN
Emory University

Many cancer patients worry about their finances, especially when they need expensive, long-term treatment. The adverse impact of cancer on a patient's financial well-being is called "financial toxicity," and it often decreases cancer patients' quality of life and overall health. My research aims to better understand financial toxicity by using a framework to study its potential factors and effects. My study explores how financial toxicity changes over time and how it affects cancer patients. By doing this research, I hope to identify factors that can be changed or improved to reduce financial toxicity for cancer patients. This knowledge can help health care providers better assess the risks and find ways to help patients who face this problem during their cancer journey.

Examining the Effect of Discharge Education on Post-Stroke Outcomes

Karlon Johnson Jr., MPH
University of Miami

When patients are released from the hospital after a stroke, they are provided with information to help guide their recovery, such as education on medication, diet, exercise, and substance use cessation (if applicable). The impact of this discharge education on post-stroke health outcomes is an understudied yet vital topic for improving hospital-to-home care transitions for patients. My research investigates factors related to patient-reported receipt of sufficient discharge education and guidance among acute stroke patients discharged to home or inpatient rehabilitation. I will examine the role of this information in increasing healthy lifestyle and behavioral modifications at 30 days post-discharge and reducing death, rehospitalization, or emergency room readmission at 90 days post-discharge. Racial disparities in post-stroke outcomes are prevalent in the U.S., so I will also assess these observed associations by race/ethnicity to better understand the need for effective intervention strategies to improve health outcomes for stroke patients.

Assessing the Value of Acupuncture for Cancer-Related Pain

Ding Quan (Quinton) Ng
University of California, Irvine

Pain is arguably the most feared and debilitating symptom faced by cancer patients. Pain can stem from tumors pressing on nerves or even from the cancer therapy itself. This pain may cause patients to stop lifesaving cancer treatment, leading to worse health outcomes. Acupuncture is now recommended in multiple guidelines for relieving pain experienced by cancer patients. However, the use of acupuncture in clinics is hindered by the lack of coverage across health plans and many clinicians' skepticism in acupuncture as an evidence-informed treatment. Thus, my research aims to assess the economic and health benefits of acupuncture for cancer patients to address these concerns from insurance companies and clinicians. My project will compare the medical costs to insurance companies before and after cancer patients begin acupuncture for treating pain and assess whether cancer patients maintain the recommended frequency and duration of pain-inducing cancer treatment when they receive acupuncture. These findings can inform the increase in acupuncture access for cancer patients and build upon the evidence regarding the synergy of Western and traditional medicine, also known as "integrative medicine," in managing health problems faced by cancer patients.

Helping Older Veterans to Stop Taking Potentially Harmful or Unnecessary Medications

Helen Omuya, MPA
University of Wisconsin-Madison

Many individuals develop multiple chronic health conditions as they grow older, requiring treatment with medications. In some cases, older adults could take as many as 20 medications daily. The unwanted side effects of these medications are often treated with additional medication. Although pharmacists at the Department of Veterans Affairs work hard to ensure that potentially harmful drugs or those that provide no benefit to the patient are discontinued, their efforts are limited by many factors and barriers. Furthermore, patients are sometimes afraid of stopping a medication they have been taking for a long time. My research will enable Veterans Affairs health care providers to identify ways to improve how they support patients in stopping medications that do more harm than good. I am creating a Deprescribing Intervention Quality Improvement (DIQI) instrument that focuses on what matters most to the patient. This instrument will identify ways to limit barriers and improve support for veterans.

I am very honored to receive the prestigious PhRMA Foundation predoctoral fellowship. It has provided the confidence to pursue independent research in improving cancer-related health outcomes, specifically in cancer survivorship and supportive care.

Ding Quan (Quinton) Ng
University of California, Irvine

Assessing the Link Between Newer Diabetes Drugs and Alzheimer's Disease Risk

Huilin Tang, MS
University of Florida

About 6.5 million older Americans lived with Alzheimer's disease in 2022, and this number is expected to double by 2060. Despite progress in drug development for Alzheimer's, there is still no cure. Some newer drugs used to lower blood sugar for type 2 diabetes patients have also shown potential benefits for Alzheimer's patients. My research seeks to understand how these drugs affect the risk of Alzheimer's disease and related dementias in patients with type 2 diabetes and identify who would benefit most from these medications. This study will also determine whether these drugs reduce the risk of Alzheimer's disease and related dementias through pathways beyond lowering blood sugar levels. The findings of this study will provide evidence for repurposing these drugs for Alzheimer's patients and inform personalized treatment for people with type 2 diabetes who are at high risk for Alzheimer's. Ultimately, this research could improve their long-term outcomes and promote healthy aging.

Real-World Mood Monitoring to Identify Patients at Risk of Stroke

Stephanie Zawada, MS
Mayo Clinic

A leading cause of disability in the U.S. is cerebrovascular disease, or conditions such as stroke that affect blood flow to the brain. Reducing the severity and longevity of disability linked to these conditions could exponentially decrease U.S. health care expenditures. My research project seeks to identify patients at increased risk for cerebrovascular disease so that clinicians can provide preventive treatments. Because psychological symptoms can be predictive of a stroke, we will use a smartphone app to remotely monitor mood changes in patients who have previously experienced a temporary blockage of blood flow to the brain, called a transient ischemic attack (TIA). This project aims to identify which TIA patients would benefit most from monitoring and which negative mood symptoms are best to track and at what time intervals. The identification of previously undetected at-risk patients could help clinicians target pharmacological or psychotherapy interventions to reduce a patient's risk of stroke or other post-TIA disabilities. In the short term, positive findings from this pilot study could be immediately translated into routine practice at Mayo Clinic for patients at risk of stroke.

FACULTY STARTER GRANTS

$100,000 for one year of research project support

Evaluating Racial Differences in Preferences for Multi-Cancer Early Detection Tests

Norah Crossnohere, PhD
The Ohio State University

Multi-cancer early detection tests (MCEDs) are the next frontier of cancer screening, with the potential to identify up to 50 types of cancer from a single blood sample. There is hope that they could increase screening rates among Black patients, who bear a disproportionate cancer burden. The overarching goal of my research project is to measure patient preferences for the use of MCEDs and compare preferences across racial groups. Through a literature review, patient interviews, and expert consultation, we will first identify factors that may affect patients' decisions to use MCEDs, such as the benefits, risks, and uncertainties surrounding the tests. We will use this information to create a discrete-choice experiment in which patients make decisions in hypothetical scenarios about MCEDs. This allows us to measure and compare patient preferences across a racially representative group of patients and a Black/African American group of patients. This study will inform MCED developers and regulators about what outcomes and tradeoffs are acceptable to diverse patients.

