2025 ANNUAL REPORT

PhRMA Foundation 60

INVESTING IN FUTURE LEADERS

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.

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MESSAGE FROM THE

PRESIDENT AND CHAIR

While celebrating the PhRMA Foundation's 60th anniversary in 2025, we are reflecting on our impressive legacy of investment in biomedical research, exemplified by the more than 2,700 investigators who have received Foundation awards over the past six decades.

We are honored and humbled to lead an organization that remains steadfastly committed to supporting early career researchers, including graduate students, postdoctoral trainees, and new faculty. Our fellowships and grants play a pivotal role in jump-starting careers of future scientific leaders. In a survey of past awardees, 97% agreed that receiving a PhRMA Foundation award increased their confidence and their research potential.

By funding early-career investigators, the PhRMA Foundation helps bright minds stay on vocational paths that enable scientific discoveries and influence policies to improve patient health. Our scientific advisory committees have been incredibly successful in selecting scientists who remain committed to research throughout their careers, with 94% of those still employed actively engaged in research.

Our lasting impact is through our awardees. Their achievements include sequencing the first human genome, contributing to the development of cancer and COVID-19 vaccines and drugs for HIV/AIDS and type 1 diabetes, creating diagnostic tools for infectious diseases and software to run clinical trials, founding companies that develop treatments for conditions such as depression, and winning a Nobel prize. Two-thirds of our awardees have gone on to either work in industry or partner with industry, demonstrating that we are creating a strong pipeline for the future biopharmaceutical workforce.

In 2025, the PhRMA Foundation awarded 41 fellowships and grants, totaling approximately $3 million, in the areas of drug discovery, drug delivery, translational medicine, and value assessment and health outcomes research. In addition, the Foundation continues to fund four multiyear research grants related to digital health technologies (DHTs) and patient-centered value assessment. The Foundation is now considering a potential new DHTs program and has amplified our mandate regarding patient engagement in all Value Assessment and Health Outcomes Research Awards.

This year we expanded our Challenge Award program by leveraging long-time partnerships with professional associations that own scientific journals. In three Challenge Award competitions, trainees received $5,000 for authoring outstanding papers on timely topics such as artificial intelligence in clinical and translational science and the pharmacology of next-generation therapeutics. Not only do these Challenge Awards encourage trainees to think and write about important research topics, they provide opportunities for mentorship in the craft of manuscript writing and science communication.

The PhRMA Foundation is increasingly focused on communicating compelling stories about our awardees and their research, as well as training our awardees on how to better communicate their science. Our award recipients attend an educational session that covers topics including how to write a plain language summary, how to give an elevator pitch, and how to engage in a media interview. The results can be seen in the research summaries in the annual report, which are written by each award winner. Furthermore, the Foundation interviews each awardee to create a video or blog post that tells a story about their career journey and their research.

Looking back over the past 60 years, it is clear the PhRMA Foundation's fiercest champions are our former awardees. Many stay connected with the Foundation, eventually becoming review committee members or encouraging their own students to apply for our awards. In our awardee survey, 99% said the Foundation is meeting or exceeding expectations in fulfilling our mission.

We could not do this work without the unwavering support from our contributors, which include two new companies this year. However, with an unprecedented increase in interest for our awards in the 2026 cycle, there is a dire need for increased investment. We hope more pharmaceutical companies will join the Foundation in its mission to foster biopharmaceutical innovation and value-driven health care by investing in the next generation of industry-focused scientists.

Amy M. Miller, PhD
President, PhRMA Foundation

Andrew Plump, MD, PhD
Chair, PhRMA Foundation

CELEBRATING 60 YEARS OF INVESTING IN

FUTURE SCIENTIFIC LEADERS

1965

OUR FOUNDING

Over 60 years, PhF has helped seed and grow many areas of research based on the changing needs of the scientific ecosystem and industry.

1966

PHARMACOLOGY/ TOXICOLOGY

During its first two decades, PhF focused its awards on toxicology and pharmacology, closely related fields that study how drugs affect the body.

1987

PHARMACEUTICS

New PhF awards aimed to address a shortage of qualified personnel in pharmaceutics, the study of the physical, chemical, and biological properties of drugs and dosage forms.

1995

PHARMACOECONOMICS

In the face of widespread concern about rising health care costs, PhF introduced awards in pharmacoeconomics, a field that compares the clinical effectiveness of medicines and the costs.

1997

INFORMATICS/GENOMICS

PhF stayed on the frontiers of science with informatics awards that encouraged the use of computer technologies and biological databases to advance drug discovery.

Our Origin Story

The Pharmaceutical Manufacturers Association Foundation (now PhRMA Foundation) was established on May 31, 1965. Its creation came in the wake of the thalidomide tragedy, in which a treatment given to pregnant women with morning sickness caused severe birth defects in thousands of children across multiple countries, leading to a major shift in drug approval processes and greater emphasis on drug safety regulations globally.

The Foundation's initial activities focused on supporting fundamental research in toxicology and the education of personnel in clinical pharmacology and drug evaluation. For 60 years, our grants and fellowships have helped to build and train the scientific workforce, setting the stage for tomorrow's biomedical breakthroughs that could improve patients' lives. The Foundation has provided more than $110 million in research funding to over 2,700 researchers at hundreds of U.S. universities and institutions.

Our work is made possible by consistent support from major pharmaceutical companies. Some of our very first contributors were Eli Lilly and Co., Johnson & Johnson, Merck & Co., and Pfizer, all of which still support the Foundation in 2025.

Launching Research Careers

The PhRMA Foundation has a legacy of supporting young investigators — medical students, graduate students, postdoctoral trainees, and early career faculty. For most Foundation-funded researchers, our award is their first independent funding, helping them explore new areas and attract further grants. Many awardees have gone on to distinguished careers in academia, industry, or government, where they are making discoveries, influencing policy, and training future scientists. Notable alumni include Susan Band Horwitz (discovered how the cancer drug Taxol works), Arthur H. Hayes (FDA Commissioner), Louis Ignarro (Nobel Prize winner), J. Craig Venter (mapped the human genome), and Namandjé Bumpus (FDA Chief Scientist).

Awardee Impact

Results from our survey of award recipients show:

- 84% are still involved in research
- 66% either work in industry or partner with industry
- 71% have received NIH funding
- 37% have received funding from industry
- 78% mentor young scientists
- 25% said their research resulted in one or more clinical trials
- 35% said their research resulted in one or more patents
- 15% said their research led to one or more startup companies

Fostering New Fields

The Foundation has played a pivotal role in developing fields such as pharmacology, toxicology, pharmaceutics, pharmacoeconomics, informatics, genomics, translational medicine, health outcomes research, and value assessment. The Foundation is flexible and willing to fund risky research that may not have immediate results but could lead to significant breakthroughs. Check out the timeline on this page to see how our programmatic focus has evolved over the years.

Meeting the Moment

Throughout our 60-year history, the PhRMA Foundation has continually adapted to meet the moment and respond to the evolving needs of the biopharmaceutical industry. We've achieved all this while staying true to our core mission: funding future leaders who conduct innovative research that could improve patient health.

Looking forward, the PhRMA Foundation will continue to meet the moment and adjust our operations to support foundational biomedical research and cultivate future generations of researchers.

To learn more about the PhRMA Foundation's history and the results of our award recipient survey, please visit our website to read our 60th anniversary report.

2002

HEALTH OUTCOMES

With new awards, PhF strengthened the field of health outcomes research, which studies the effectiveness of health care interventions in the real world, with a focus on outcomes important to patients.

2013

TRANSLATIONAL MEDICINE

PhF wanted to bridge the gap between scientific research and clinical practice by funding scientists who bring discoveries from the laboratory to the clinic.

2016

VALUE ASSESSMENT

PhF created the Value Assessment Initiative to identify and address challenges in assessing the value of medicines and health care services, with an emphasis on patient perspectives.

2024

DIGITAL HEALTH TOOLS

The COVID-19 pandemic accelerated the adoption of DHTs, and these PhF awards recognized the potential for DHTs to foster more equitable participation in medical research.

2025

60TH ANNIVERSARY

PhF remains nimble and adaptable to invest in emerging fields of research.

2025

YEAR IN REVIEW

41
fellowships and grants totaling $3M

- **16** in drug discovery
- **5** in value assessment and health outcomes research
- **10** in drug delivery
- **10** in translational medicine

- **39%** Men
- **61%** Women

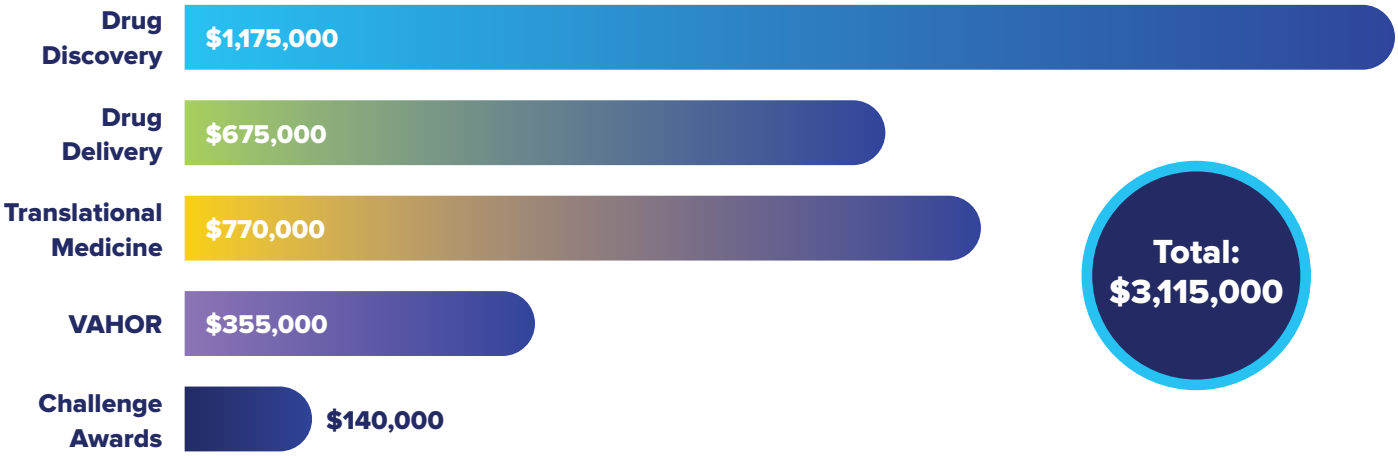
- **2.5%** Black
- **2.5%** Native Hawaiian or Other Pacific Islander
- **5%** Other
- **10%** Hispanic/ Latino
- **44%** White
- **46%** Asian

26 predoctoral students

7 postdoctoral fellows

8 faculty members

Funding by Research Area



Awardees From Around the World

The Foundation funds scientists conducting research at U.S.-based institutions.