> **I could not be more excited to receive this award. It is a huge milestone in my professional career and will support much needed research into preferences and equity for multi-cancer early detection tests.**
>
> **Norah Crossnohere, PhD**
> The Ohio State University

Examining the Benefits and Challenges of Self-Treatment for Opioid Addiction

Jarratt Pytell, MD
University of Colorado, Denver

Many people battling opioid addiction struggle to access medication treatment due to various life challenges, such as transportation issues, financial constraints, or long wait times for appointments. As a result, some resort to self-treating their addiction by obtaining opioid addiction medications from friends, family, or other contacts. This self-treatment approach is similar to how some people self-medicate common ailments using over-the-counter drugs or prescribed medications they get from friends, family, or acquaintances. Although preliminary studies suggest that individuals can effectively self-treat opioid addiction using such medications, our study aims to explore this on a national scale. By examining a larger and more diverse sample representing the entire U.S., we hope to determine whether self-treatment reduces overall opioid usage and addiction severity. Our findings will provide valuable insights for public health officials, guiding potential decisions on whether certain opioid addiction medications should be available without a doctor's prescription.

> **The overdose crisis demands urgent action. As an addiction medicine physician, I have witnessed the devastating impact of opioids and other drugs on my patients, their families, and communities. This award will allow me to initiate a new and innovative research program to improve the lives of those who use drugs.**
>
> **Jarratt Pytell, MD**
> University of Colorado, Denver

MID-CAREER FACULTY GRANTS

$100,000 for one year of research project support

Incorporating Patient Preferences for Risk Into the Value of Treatment for Diabetic Macular Edema

Karen Mulligan, PhD
University of Southern California

Cost-effectiveness analysis (CEA) is a method for assessing the costs and health outcomes associated with health care interventions and is commonly used to guide coverage and pricing decisions. Yet concerns remain that CEA omits important elements of value such as equity and potentially discriminates against the aged, disabled, or terminally ill. The generalized risk-adjusted cost-effectiveness (GRACE) model addresses these issues but has remained theoretical to date because key parameters measuring patient risk preferences over health have been unknown. Risk preferences are estimated by examining how individuals make treatment decisions, such as when deciding between an option with a certain health outcome versus an option with an uncertain outcome. This study will present an application of GRACE using recently published estimates of risk preference parameters. In particular, we will estimate the value of treatments for diabetic macular edema, an eye condition that affects nearly 1.1 million people in the U.S. and is the most common cause of vision loss among adults with diabetes. A comparison of value estimates from GRACE and CEA will enhance our understanding of how decision-making will change as we account for patient preferences for risk and adjust for disability and disease severity.

Incorporating Equity Into Value Assessment of Health Care

Surachat Ngorsuraches, PhD
Auburn University

When using patient preferences to assess the value of health care, many studies deploy a personal or self-interest approach. However, patients may have preferences that extend beyond concern for themselves, including equity (e.g., a treatment's benefits and risks for patients with worse health). In addition, previous studies examining societal preferences on equity vs. efficiency (e.g., cost-effectiveness of a treatment, number of potential beneficiaries) tended to use an impersonal approach that failed to account for individuals' preferences on these issues. My proposed study will demonstrate a value assessment method that simultaneously captures patient preferences as well as equity and efficiency considerations. This type of assessment could help facilitate health care policy decisions that respect the preferences of the affected population while also considering the extent to which the population would be willing to trade efficiency for equity.

DRUG DISCOVERY

All people deserve access to innovative medicines that help them live longer, healthier, and more productive lives. Early scientific research plays a critical role in the discovery of cutting-edge technologies and therapeutic approaches for modern medicines. The PhRMA Foundation's Drug Discovery Program supports predoctoral students, postdoctoral trainees, and early-career faculty conducting early-stage research toward the creation of efficacious, safe, and differentiated treatment options for patients. The Foundation has funded fellowships and grants in drug discovery (formerly known as pharmacology and toxicology) since 1978.

Rhashanda Haywood, University of Maryland, Eastern Shore

PREDOCTORAL FELLOWSHIPS

$25,000 per year of stipend support for up to two years

Developing Effective Chronic Pain Treatments With Reduced Side Effects

Sarah Bernhard
Washington University in St. Louis

Chronic pain is a major reason why adults seek medical attention, but current treatments are often ineffective or have unwanted side effects. Many prescribed pain medications work by activating mu opioid receptors (MORs) on the outside of nerve cells. When opioids attach to these receptors, they trigger chemical changes that lead to feelings of pain relief and pleasure. While MORs can help relieve chronic pain, their activation can also cause negative side effects like addiction and trouble breathing, which can be fatal in cases of overdose. My research focuses on delta opioid receptors (DORs), which are in the same family as MORs but are associated with fewer side effects. While DORs can relieve chronic pain, their activation can sometimes trigger seizures, limiting their potential as a target for new drugs. My study aims to investigate the mechanisms that cause seizure symptoms of DOR drugs using human cell lines and develop new seizure-free DOR drugs. Through this study, we hope to develop safer and more effective treatments for chronic pain.

Designing Novel Organic Molecules as Anti-Seizure Agents

Rhashanda Haywood
University of Maryland, Eastern Shore

Epilepsy is one of the most common neurological disorders, affecting 3 million people in the U.S. and 65 million people worldwide. Epilepsy patients experience recurring, unprovoked seizures caused by an electrical imbalance in the brain. To minimize seizure frequency, researchers seek to develop novel agents that decrease or stop this imbalance in the brain with minimal side effects. Researchers hypothesize that blocking calcium and sodium ion channels in the brain can potentially reduce the occurrence of seizures. Recently, our drug discovery team identified a small organic compound (IAA65) that can block a specific type of calcium channel. My research involves synthesizing 20 new derivatives of IAA65 that we can evaluate as T-type calcium channel blockers. With this research strategy, we hope to identify better drug candidates that will block calcium channels as effective anti-seizure agents with minimal side effects in preclinical and clinical trials.

Examining Ways to Improve Immunotherapy for Lung Cancer

Aakanksha Rajiv Kapoor
Weill Medical College of
Cornell University

Lung cancer is the leading cause of cancer-related deaths worldwide. Immunotherapy, a treatment that activates a patient's immune system to kill cancer cells, has significantly improved outcomes for lung cancer patients, but it only works for about 20% of patients. The treatment fails when immune cells known as myeloid suppressor cells inhibit the function of T cells, which are important for fighting tumors. Hence, it is crucial to develop strategies that target myeloid suppressor cells. My research investigates how proteins produced by club cells, which line the airways in the lungs, can prevent myeloid suppressor cells from blocking the immune response needed to fight cancer. By understanding the mechanisms behind this process, we hope to uncover valuable insights that could enhance the efficacy of immunotherapy in lung cancer treatment. Ultimately, this research could contribute to the development of more effective and targeted treatments for lung cancer patients.

To me, being a PhRMA Foundation award recipient means that genuine hard work and effort can and will pay off, no matter where you come from or whom you know. Oftentimes, when it comes to awarding grant money, political decisions play a higher role than the research itself — the one thing that it should solely be about. Therefore, I owe the PhRMA Foundation not only my deepest gratitude but also my utmost respect.