TRAINEE CHALLENGE AWARDS:

AMPLIFYING THE VOICES OF EARLY CAREER RESEARCHERS

In honor of our 60th anniversary, the PhRMA Foundation expanded the Challenge Award program by partnering with three scientific journals on competitions specifically for trainees, including graduate students, postdoctoral fellows, and clinical residents.

In each competition, the Foundation provided $5,000 Trainee Challenge Awards to the first authors of outstanding papers submitted in response to special calls for research on timely topics. Additionally, the Foundation paid the publishing fee for the winning papers.

The PhRMA Foundation selected 14 papers for awards, for a total value of $140,000.

Artificial Intelligence and Machine Learning in Clinical and Translational Science

Six awarded papers in the October issue of *Clinical and Translational Science*, a journal of the American Society for Clinical Pharmacology and Therapeutics:

- Network Modeling of Biomarker Systems in Liver Steatosis and Fibrosis, Amruta Gajanan Bhat and Murali Ramanathan
- A Comparison of AI and Population PK Models to Predict Antiepileptic Drug Concentrations Using Therapeutic Drug Monitoring Records, Tae Kyu Chung and Howard Lee
- Application of Machine Learning for Predicting Progression-Free and Overall Survival in Patients with Renal Cell Carcinoma, Caroline W. Grant, Jerry Li, Swan Lin, Dana Nickens, Daniele Ouellet, and Mohamed H. Shahin
- Development of a Novel Machine Learning Method for Estimating Lifelong Chronic Disease Progression and Its Applications in Type 2 Diabetes, Yamato Sano*, Ryota Jin*, Hideki Yoshioka, Yuki Nakazato, Hiromi Sato, and Akihiro Hisaka
- AI-Driven Variant Annotation of Precision Oncology in Breast Cancer, Kriti Shukla, Yue Wang, Philip M. Spanheimer, and Elizabeth Brunk
- Out-of-Distribution Detection as a Risk-Control Strategy for Medical Classification Machine Learning Models, Chu Weng*, Joshua Ward*, Wesley Lin, Sherry Dong, Qi Liu, and Hanrui Zhang

**co-first authors*

Artificial Intelligence in Health Economics and Outcomes Research

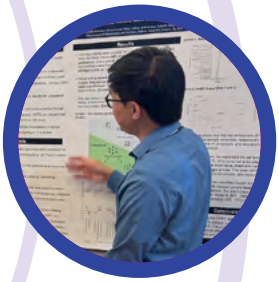
Four awarded papers in the November issue of *Value in Health*, a journal of ISPOR — The Professional Society for Health Economics and Outcomes Research:

- Unravelling Public Preferences for the Use of Artificial Intelligence Mobile Health Applications in Australia, Vinh Vo, Maame E. Woode, Stacy M. Carter, Chris Degeling, and Gang Chen
- Roles of AI-Based Synthetic Data in Health Economics and Outcomes Research, Tim C. Lai and Surachat Ngorsuraches
- Use of Large Language Models to Extract Cost-Effectiveness Analysis Data: A Case Study, Xujun Gu, Hanwen Zhang, Divya Patil, Zafar Zafari, Julia Slejko, and Eberechukwu Onukwugha
- Role of Generative Artificial Intelligence in Assisting Systematic Review Process in Health Research: A Systematic Review, Muhammed Rashid, Cheng Su Yi, Thipsukhon Sathapanasiri, Sariya Udayachalerm, Kansak Boonpattharatthiti, Suppachai Insuk, Sajesh K Veettil, Nai Ming Lai, Nathorn Chaiyakunapruk, and Teerapon Dhippayom, for the Generative Artificial Intelligence for Navigating Systematic Reviews (GAINSR) working group

Pharmacology of Next Generation Therapeutics

Four awarded papers in the December 2025 issue of *The Journal of Pharmacology and Experimental Therapeutics*, a publication of the American Society for Pharmacology and Experimental Therapeutics:

- Emerging pharmacology of targeted protein degraders, Samir H. Barghout and Mohamed A. Eldeeb
- Pharmacological considerations for next-generation protein therapeutics in cardiovascular disease, Emily Lin and Noor Momin
- Next-Generation T-Cell Engagers in Oncology: Pharmacologic Evolution from Bispecific to Trispecific Antibodies, Tarek Nahle, Viraj Shah, Sami Abi Farraj, and Ali Atoui
- Kidney-specific Delivery of an MMP-2 Inhibitory Peptide Fused to Elastin Like Polypeptide Reduces Proteinuria and Renal Fibrosis in a Model of Salt-sensitive Hypertension, Adesanya A. Akinleye, Heather M. Chapman, Richard J. Roman, and Gene L. Bidwell




Knowledge Gap
Why It Matters
Community Connection

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.