Jacqueline Plau
Case Western Reserve
University School of Medicine

A New Approach to Treating Muscle Diseases With Lasting Therapeutic Genes

Made Harumi Padmaswari
University of Arkansas

Duchenne muscular dystrophy (DMD) is a common genetic disease that mainly affects young boys. The DMD gene, the largest in our body, has mutations scattered throughout, making it tough to fix just one type. These mutations cause a lack of functional dystrophin protein, leading to muscle damage and deterioration. Current clinical trials aim to deliver a shorter but functional version of dystrophin called microdystrophin. Although the results are promising, the therapy does not last long term because the protein is supplied from outside the body. To address this, my project aims to create a universal platform for integrating therapeutic genes into a specific spot in muscle cells so the gene can be produced internally. This site has a strong muscle gene promoter, ensuring the integrated gene is expressed well and safely. We will evaluate the safety and efficiency of this approach and optimize the design of integration. This innovative method could potentially lead to a secure and lasting recovery of dystrophin expression, opening new possibilities for treating muscle diseases.

Developing a Therapeutic Strategy for Blinding Eye Diseases

Jacqueline Plau
Case Western Reserve University School of Medicine

When light hits our eyes, special cells called photoreceptors convert the light into electrical signals for our brains, enabling us to see. Vitamin A plays a crucial role in this visual process. However, sometimes the process goes awry and causes the accumulation of toxic byproducts of Vitamin A in the eye. This is found in common eye diseases such as age-related macular degeneration (AMD) and Stargardt disease and can ultimately lead to blindness. Stopping the overproduction of these byproducts may be a potential approach for treating these diseases. My project describes a new methodology for controlling Vitamin A byproducts in the eye by developing drug candidates and assessing their potential effects in animal models of human eye diseases.

Examining Novel Compounds to Treat Brain Disorders by Restoring Glutamate Levels

Katelyn Reeb
Drexel University College of Medicine

Glutamate is a chemical that facilitates the communication between brain cells called neurons. Glutamate transporters are proteins that move glutamate back into cells after a message is transmitted, allowing glutamate to be recycled and used again. In many neurological and neuropsychiatric disorders, this system is disrupted by changes in glutamate transporter function. Therefore, restoring the proper function of these glutamate transporters could provide new treatment options for many disorders. My research focuses on understanding how novel compounds identified by our lab affect glutamate transporter function. Through medicinal chemistry, we built a library of new compounds that can increase or decrease the transporters' ability to move glutamate by interacting with a key region of the transporter. My data supports the idea that these new compounds could be therapeutic in ischemic stroke and cocaine use disorder in rodent models. We hypothesize that our compounds may provide clinical benefit by interacting with this key region of the glutamate transporter.

Developing a First-in-Class Therapy to Suppress Highly Aggressive Prostate Cancer

Kinza Rizwan
Baylor College of Medicine

Prostate cancer is the second-leading cause of cancer-related death in men. Prostate cancer is driven by male sex hormones called androgens, and resistance to current treatments can occur through various mechanisms involving the androgen receptor, which mediates the effects of these hormones. For my project, we explored a new approach to treating prostate cancer by targeting a protein called SPOP. Our experiments in mouse models and human prostate cancer cells showed that removing or silencing the SPOP gene significantly reduced androgen receptor signaling. This suggests that SPOP plays a crucial role in driving prostate cancer growth through the androgen receptor pathway. These findings suggest that SPOP could be a promising and innovative target for developing new therapies to combat prostate cancer, especially in cases where current treatments are no longer effective. This research holds great promise for improving the outlook for prostate cancer patients worldwide.

POSTDOCTORAL FELLOWSHIPS

$60,000 per year of stipend support for up to two years

Targeting the Drug-Resistant Cancer Cells in Follicular Lymphomas

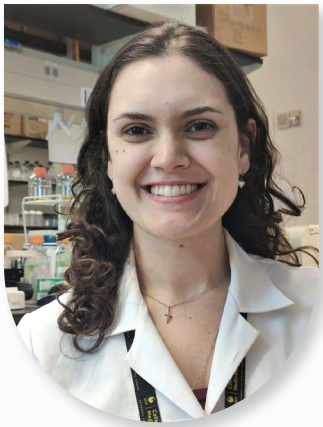
Lydie Debaize, PhD
Dana Farber
Cancer Institute

Follicular lymphoma is a cancer that affects white blood cells called lymphocytes that help the body fight infections. While most patients achieve remission after treatment, many will experience cancer recurrence due to rare drug-tolerant persister cells that remain in the body after treatment and cause cancer to regrow. My research aims to identify the vulnerabilities of persister cells and identify new treatment combinations that can kill them. This project will use follicular lymphoma cells from human patients before treatment, during treatment, and after treatment. With collaborators at the Massachusetts Institute of Technology, we will implement innovative approaches to detect and characterize persister cells and identify potentially targetable features. This work will allow us to nominate a candidate drug and validate it in a preclinical study. The ultimate goal of this work is to transform the way we approach lymphoma therapy by targeting the few remaining cancer cells and thereby convert remissions into cures.

This recognition of my research on prostate cancer disparities is a major step forward in my journey as a PhD candidate and will greatly aid me in my efforts to make a meaningful impact in the field. I am thankful for the support and resources provided by the PhRMA Foundation, which will help me to continue exploring innovative solutions to improve outcomes for men facing prostate cancer disparities.

Kinza Rizwan
Baylor College of Medicine

Strategies to Improve the Discovery of New Tuberculosis Drugs From Natural Products



Priscila Cristina Bartolomeu Halicki
University of Central Florida (UCF)

Tuberculosis is an infectious disease caused by *Mycobacterium tuberculosis (Mtb)* and is the second leading cause of death from a single infectious agent after COVID-19. In 2021, the World Health Organization estimated that 10.6 million people contracted tuberculosis and 1.6 million died. Tuberculosis treatment requires a cocktail of four antibiotics taken for at least six months. Because of the long treatment duration and adverse side effects, there is an urgent need for potent new drugs with novel modes of action for killing Mtb. Natural products — chemical compounds found in nature (such as in plants) — are an outstanding source for potential new drugs. However, prioritizing compounds to investigate is challenging due to their structural diversity, chemical complexity, and broad applications. Using innovative chemical and microbiological strategies, we are developing a method to better assess and prioritize compounds from natural products for their drug development potential. This project will accelerate the identification of compounds with mechanisms of action that could target Mtb.

Combating Chemotherapy Resistance With a New Class of Cancer Drugs

Yongbin Liu, PhD
Houston Methodist Research Institute

More than 50% of cancer patients who receive chemotherapy are treated with platinum-based drugs. However, due to their severe toxicity, these drugs kill both cancer cells and normal cells, resulting in over 40 side effects. In addition, cancer cells can easily develop resistance to these drugs so patients stop responding to treatment. There are limited solutions once chemoresistance has developed, leading to poor outcomes and high mortality for cancer patients. Thus, the discovery of a new class of platinum drugs with lower toxicity and the ability to overcome drug resistance is urgently needed. Using novel nanotechnology, my research aims to develop new platinum drugs that selectively kill both parental and drug-resistant cancer cells without harming the non-cancerous cells, overcoming the limitations of conventional platinum drugs. My project will also demonstrate the feasibility of combining our novel drugs and immunotherapy to eliminate treatment-resistant cancer in patients.

Developing Novel Drugs to Combat the Effects of Synthetic Opioids

Piyusha Pagare, PhD
Virginia Commonwealth University

The opioid epidemic is a national public health crisis, leading to more than 80,000 deaths in 2022. Recently, a new class of synthetic opioids called nitazenes has emerged in the illicit drug market. Nitazenes are highly potent and pose significant risk for addiction and respiratory depression, a breathing disorder characterized by slow and ineffective breathing. Finding efficient reversal agents to counter nitazenes is challenging because we do not understand how these drugs activate the mu opioid receptor, which drives their extraordinary potency and harmful effects. In addition, the efficacy of naloxone, a reversal agent approved by the FDA, in countering nitazenes is uncertain. My research unveiled a unique mechanism by which nitazenes bind with the mu opioid receptor, potentially explaining their high potency. I aim to invent new drug compounds targeting this mechanism and assess their ability to counter nitazene-induced respiratory depression in animal models. My research will form a solid foundation for the development of innovative and effective treatment options for opioid overdose.

After years of hard work in the drug discovery field, being awarded by the PhRMA Foundation makes me believe I am on the correct path. It is a significant step to motivate me even more to find novel solutions for infectious diseases.

Priscila Cristina Bartolomeu Halicki
University of Central Florida (UCF)

Exploring Multiple Strategies to Develop a Drug for a Promising Cancer Treatment Target

Mélanie Uguen, PhD
University of North Carolina at Chapel Hill

Gene expression is the process by which instructions in our DNA are converted into proteins, which are vital to the normal function of our bodies. Abnormal gene expression is linked with disease development, especially cancer. SETDB1, a protein involved in controlling the accessibility of DNA for this process, is a promising target for drug development for cancer. My work focuses on using parallel strategies to develop a drug for SETDB1 to increase the chances of finding one that works and can reach patients soon. One strategy consists of developing drugs that can either temporarily or permanently block the normal activity of SETDB1. Another strategy aims to get rid of the entire SETDB1 protein by using the body's normal protein disposal system. Hopefully, one of these strategies will lead to the discovery of the first drug able to prevent SETDB1 from abnormally reducing the accessibility of DNA for gene expression.

Creating a New Class of Antibody Therapies With Improved Stability and Efficacy

Joshua Walker, PhD
University of California, Berkeley

Antibody therapies include FDA-approved treatments for cancers and autoimmune and infectious diseases. However, the therapeutic proteins used in antibody treatments are susceptible to degradation, which may neutralize their effectiveness. My project seeks to create a new class of drugs that combines antibodies with the properties of natural products that are resistant to degradation. Using innovative technologies, we aim to graft the chemistry of natural products into the protein's structure and then evaluate the impact on the performance of antibody therapeutics. This work will establish antibody-natural product chimeras as a novel class of therapeutics that exhibit improved stability and efficacy. This work can set the stage for the discovery of new protein editing chemistries to accelerate the field of protein therapeutics.

FACULTY STARTER GRANTS

$100,000 for one year of research project support

Treating Opioid Use Disorder Using a Novel Class of Psychoplastogens

Swarup Mitra, PhD, MSc
Oklahoma State University Center for Health Sciences

Opioid use disorder is a major public health crisis. The existing therapeutic options are limited and fail to reverse the long-lasting changes in brain function that underlie vulnerability for relapse. Psychoplastogens are a novel class of drug molecules that are similar to psychedelics. These drugs produce rapid and long-lasting changes in the brain. However, the precise brain mechanisms regulated by psychoplastogens remain elusive. Using a rat model, my study seeks to unravel how two novel psychoplastogens — DM506 and TBG — counter the addictive effects of opioid consumption. We propose that this new generation psychoplastogens that do not produce hallucinations will reduce opioid-seeking behavior in rodents by altering brain mechanisms.

This award will support my research on the discovery and development of novel therapeutic modalities for cancer treatment. I am thankful to the PhRMA Foundation for supporting a French female researcher, and I hope this will encourage many other international women to pursue research in the USA.

Mélanie Uguen, PhD
University of North Carolina at Chapel Hill

DRUG DELIVERY

Drug delivery research focuses on using novel methods to ensure patients get the most benefit from their medicines with the fewest side effects. The PhRMA Foundation Drug Delivery Program funds predoctoral students, postdoctoral trainees, and early-career researchers studying ways to optimize drug composition, dosage, and delivery to make treatments safer, more effective, and easier to manage for patients. The Foundation has funded fellowships and grants in drug delivery (formerly known as pharmaceutics) since 1972.

Fatima Rivas, PhD, Louisiana State University

PREDOCTORAL FELLOWSHIPS

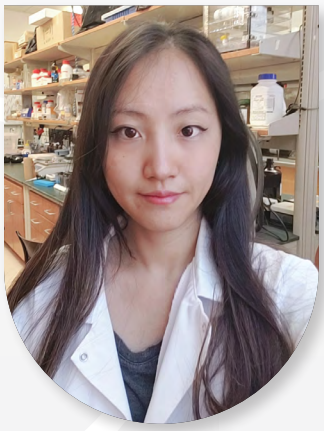
$25,000 per year of stipend support for up to two years

Developing a New Way to Deliver Combination Therapies for Spinal Cord Injury

Neil Baugh
Stanford University

Spinal cord injury results in a traumatic loss of function that is not easily treated with the delivery of single drugs. Complex combination therapies of cell and protein drugs offer a promising avenue to improve regeneration and functional recovery, but better strategies are needed to maximize their delivery. My work will address the limitations of cell and protein codelivery through the creation of an injectable hydrogel system designed to decouple the gel's physical properties from the drug release. This system will protect cells from damage during the injection process and encapsulate the drugs within liposomes — tiny protective spheres — to ensure they are delivered only to the desired injury site. My goal is to develop a system that can improve our ability to deliver complex combination therapies.

Enabling Immune Response to Cancer With the Help of Low-Dose Radiation Therapy



Cynthia Choi
University of Wisconsin
Carbone Cancer Center

Abnormal cell growth in our body is usually eliminated by our immune system. In some cases, these abnormal cells can evade the immune system and develop into cancer tumors. Immunotherapy focuses on stimulating the immune system to recognize the tumors as abnormal cells and naturally destroy them. Unfortunately, some cancer types — known as immunologically "cold" cancers — do not respond to immunotherapy, staying under the radar of the immune system. However, recent studies have shown that low-dose radiation may change these "cold" cancers so that they respond to immunotherapy. Working in mouse models, I will use artificially manufactured proteins called monoclonal antibodies to carry low-dose radiation to "cold" tumors to turn them more responsive to immunotherapy and study how the immune system is being stimulated by this combination treatment. My research project aims to understand the inner workings of this combination treatment, with the hope that it can eventually be used in clinics for human cancer patients.

Engineering Nanoparticles for Improved Spinal Cord Injury Regeneration

Leora Goldbloom-Helzner
University of California, Davis

Spinal cord injury affects over 12,500 people annually in the U.S., causing tissue damage and, depending on injury severity, irreversible paralysis. Spinal cord injury results in widespread cell death and inflammation, which leads to slowed tissue regeneration and continued tissue damage. Currently, most drug therapies fail to provide sustained regeneration at the injury site and do not halt injury progression. My work uses small nanoparticles called extracellular vesicles (EVs), derived from the membrane of stem cells, to deliver regenerative treatment to injury sites. Though EVs have long-term stability and are easily accepted into the body (compared to cells), research has shown that most EVs collect in the liver after injection into the body, while few are found in the spinal cord. My project aims to engineer EVs with a molecule that targets the spinal cord for improved therapeutic delivery and long-term retention in the body, ultimately preventing irreparable damage to the spinal cord.