PREDOCTORAL FELLOWSHIPS

$30,000 per year of stipend support for up to two years

Bernardo Aguzzoli Heberle

University of Kentucky

RNA Isoforms: Cracking Alzheimer's Hidden Messages

RNA is a molecule similar to DNA that is essential for most biological functions. Our genes can send out many different RNA "messages" called isoforms, each with its own set of instructions for how our cells should function. These isoforms are crucial to keep our cells functioning properly, but when their balance is disrupted, they can send cells the wrong instructions and lead to disease. Until recently, scientists had to group all isoforms from a single gene into one measurement because older technologies could not tell them apart. Now, with a powerful tool called long-read sequencing, we can measure different isoforms from the same gene and better understand their impact in diseases. We're using this technology to study brain samples from people who died with Alzheimer's disease and comparing them to samples from people who died without the disease. By doing this, we hope to find disrupted isoforms in Alzheimer's disease brains — potentially revealing new targets for better treatments and earlier, more accurate diagnosis.

Azlann Arnett

Baylor College of Medicine

Reprograming CAR T Cells to Overcome Barriers in Treating Solid Tumor Cancers

Chimeric antigen receptor (CAR) therapy is a revolutionary treatment that can cure many types of cancer. The therapy works by allowing immune cells called T cells to recognize and kill cancerous cells. Unfortunately, this therapy has been ineffective on cancer types that form solid tumors. Solid tumor cancers cause the CAR T cells to eventually become dysfunctional and no longer able to effectively kill tumor cells. We have designed a new CAR therapy to treat several types of cancer cells that have a molecule called glypican-3 on their surface. While this treatment has been proven safe in our ongoing clinical trials, additional improvements are needed to cure patients. I developed a system to identify genes that prevent T cells from becoming dysfunctional or reverse the process that leads to dysfunction. The goal is to find genes that improve the function of T cells and determine how they do so. Ultimately, we plan to activate these genes to reprogram CAR T cells to effectively eliminate patients' tumors.

Jacob Capener

University of North Carolina at Chapel Hill

Creating Chemical Tools for Understudied Proteins to Advance Cancer Drug Development

For decades, drug discovery research has focused on only a handful of proteins out of the 20,000 proteins in a human cell. Often, the choice of a protein to study is influenced by the tools available to study it, rather than its actual importance. To expand the ways we can work on eliminating disease, our research aims to develop chemical tools for understudied proteins. Specifically, my research seeks to create a chemical that can deactivate an understudied protein known as CK1g that is associated with cancer. CK1g activity is known to be involved in disease-causing cellular events, yet it remains largely overlooked by biomedical research, with only a few articles detailing its functions. This chemical could act like a switch, allowing researchers to turn off the activity of this protein and gain a better understanding of its effects on cancer cell growth and survival. Developing tools like these spurs research on understudied proteins, providing new ways to combat disease.

Anh T.Q. Cong, MS

Mayo Clinic, Rochester

Finding Drugs to Protect the Heart During Chemotherapy

Anthracyclines are effective chemotherapy drugs against many cancers, but treatment often leaves patients with heart complications. Anthracyclines block proteins called Topoisomerase 2 (TOP2), including TOP2A and TOP2B in humans. Inhibiting TOP2A stops cancer DNA from being repaired during replication, but inhibiting TOP2B causes DNA damage in the heart. A drug that stops anthracyclines from binding with TOP2B while still allowing binding with TOP2A could protect the heart during chemotherapy. Currently, no such treatment exists in the clinic, and it's my goal to discover one. Good drugs fit into a protein like a key into a lock. Most of the "locks" on TOP2A and TOP2B are identical. However, I have discovered a lock on TOP2 that has subtle differences between TOP2A and TOP2B. By leveraging these differences, I am creating new molecules (keys) that are selective for TOP2B over TOP2A. Additionally, I will use cell models to evaluate the effectiveness of the molecules in protecting the heart and whether they interfere with chemotherapy. The outcome of this project will provide a group of potential agents for future studies in animal models and later clinical trials.

Claire Fleming, MS

University of Virginia

Targeting an Immune Signal That Drives Severe Disease in COVID-19

In response to an infection, the immune system activates to clear the invading infectious agent. However, if too strongly activated, the immune response can itself cause tissue damage and disease. Such is the case in COVID-19, where an overactive immune response to the SARS-CoV-2 virus can further damage the lungs and drive severe disease. Our research is focused on understanding the signals that lead to an overactive immune response to SARS-CoV-2 infection. Specifically, we are focused on a protein called interleukin-33 (IL-33). IL-33 is released from damaged cells and robustly activates immune pathways in the lungs. Using a mouse model of COVID-19, we have found that blocking IL-33 signaling is protective. My future research seeks to determine how IL-33 drives severe disease, furthering our understanding of this important and therapeutically relevant pathway in COVID-19.