I am honored to receive a Predoctoral Fellowship in Drug Delivery from the PhRMA Foundation. This support is instrumental for conducting my current PhD research and gives me confidence and a boost to pursue a career researching protein therapeutics.

Cydney M. Martell
Northwestern University

Creating Robust Proteins for Treating Disease

Cydney M. Martell
Northwestern University

Proteins are essential molecules in living organisms and play critical roles in biological processes. Their precise three-dimensional shapes are crucial for their functions, and if they lose their shape, it can cause disease. Protein therapy is an effective treatment that involves using engineered proteins to repair or replace damaged ones in the body. The success of these therapies depends on maintaining the proper shape for interacting with disease targets. Yet proteins can lose their shape and clump together when exposed to stressors such as changes in temperature or pH. These clumps, called aggregates, compromise a drug's safety and efficacy, which is why drugs require strict storage conditions and extensive testing to find the best formulation. My research addresses this challenge by learning how to engineer proteins that fold into the desired shape and resist aggregation after stress exposure. I will measure the stress-induced aggregation for thousands of proteins at once. Then I will use computational modeling to elucidate why some proteins aggregate and others do not. These insights will inform how to design new proteins that resist aggregation, reducing the need for stringent formulation and delivery constraints to enhance their potential in treating disease.

Engineering Stem Cells for Targeted Osteoarthritis Therapy

Vladimir Molchanov
Van Andel
Research Institute

Photo Credit: VARI

Osteoarthritis is a disease that causes loss of joint cartilage, resulting in swelling, pain, and loss of joint motion for millions of people worldwide. Most current treatments aim at temporarily relieving these symptoms rather than restoring the lost tissue. To tackle this problem, researchers are attempting to heal osteoarthritis lesions by generating new cartilage through the transplantation of stem cells. However, clinical trials show that stem cells fail to repair cartilage in patients with osteoarthritis, likely due to poor attachment in the joint and unsuitable conditions for regeneration. My research aims to overcome these obstacles by engineering stem cells to enable their targeted delivery to damaged cartilage regions and localized production of a drug that can accelerate cartilage regeneration. These engineered stem cells hold great potential for providing many osteoarthritis patients with a minimally invasive and highly effective treatment option.

Improving T Cell Therapies for Cancer Treatment

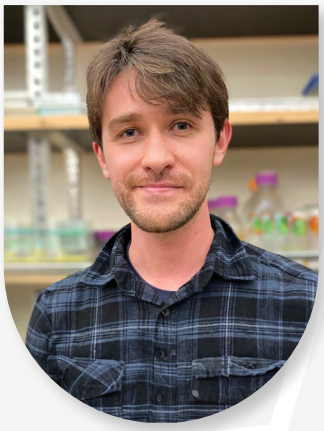
Claire Shudde
University of Michigan

The immune system is critical for fighting cancer. T cells in particular are important for directly killing cancer cells. There are treatments currently available that work to boost the ability of T cells to recognize cancer, however, only 30% of patients respond to these treatments. This is because cancer can create a signal "desert" that does not have the necessary signals that killer T cells need to function and survive. My research is focused on designing a synthetic activator to insert into killer T cells that will enable them to survive and kill cancer cells even in a signal desert. In the future, I will test this activator in a different group of T cells, called helper T cells, which help build an immune response to fight cancer. If this activator can be used in helper T cells in combination with killer T cells to increase their survival and function without need for outside signals, then we can hopefully reach the 70% of cancer patients who do not respond to current T cell therapies.

POSTDOCTORAL FELLOWSHIPS

$60,000 per year of stipend support for up to two years

Modifying Undesired Gut Bacterial Behavior to Treat Disease



Rogerio A. Bataglioli, PhD
Virginia Tech

The gut microbiome, the collection of microbes living in the human gut, plays an essential role in our development and health. The microbiome can be both helpful and potentially harmful depending on the kinds of bacteria present and how they interact with the human body. Changing this bacterial composition toward one that benefits human health is challenging. Bacteriophages (phages) are predators of bacteria that are natural residents of the gut microbiome. Phages can transform bacteria by injecting their genetic material into the targeted cell. My study aims to use engineered phages to alter how specific gut bacteria behave, but not their composition. I will first identify and tune phage traits to maximize gene delivery with minimal impact on bacterial fitness. I will then use this knowledge to target genes in gut bacteria associated with the development of colon cancer and investigate the efficacy of this approach using mouse models. This work will improve our understanding of how phages can alter the behavior of gut bacteria as a long-lasting, noninvasive therapy for diseases in the gut.

Improving the Safety and Efficacy of mRNA Technology

Zhicheng Wang, PhD
University of Pennsylvania

The messenger RNA (mRNA) technology used to develop COVID-19 vaccines has the potential for treating other diseases, but there are challenges that could limit its use. mRNA, a molecule that contains instructions for protein production, is delivered into cells by using lipid nanoparticles (LNPs) as a protective bubble. The mRNA tells our cells to make proteins like those on the COVID-19 virus, which triggers our immune system to recognize and fight the disease. One issue is that LNPs quickly bind with proteins in the blood, causing a reaction similar to anaphylaxis and making them susceptible to elimination by immune cells. In addition, when immune cells take up these LNPs in animals with existing inflammation, the inflammation significantly worsens. My research aims to modify this technology to avoid these drawbacks. We will attach two components — Factor I (a natural enzyme that breaks down complement proteins) and CD47 (a "don't eat me" signal) — to the surface of the LNPs to try to prevent them from being taken up by immune cells. If successful, this project could significantly advance mRNA technology, making it more effective and safer for therapeutic applications beyond COVID-19 vaccines.

The PhRMA Foundation fellowship provides me the flexibility to advance my research on bacteriophage engineering for treating chronic gastrointestinal diseases. It's an honor to participate in this talented and vibrant scientific community.

Rogerio A. Bataglioli, PhD
Virginia Tech

FACULTY STARTER GRANTS

$100,000 for one year of research project support

Beating Antibiotic Resistance Through Inhaled Delivery of Multi-Drug Particles

Ashlee Brunaugh, PhD
University of Michigan

Antibiotic-resistant infections are a major threat facing humanity. Bacteria have several ways to reduce effectiveness of drugs, such as changing their cell membrane to make it harder for drugs to enter or using special pumps to remove the antibiotic from the cell. The location of bacteria in the body can also make treating infections more difficult. In this project, we will increase the ability of existing antibiotics to kill bacteria by pairing them with non-antibiotic compounds that can alter bacterial cell membranes, thus improving the ability for the antibiotic to get inside the bacteria. We will engineer these combinations into particles that can be inhaled by patients with lung infections. The inhaled particles will target the drug combinations to the infection's location to minimize exposure to the rest of the body, thereby increasing the safety and effectiveness of antibiotic treatment.