> **“The receipt of the PhRMA Foundation fellowship was very impactful to me. Of course, the graduate student stipend is very helpful, but it’s also a form of external review of your graduate work and external validation that the work that I’m doing in the lab everyday is valuable and promising.”**
>
> **Claire Fleming, MS**

Thomas Kuret, MS

Thomas Jefferson University

GUCY2C Proteins Protect Brain Cells From Parkinson's Disease

Parkinson's disease (PD) is a common brain disorder that makes it hard to move and control muscles, but its exact cause is unclear. Scientists believe a combination of genetic and environmental factors are to blame. New findings also link brain cell damage in PD patients to inflammation caused by misfolded proteins (α-synuclein) or COVID-19 infection. This damage is caused by stressed mitochondria, the energy-producing parts of a cell. When damage accumulates, treatments can manage symptoms but not stop the disease from getting worse. Our project focuses on a protein called GUCY2C, found in brain cells that produce dopamine, a chemical that helps control movement. When activated, this protein protects cells from damage by boosting mitochondrial health. GUCY2C is naturally increased in PD and after COVID, suggesting it's the brain's way of protecting itself. We aim to use existing FDA-approved drugs to activate GUCY2C and thereby prevent or reverse brain cell death. This approach could shift how we treat PD, from managing symptoms to slowing or even stopping the disease itself.

Sergei Kutseikin

Scripps Research Institute

A New Approach to Treating Fatty Liver Disease

Metabolic dysfunction-associated fatty liver disease (MAFLD) is a condition where fat builds up in the liver, and in its severe form, metabolic dysfunction-associated steatohepatitis (MASH), this buildup leads to inflammation, liver cell damage, and liver scarring. These disorders are more common in people with metabolic conditions like type 2 diabetes or obesity. There are limited approved treatments, and development of new therapies is challenging given the disease is driven by a complex mix of metabolic issues. A common feature in obesity-linked organ defects is an imbalance in the cell's natural stress response system. We have developed drugs that help fix this by boosting the protective signals of the stress response system. Studies in obese-diabetic mice show treatment with these drugs improved metabolic health. My project will investigate these drugs in MAFLD/MASH mouse models and uncover their molecular mechanism of action. Our goal is to develop effective new treatments for MAFLD/MASH, improving liver function and overall metabolic health.

Texia Loh

Sanford Burnham Prebys Medical Discovery Institute

Targeting DNA Repair: A New Approach to Improve Treatment for Small Cell Lung Cancer

Small cell lung cancer (SCLC) is an aggressive malignancy with limited long-term treatment success. The five-year survival rate remains around 7%. In SCLC, the cancer cells produce a high level of PARP enzymes, which help fix broken DNA so the cancer cells can keep growing and dividing. PARP inhibitors have shown promise in preclinical studies. However, early clinical trials yielded modest responses, emphasizing the need for strategies to enhance their effectiveness. DNA helicases are enzymes that "unzip" the DNA double helix, allowing the cell to repair its genetic material. Recent data suggest that loss of a DNA helicase enhances sensitivity to PARP inhibitors. We developed a novel small molecule drug that leverages the cell's own protein disposal system to eliminate the helicase. Our initial results show that helicase degradation works synergistically with the FDA-approved PARP inhibitor Olaparib to suppress SCLC cell growth. This strategy may enable more effective, targeted treatments for SCLC.

Wei "Adelyn" Tsai

Mayo Clinic, Jacksonville

Boosting Resilience to Alzheimer's Disease Through the 'Powerhouse' of the Cell

In Alzheimer's disease (AD), an abnormal build-up of protein plaques kills brain cells, leading to memory loss and other symptoms. Two main risk factors of AD are aging and inheriting a specific version of the apolipoprotein E gene, called APOE-ε4. However, some people with these risk factors do not develop dementia or protein plaques. These individuals are considered "resilient," but the reasons for their resilience are not well understood. Our previous work found that increased expression of a gene, NDUFV1, is linked to resilience in APOE-ε4 carriers aged 80 years or older. NDUFV1 regulates the function of the mitochondria, the powerhouse of the cell. In this project, we first aim to develop gene therapy that can boost expression of NDUFV1. We plan to test the efficacy of this therapy using neurons grown from stem cells of AD patients or normal donors. We will also explore new mitochondrial mechanisms and genes in different brain cell types that may protect against AD in elder people. This study will contribute to the discovery of new drug targets by leveraging mitochondria to increase resilience to AD.

Copper Depletion as a New Treatment Strategy for Pediatric Leukemia

Alan Wong

Harvard Medical School

In the same way that humans adapt to new environments to survive, so too do cancer cells. When cancer cells spread to different parts of the body, they adapt to different nutrient environments. In childhood leukemia, it's especially deadly when cancer cells spread to the brain. In our work, we are studying whether adapting to different environments might create vulnerabilities in leukemia cells that can be targeted by drugs. We have shown that leukemia cells depend on copper, an essential nutrient that they exploit to stimulate their growth and spread. In mouse models of human leukemia, restricting dietary intake of copper slows the growth of systemic leukemia and leukemia in the brain, and improves the effect of existing therapies. This work could broaden the effective therapies for leukemia, with special attention to the spread of leukemia to the brain, helping to improve long-term outcomes for childhood leukemia patients.