Improving the Uptake of Natural Chemical Compounds to Treat Health Problems

Fatima Rivas, PhD
Louisiana State University

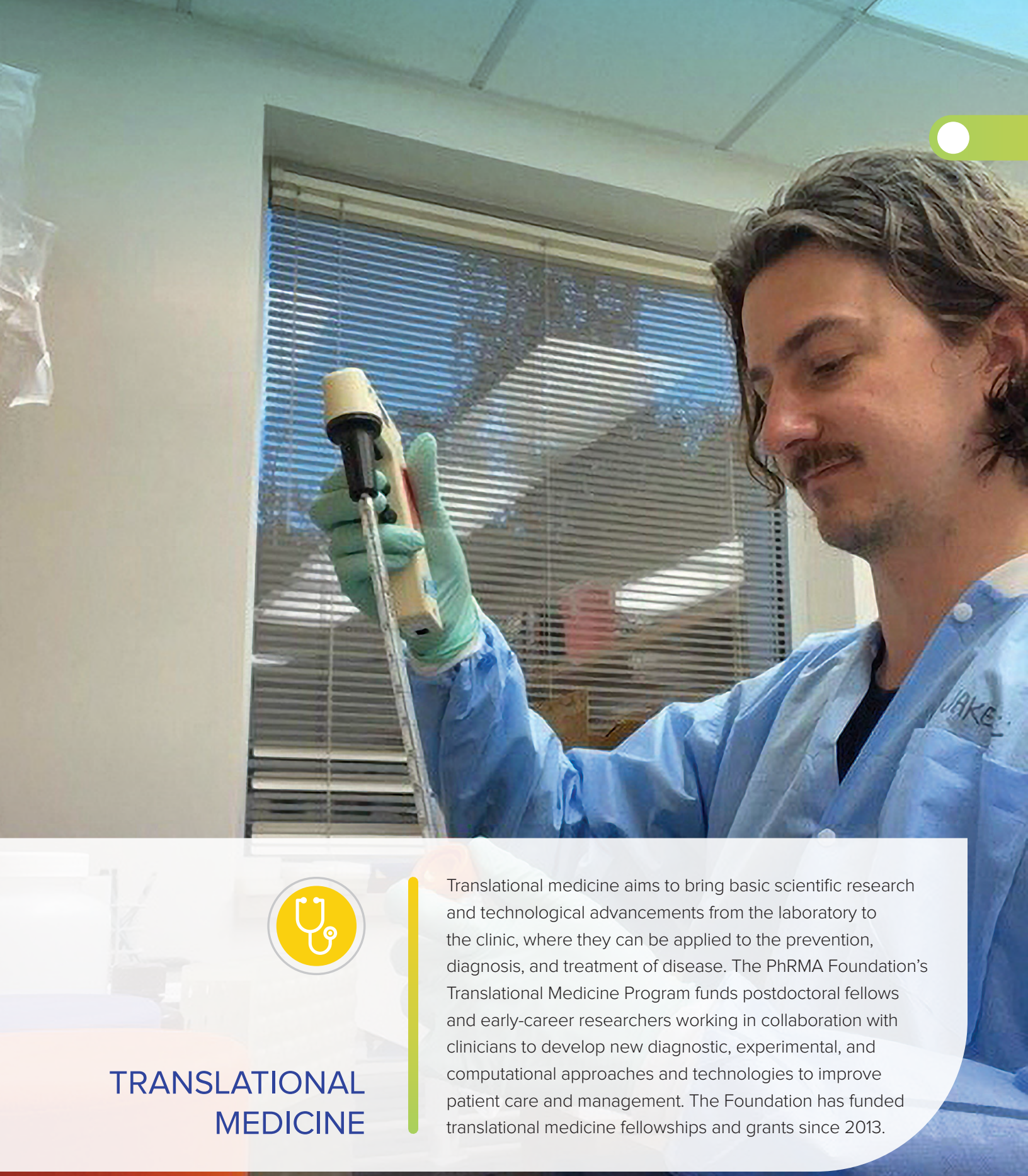
More than 40% of the American population suffers from obesity-related health conditions such as type 2 diabetes mellitus, a serious chronic disorder. Although many naturally occurring chemical compounds have demonstrated therapeutic potential for these health conditions, unfortunately, our bodies cannot properly absorb them because they do not dissolve well in water. My research seeks to identify chemical modifications that will enable these natural products to dissolve in water, leading to improved uptake in the human body. I will then test these modified natural products in mouse models to evaluate their capacity to treat health disorders. This study will provide the necessary information to potentially advance these natural products to clinical trials.

Armoring Viruses to Fight Antibiotic-Resistant Infections

Kevin Yehl, PhD
Miami University

Antibiotic resistance is an urgent public health threat, resulting in about 5 million deaths annually worldwide. In the U.S., there are about 3 million infections a year, causing 35,000 deaths. When antibiotics fail, patients must undergo surgical removal of infection, live with recurring infections, or even succumb to infection. Phage therapy offers a promising potential solution. Phages are viruses that can target and kill bacteria. However, delivering phages to the infection site is a major unsolved challenge. Phages can trigger the patient's immune system, leading to their rapid clearance from the body, which results in treatment failure. Through bioengineering and material science, my research seeks to synthesize "stealth" phage therapies by encasing individual phage particles in nano-glass armor to hide the phage from the immune system, while maintaining therapeutic efficacy. This project will also load antibiotic drugs into the armor shell to study the potency of combination therapy. Ultimately, these studies could open the door for the widespread application of phage therapy and improve outcomes for patients who suffer from antibiotic-resistant infections.

> **This award will have a tremendous impact on my research trajectory by providing the necessary resources to develop a novel inhaled drug delivery platform. Further development of this technology will help establish myself and my research group on the forefront of the respiratory drug delivery field.**
>
> **Ashlee Brunaugh, PhD**
> University of Michigan



TRANSLATIONAL MEDICINE

Translational medicine aims to bring basic scientific research and technological advancements from the laboratory to the clinic, where they can be applied to the prevention, diagnosis, and treatment of disease. The PhRMA Foundation's Translational Medicine Program funds postdoctoral fellows and early-career researchers working in collaboration with clinicians to develop new diagnostic, experimental, and computational approaches and technologies to improve patient care and management. The Foundation has funded translational medicine fellowships and grants since 2013.

Jake Rhodes, PhD, Emory University

POSTDOCTORAL FELLOWSHIPS

$60,000 per year of stipend support for up to two years

Developing Brain Cell Models to Better Understand Schizophrenia

Christina Michalski, PhD
Emory University

In the U.S., about 1 out of 100 people has schizophrenia, a severe psychiatric disorder that impacts mood and thought processes. The molecular cause of schizophrenia is unknown, making it difficult to develop treatments. Patients with a rare genetic disorder called 22q11.2 deletion syndrome (22qDS) have a ~25-fold higher risk than the general public to develop schizophrenia. In this project, we will use blood samples from patients with 22qDS to make innovative models of brain cells in the lab. We will particularly focus on microglia (a type of immune cell in the brain) and compare the molecular makeup of microglia from 22qDS patients and healthy people. These experiments will help to understand disease mechanisms underlying schizophrenia and identify potential therapeutic targets. We hope this project will ultimately lead to better treatment options for 22qDS patients and individuals with schizophrenia.