In Vivo CAR T Cell Engineering for More Effective, Affordable Cancer Treatment

Jingyu Zhao, MS

University of North Carolina at Chapel Hill

Chimeric antigen receptor (CAR) T cell therapy is a powerful treatment for several blood cancers that works by reprogramming a patient's own T cells to find and kill cancer cells. However, the process of extracting the patient's T cells, modifying them in a lab, and then reinjecting them into the patient is complicated, time-consuming, and expensive. Our research aims to simplify this process by creating CAR T cells directly inside the patient. We will use viruses specially engineered to safely deliver the CAR gene to the patient's T cells. To improve the efficacy, we also combine the virus with certain chemicals that enhance its effectiveness. In preliminary studies, this system successfully produced CAR T cells and eliminated B cell lymphoma tumors in animal models. We are now testing this system against different solid tumors, such as lung cancer and pancreatic cancer, in animal models, evaluating its effectiveness, safety, and potential side effects. If successful, this approach could make CAR T cell therapy more accessible and affordable for patients.

POSTDOCTORAL FELLOWSHIPS

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

Aakriti Gangwal, MSc, PhD

Stanford School of Medicine

Broad-Spectrum Antivirals: One Treatment for Many Viruses

Viruses like dengue and COVID-19 hijack our cells' machinery to spread, making it difficult to treat them with traditional drugs that target only one virus at a time. My research explores a smarter approach: blocking key human proteins — called kinases — that many viruses need to survive. We've discovered that targeting certain kinases can protect mice from deadly viruses without harming healthy cells. I'm now testing a new drug that precisely blocks one such kinase family called Numb-associated kinases (NAKs) and shows strong promise against dengue virus. I'm also working to map the signaling pathways that NAKs regulate to better understand how they help viruses so we can develop safer, more effective antiviral drugs. This work could lead to improved broad-spectrum antiviral drugs and better prepare us for future outbreaks. With this fellowship, I aim to turn these discoveries into effective treatments and grow as an independent scientist focused on fighting infectious diseases.

> "As a young scientist, you often have bold ideas but not always the resources to test them. Being selected for this award gave me confidence in my project and independence to take risks. Funding early-career researchers is vital because this is when creativity is at its peak, and supporting that can spark discoveries that might otherwise be missed."
>
> **Aakriti Gangwal, MSc, PhD**

Erick Rodriguez-Palma, MSc, PhD

University of Florida

A New Hope for Fibromyalgia: Blocking the Channels Behind Pain

Fibromyalgia is a long-lasting condition that causes widespread pain and affects millions of people, especially women. Current treatments do not work well for everyone and cause serious side effects. Even though fibromyalgia is common, we still do not fully understand what causes it. My research focuses on a protein called CRMP2, which helps control sodium and calcium channels — tiny gates in nerve cells that send pain messages in the body. If we can understand how CRMP2 affects these channels in fibromyalgia, we might find new ways to reduce pain. I'm testing a new drug called A5-14, which is designed to block CRMP2 activity. My goal is to see whether this drug can reduce pain in an animal model of fibromyalgia. If it works, this could lead to a new kind of treatment that does not rely on opioids and may be safer for people living with fibromyalgia.

Allison Siegenfeld, MS, PhD

Harvard Medical School

Understanding a Tiny RNA Modification With an Outsized Function in Acute Myeloid Leukemia

A hallmark of cancer is the dysregulation of messenger RNA levels. Messenger RNA (mRNA) harbors the instructions to make the proteins that perform essential functions in the cell. Small chemical modifications to mRNA can impact protein production through mechanisms that are intensely debated. We study the most common mRNA modification, N6-methyladenosine (m6A), which is overabundant in cancer. Notably, a drug that blocks m6A modifications has entered clinical trials to treat cancer, raising great interest in better understanding how m6A influences the RNA lifecycle. My research combines fast-acting clinically relevant drugs that target m6A with cutting-edge techniques to measure the drugs' impact on mRNA synthesis, maturation, and stability. By providing a comprehensive understanding of the effects of m6A, my work aims to clarify the mechanism of action of the promising m6A inhibitors and propose new therapeutic strategies.

FACULTY STARTER GRANTS

$100,000 for one year of research project support

Elizabeth Brunk, MSc, PhD

University of North Carolina at Chapel Hill

Tracking and Targeting Cancer's Evasive DNA Escape Routes

Some of the most aggressive cancers evade treatment using rogue circles of DNA called extrachromosomal DNA (ecDNA), which live outside the chromosomes in our cells. ecDNA carry extra copies of cancer-driving genes like HER2 and MYC. ecDNA can "shape-shift" back into chromosomes to temporarily hide from treatments and then re-emerge later, making tumors hard to kill. My lab uses artificial intelligence to analyze thousands of high-resolution images to track how ecDNA disappears and reappears during treatment. We've discovered that this process happens in cells with *less* ecDNA and that the drug used can influence how often it occurs. We are combining this AI-driven imaging with cutting-edge single-cell genomics to understand the DNA and gene activity of individual cells and block these DNA-based adaptations that help cancer. This research could lead to new treatments that block cancer's ability to adapt and resist therapy, transforming how we treat hard-to-target tumors.