Using AI to Improve Diagnosis of Systemic Amyloidosis

Nir Pillar, MD, PhD
University of California, Los Angeles

Systemic amyloidosis is a group of rare disorders characterized by the abnormal buildup of misfolded proteins in various tissues, leading to progressive organ dysfunction and death. The symptoms can vary depending on the organs affected and may include fatigue, weight loss, and swelling. Diagnosing amyloidosis is challenging due to the non-specific nature of the symptoms and because it requires the microscopic identification of amyloid deposits in tissue samples. The current diagnostic methods have limitations in terms of sensitivity and specificity and are technically difficult to interpret. Misdiagnosis can lead to the delay of life-saving treatments. For more accurate detection of systemic amyloidosis, my research will use an innovative approach to conduct virtual histological staining, a process that highlights the microscopic cellular appearance of tissue sections without using any chemical stains or additional laboratory steps. We will combine novel microscopy methodologies and deep learning, a type of artificial intelligence that can learn and improve like the human brain, to generate a virtual stain that highlights amyloidosis in a more precise manner. The proposed methodology has the potential to advance systemic amyloidosis detection and, consequently, patient treatment.

Identifying People With Higher Risk of HIV Acquisition

Jake Rhodes, PhD
Emory University

Despite the discovery of medications that prevent HIV infection, 1.7 million people worldwide are still infected each year. This is primarily due to challenges around access to medication in health care settings with limited resources and the high cost of daily medication. Until improved medications are readily available, we could better control HIV infections by identifying individuals who have a significantly higher risk of HIV infection and prioritizing these individuals to receive current medications and other prevention methods. My lab has previously identified two biomarkers — molecules within people's blood that are a sign of a specific condition — that are found in people who are much more likely to become HIV positive. This study aims to better understand the cause of these increased biomarkers and the role they may play in HIV infection. We hope this information can be used to implement more targeted measures to prevent transmission and potentially even develop improved medications against HIV infection.

FACULTY STARTER GRANTS

$100,000 for one year of research project support

Novel Approach to Improve Immunotherapy Response for Acute Myeloid Leukemia

Shengqing (Stan) Gu, PhD
The University of Texas MD Anderson Cancer Center

Acute myeloid leukemia (AML) is one of the most common types of leukemia, a cancer of the blood and bone marrow. Standard treatment involves chemotherapy, but most patients develop resistance and succumb to this disease. Immunotherapy has the potential to cure AML, but again, most patients develop resistance. Immunotherapy helps the body's immune system target and kill cancer cells by detecting antigens found on cancer cells. Immunotherapy resistance occurs when not enough antigens are present to detect the cancer cells. My lab previously found that a class of drugs called SMAC mimetics can boost cancer antigen presentation and enhance the efficacy of immunotherapy, but it is unclear whether these drugs will work for AML patients. This study will assess the efficacy of SMAC mimetics for AML patients, providing a novel approach to improve patient outcomes.

A New Noninvasive Test for Monitoring Cancer Treatment Response

Liangliang Hao, PhD
Boston University

Immunotherapies that use a person's own immune system to fight tumors have revolutionized the way we treat cancer. One promising immunotherapy strategy is a class of drugs called bispecific T cell engager (BiTE) antibodies, which make use of a patient's T cells to help destroy cancer cells. However, this method is limited by difficulties in determining early on whether the treatment is working. In this study, we propose a novel monitoring test to predict how well the tumors are responding to BiTE treatment. This test will measure the activity of tumor-producing proteases, enzymes that are involved in many processes of cancer progression. During treatment, proteases shed specific sequences of DNA that are excreted in urine. By analyzing these DNA "barcodes," we can predict treatment efficacy or resistance to therapy. This test would be convenient and noninvasive, with a simple urine sample on a paper-based system similar to home pregnancy tests. This test could bring sophisticated cancer monitoring to under-resourced areas at low cost.

The PhRMA Foundation grant provides tremendous support for me to hit the ground running during my early independence. This award will help my lab develop noninvasive diagnostics for disease detection and treatment monitoring at the point of care, and ultimately, make precision health care accessible for cancer patients.

Liangliang Hao, PhD
Boston University

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Michy P. Kelly, PhD
Associate Professor, Dept. of
Anatomy & Neurobiology
Center for Research on Aging
School of Medicine
University of Maryland

Michelle Kimple, PhD
Associate Professor,
Endocrinology, Diabetes &
Metabolism
School of Medicine
and Public Health
University of Wisconsin

Fernanda Laezza, MD, PhD
Mary J. Palmer Saunders Professor
Graduate Program Director
Dept. of Pharmacology
& Toxicology
University of Texas Medical Branch

Alastair Lawson, PhD
Vice President and Senior Fellow
UCB

Frank Lee, PhD
Vice President, Biology
Angex Pharmaceuticals, Inc.

Erika Mathes Lisabeth, PhD
Assistant Professor & Director
of Assay Development & Drug
Discovery Core
Dept. of Pharmacology
and Toxicology
Michigan State University

Ditte Lovatt, PhD
Associate Principal Scientist
Merck

Mark S. Marshall, PhD
Scientific Director of
Precision Medicine
Riley Hospital for Children

Nicholas Pullen, PhD
Chief Scientific Officer
ARTBIO

Gary O. Rankin, PhD
Vice Dean for Basic Sciences
Professor and Chair, Dept. of
Biomedical Sciences
Joan C. Edwards School of
Medicine
Marshall University

Darryle D. Schoepp, PhD
Retired as Vice President
& Therapeutic Area Head
Neuroscience, Discovery,
Preclinical and Early Development
Merck Research Laboratories

Anarug Singh, PhD
Director, Cell Biology and
Translational Research
Pfizer

Jonathan Sockolosky, PhD
Director, Company Value Creation
Curie Bio

Danyelle M. Townsend, PhD
Professor, Drug Discovery and Experimental Therapeutics
Director, Analytical Redox Biochemistry
Medical University of South Carolina

Scott A. Waldman, MD, PhD
Samuel M.V. Hamilton Professor of Medicine
Chair, Dept. of Pharmacology & Experimental Therapeutics
Director, MD/PhD Program
Thomas Jefferson University

Jun Wang, PhD
Associate Professor
Dept. of Medicinal Chemistry
Ernest Mario School of Pharmacy
Rutgers, the State University of New Jersey

Wei Wang, PhD
Co-Director, Arizona Center for Drug Discovery
Professor, Pharmacology and Toxicology
University of Arizona

Maria Wilson, PhD
Executive Director
Project Team Leadership
Genentech

Megan Yao, PhD
Retired as VP of Oncology Translational Medicine
Eli Lilly & Company

DRUG DELIVERY

Robin Bogner, PhD, FAAPS (Chair)
Professor of Pharmaceutics
University of Connecticut

Cory Berkland, PhD
Professor
Dept. of Biomedical Engineering
Dept. of Chemistry
Washington University in St. Louis

Susan "Susie" Crowell, PhD
Senior Principal Scientist
Genentech

Courtney Cullis, PhD
Head of DDS Chemistry
Takeda

Maureen D. Donovan, PhD
Professor of Pharmaceutical Sciences and Experimental Therapeutics
University of Iowa

Darin Y. Furgeson, PhD
Associate Director, Product Manager, Drug Development
Thermo Fisher Scientific

Arash Hatefi, PharmD, PhD
Professor of Pharmaceutics
Rutgers, the State University of New Jersey

Anthony "Tony" Hickey, PhD, ScD
Distinguished Fellow, Aerosol and Nanomaterials Engineering
RTI International

Jeffrey Hughes, PhD
Scientist
Invaio

Ankit Kanthe, PhD
Scientist, Drug Product Development
Bristol Myers Squibb

Margaret "Meg" Landis, PhD
Global Research & Development
Molecular Pharmaceutics/ Pharmaceutical Sciences
Pfizer

Frank Lee, PhD
Vice President, Biology
Angex Pharmaceuticals, Inc.