Noor Momin, PhD

University of Pennsylvania

Treating Irregular Heartbeat by Targeting the Immune System

Atrial fibrillation (AFib) is an irregular heartbeat that affects many older adults. Current treatments help prevent complications like stroke but don't fix the underlying problem. AFib happens when heart tissue becomes scarred. This scarring is partially caused by immune cells in the heart that release a harmful protein. The scarring disrupts normal electrical signals and causes irregular heartbeats. We will create a combination therapy that links two parts: an antibody (which works like a GPS to find the right cell) and a gene-silencing molecule called a small interfering RNA, or siRNA (which halts the production of the culprit protein). The antibody will guide the siRNA therapy directly to the immune cells in the heart. Once there, the gene-silencing molecule will stop these cells from making the protein that causes scarring. In our early tests with mice, this approach has successfully reduced irregular heartbeats. We are now focused on clinical translation by 1) testing efficacy in human tissues and 2) optimizing delivery. This therapy could fix the root cause of AFib instead of just managing symptoms. If successful, some patients might be freed from AFib completely. This approach could potentially be used for many other diseases where specific cells need to be targeted.

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.

PREDOCTORAL FELLOWSHIPS

$30,000 per year of stipend support for up to two years

Ameya Chaudhari, MS

University of North Carolina at Chapel Hill

Designing Innovative Skin Meshes for Accelerated Diabetic Wound Healing

Chronic wounds due to factors such as diabetes, obesity, and aging affect an estimated 10.5 million people in the United States. Non-adhesive wound dressings may necessitate sutures or staples for attachment, which can cause injuries to the skin, while conventional adhesive patches cannot adapt to repetitive physiological motions such as flexing and tension. To address these shortcomings, we have developed auxetic, elastic, and sticky (AuxES) skin meshes to accelerate wound healing. We screened over 60 geometric structures for the patches to identify the ones with the best auxetic properties, meaning they can expand and contract in ways that match human skin and movement properties. In addition, our meshes are drug loadable, allowing controlled release of medicines to promote healing, as well as biodegradable and 3D-printable. Because diabetic wounds are especially difficult to treat and often fail to heal on their own, we tested our AuxES patch loaded with an FDA-approved therapy for diabetic wounds in a mouse model of diabetes. We found that these drug-loaded patches significantly improved wound healing in mice.

Shruti Dharmaraj, MS

University of Maryland, Baltimore

Treating Allergies by Targeting Underlying Cellular Dysfunction

Allergies are a significant health burden in the United States, affecting over 50 million individuals annually. Current treatments like Claritin® or inhalers focus primarily on managing symptoms and are non-curative. When allergens like pollen enter our lungs, cells will “eat” the allergens and display pieces of them, called antigens, on the cell surface. This activates the immune system’s response, leading to symptoms like difficulty breathing and itchy eyes. When the immune system overreacts to an allergen, mitochondria — the “powerhouse” of the cell — can become dysregulated, affecting the production of small molecules called metabolites that are essential to mitigating the immune response. I will develop tiny drug carriers called nanoparticles derived from these metabolites to counter this metabolic dysfunction while also delivering antigens to the body to train it to ignore those allergens in the future. My preliminary data show these metabolite-based nanoparticles can effectively mitigate allergic disease. Not only will this research establish a new treatment for allergies, but it has the potential to treat other immune diseases with metabolic dysfunction.

Maria Hudock

Columbia University

Cracking the Case on Gene Therapy for Cystic Fibrosis

Cystic fibrosis (CF) is a genetic disease that causes severe lung damage over time, sometimes requiring a lung transplant or resulting in early death. Researchers have been working to create a gene therapy that would correct the genetic mutation that causes CF. However, gene therapies that have worked in animals and in human lung cells in the lab have not worked in humans. Why? Research shows that endocytosis, the process by which cells take up large objects (like gene therapy carriers), works differently in animals than in humans. It also works differently in human cells outside vs. inside the body. Using a tool that I custom-built to keep small pieces of lung alive outside the body, I will compare endocytosis mechanisms in animal and human tissue to understand exactly how this process differs, so we can better predict which gene therapies will be successful in humans. I will also identify which carrier properties (size, shape, etc.) result in more cell uptake, so we can design better gene therapy carriers.

> **“Receiving this fellowship has increased my confidence as an investigator by affirming that I can generate interesting, relevant project ideas, by helping me to practice study planning, and by bolstering me to follow through and work with even more conviction. For this, I am extremely grateful.”**
>
> **Maria Hudock**

Alina Ringaci
Boston University

Improving the Safety and Efficacy of Existing Antibody-Drug Conjugates for Cancer Treatment

Antibody-drug conjugates (ADCs) are a new type of cancer treatment that use special proteins called antibodies to deliver drugs directly to cancer cells. This targeted approach helps kill cancer cells while sparing healthy ones. Several ADCs are already used in clinics, but they often release the drug too early, before reaching the cancer cells, which affects healthy cells and causes side effects. I'm developing a new way to connect the drug to the antibody using short proteins called coiled-coil peptides. One set of these peptides is attached to the antibody, and the matching set is attached to the drug. When mixed together, the peptides fit like puzzle pieces, linking the drug to the antibody. This approach allows us to create well-defined and stable antibody-drug conjugates, holding the drug securely and potentially reducing the risk of the drug falling off before reaching a cancer cell.