Tonglei Li, PhD
Allen Chao Chair
Professor of Industrial & Physical Pharmacy
Purdue University

Rushit N. Lodaya, PhD
Expert Scientist
GSK

Heidi M. Mansour, PhD, RPh, FAIMBE, SMNAI
Professor, Center for Translational Science
Professor, Robert Stempel College of Public Health & Social Work
Professor, Herbert Wertheim College of Medicine
Florida International University

Serkan Oray, PhD
Vice President
Devices, Packaging, & Wearable Technologies
UCB

Kumar Soppimath, PhD
Chief Scientific Officer
Nevakar, LLC

Srini Sridharan, PhD
Executive Director
Drug Delivery & Product Integration
Bristol Myers Squibb

Changquan "Calvin" Sun, PhD, FRSC
AAPS Fellow Professor
Director of Graduate Studies
Associate Department Head, Pharmaceutics
University of Minnesota

Adam Swick, PhD
CMC Program Lead
Moderna Therapeutics

Oleh Taratula, PhD
Associate Professor
Oregon State University

TRANSLATIONAL MEDICINE

Kathryn Sandberg, PhD (Chair)
Professor & Vice Chair of Research, Dept. of Medicine
Director, Predoc and Postdoc Training Program in Translational Biomedical Science
Georgetown University

Craig T. Basson, MD, PhD
Chief Medical Officer
Bitterroot Bio, Inc.

Anastasia M. K. Christianson, PhD
VP and Global Head,
AI/ML/Analytics & Data
Pfizer

Vicki L. Ellingrod, PharmD, FCCP, FACNP
Dean and John Gideon Searle Professor of Clinical and Translational Pharmacy, College of Pharmacy;
Professor of Psychiatry and Adjunct Professor of Psychology;
Associate Director, Michigan Institute for Clinical and Health Research (MICHR) and Director of the Education and Mentoring Group
University of Michigan

Ritika Jaini, PhD
Senior Scientist Immunology Pre-Clinical Delivery
Life Edit Therapeutics

Mehdi Javanmard, PhD
Associate Professor
Rutgers, The State University of New Jersey

Donald E. Mager, PharmD, PhD
Professor and Vice Chair
Dept. of Pharmaceutical Sciences
SUNY Buffalo State University

Jackie Pierce, PhD
Retired – Global Program Manager, Oncology Therapeutic Area Unit
Takeda

Francisco Ramirez-Valle, MD, PhD, FAA
Senior Vice President
Bristol Myers Squibb

Emanuela Ricciotti, PharmD, PhD
Research Assistant Professor of Pharmacology
University of Pennsylvania

Jonathan Sockolosky, PhD
Director, Company Value Creation
Curie Bio

Danyelle M. Townsend, PhD
Professor, Drug Discovery and Experimental Therapeutics
Director, Analytical Redox Biochemistry
Medical University of South Carolina

VALUE ASSESSMENT AND HEALTH OUTCOMES RESEARCH

Eberechukwu Onukwugha, MS, PhD (Chair)
Executive Director, Pharmaceutical Research Computing
Associate Professor, Dept. of Pharmaceutical Health Services Research
University of Maryland School of Pharmacy

Aasthaa Bansal, PhD
Associate Professor, Comparative Health Outcomes, Policy, and Economics (CHOICE) Institute
University of Washington

Benjamin Craig, PhD
Associate Professor of Economics
University of South Florida

Elizabeth Franklin, PhD, MSW
Head, US Public Affairs and Patient Advocacy, Oncology
Sanofi

Jan Hansen, PhD
Vice President, Evidence for Access Unit
US Medical Affairs
Genentech

Marianne Laouri, PhD
Executive Director, Value Demonstration Team
Boehringer Ingelheim Pharmaceuticals, Inc.

Pei-Jung "Paige" Lin, PhD
Associate Professor of Medicine
Center for the Evaluation of Value & Risk in Health, Institute for Clinical Research & Health Policy Studies
Tufts Medical Center

R. Brett McQueen, PhD
Associate Professor
Skaggs School of Pharmacy
University of Colorado

Philip Naughten, PharmD
Vice President, HEOR
Takeda

Christian Nguyen, PharmD, MBA, MS
Vice President, Global Patient Outcomes and Real World Evidence
Eli Lilly and Company

Megan O'Brien, PhD
Associate Vice President, CORE
Merck

Shelby D. Reed, PhD, RPh
Professor, Depts. of Medicine and Population Health Sciences
Director, Preference Evaluation Research Group
Center for Informing Health Decisions
Duke Clinical Research Institute
Duke University

Sarah E. Tom, PhD, MPH
Assistant Professor of Epidemiology
Columbia University

Junling Wang, PhD
Professor & Vice Chair for Research
Dept. of Clinical Pharmacy & Translational Science
College of Pharmacy
University of Tennessee

Tatia Woodward
Vice President
Value and Evidence Strategy Lead
Pfizer

Gergana Zlateva, PhD
Vice President, Patient & Health Impact, Oncology
Pfizer

Finances

STATEMENT OF FINANCIAL POSITION

As of December 31, 2023

ASSETS		2023
Cash and Cash Equivalents	$	1,396,883
Investments	$	22,970,816
Other Assets	$	113,610
Total Assets	**$**	**24,481,309**

LIABILITIES AND NET ASSETS		
Accounts Payable	$	404,521
Net Assets Without Donor Restrictions	$	24,076,788
Total Liabilities and Net Assets	**$**	**24,481,309**

STATEMENT OF ACTIVITIES

For the year ended December 31, 2023

REVENUE AND SUPPORT		2023
Contributions Received	$	3,576,216
Contributed Non-Financial Assets*	$	64,387
Interest and Dividends	$	496,762
Realized and Unrealized Investment Gains	$	2,989,975
Total Revenue and Support	**$**	**7,127,340**

EXPENSES**		
Grants and Awards	$	3,780,002
Program Services	$	28,713
Supporting Services	$	561,928
Total Expenses	**$**	**4,370,643**

**Rent and services contributed by PhRMA*

***Expenses include allocated indirect overhead costs.*

Amounts reported above are derived from the PhRMA Foundation's unaudited financial statements for the year ended December 31, 2023.

PhRMA Foundation

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