> **"The PhRMA Foundation means a lot to me, and it really brings a lot of confidence and makes you feel you belong to the scientific community. It recognizes not just my efforts but also the efforts of the students I work with, all my mentors and collaborators."**
>
> **Alina Ringaci**

Madison Seefeld

University of Minnesota

Hitchhiking Across the Nose's Mucosal Lining to Improve Vaccines

Vaccines administered through the nose via mist are an appealing alternative to injection-based vaccines and could better protect against respiratory pathogens. However, the mucosal lining in the nose limits the uptake of vaccine components. To combat this challenge, my lab is engineering vaccines that hitchhike on the body's natural transport system to cross mucosal barriers. Vaccines train the immune system to recognize antigen, the component of the pathogen that the immune system responds to during natural infection. Pre-exposing the body to antigen allows the immune system to respond more rapidly to that pathogen in future infections. We will link our vaccine antigen to albumin, one of the most abundant proteins in the body, so that it can hitchhike across mucosal barriers, overcoming the challenge of uptake. I will study how the location of our antigen impacts the immune response after mucosal vaccination and apply this platform to other diseases.

Xinyi Tu

Arizona State University

Developing a Platform for Personalized Cancer Vaccines

Our bodies are protected by the immune system, which can find and eliminate cancer cells before they grow into a problem. But cancer can sometimes hide or shut down the body's normal defenses, allowing it to grow unnoticed. Cancer vaccines are a new kind of treatment that help "wake up" the immune system and train it to recognize markers found only on cancer cells. Once trained, the immune system can find and destroy cancer. Our team is developing a vaccine platform that uses nanoparticles to deliver the vaccine ingredients to the immune system. Our platform uses RNA molecules specially designed to grab the attention of the immune system and trigger a response against cancer cells. Our approach does not use materials that cause harmful side effects like fever and allows for customization for different cancer types. In short, we are creating a smarter, safer way to teach the immune system how to fight cancer — like giving it a "wanted poster" and ringing the alarm bell at the same time. If successful, our platform could become a powerful tool to help more patients benefit from cancer immunotherapy.

POSTDOCTORAL FELLOWSHIP

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

Shamimur Akanda, PhD

Washington University in St. Louis

Computational Modeling to Predict Drug Behavior in Treating Joint Diseases

Injecting medicines directly into the joint is an efficient, targeted approach to treating diseases such as osteoarthritis. Many factors influence a drug's residence time in the joint, or how long it remains attached to its biological target, including the properties of the drug itself, the interaction between the drug and surrounding tissues, and the material carrying the drug. Using these parameters, I developed a computational model for predicting drug residence time in the joint after being injected with and without a slowly releasing drug carrier. This model successfully predicted that drugs from a slow-release carrier have elevated residence time in the joint and are associated with a lower peak drug concentration in the tissue. I plan to validate this model by tagging drugs with fluorescent markers that can be traced as they move through small animal joints to compare residence time for drugs delivered within and without a drug carrier. The ability to successfully predict a drug's residence time could help improve the design of preclinical experiments and reduce the number of animal studies normally needed to figure out drug dosing for joint disease treatments.

> **"I'm really humbled to receive this award. It's not just financial support, it's also an acknowledgement of confidence for my research project. Early career scientists often work on high-risk, high-gain projects and having stable funding helps us to explore the new ideas and take those risks."**
>
> **Shamimur Akanda, PhD**

Brittany Hartwell, PhD
University of Minnesota

Activating Frontline Immune Defenses in the Nose with Hitchhiking Vaccines

To combat infectious threats like HIV, SARS-CoV-2, and influenza, vaccines are needed that trigger immune protection in places like the nose, where pathogens enter the body. While traditional injected vaccines are effective at activating "backup" defenses in the blood, they do not typically activate robust "frontline" defenses in mucosal tissues where transmission takes place. Vaccinating directly at mucosal surfaces — for example, through the nose — can promote mucosal immunity. However, vaccine delivery across mucosal barriers is challenging because they are very good at keeping most vaccine components out. Yet the naturally occurring protein albumin is known to be very good at getting in. Albumin, a major blood protein also found in mucosal fluids, is shuttled across mucosal barrier tissues by binding a receptor on its surface. Exploiting this biology, my lab is developing intranasal vaccines that "hitchhike" on albumin to deliver immune cargo in the nose to activate frontline mucosal immunity against infections like COVID-19 and influenza. We hope this engineering strategy of hitchhiking to bypass mucosal barriers will result in delivery of vaccines that are safer and more effective at protecting against infectious diseases.

Zhimin Huang, PhD
University of Pittsburgh

Engineering Noninvasive, Site-Specific Therapeutics for Brain Diseases

Many brain disorders arise from disruptions in specific brain regions, yet they are often treated with small molecule drugs that spread throughout the entire brain, affecting both diseased and healthy areas. This lack of spatial precision can lead to reduced efficacy and unwanted side effects. To address this, I developed Regionally Activated Interstitial Drugs (RAID), a strategy that uses focused ultrasound to deliver an engineered enzyme directly to selected brain regions. This enzyme locally converts an inactive drug — given to the whole body — into its active form only where needed. Unlike conventional therapies, RAID enables localized drug activation for several days without repeated ultrasound. Preliminary studies have shown successful enzyme delivery, region-specific drug activation, and behavioral changes consistent with the drug's effects. We now aim to optimize RAID by extending enzyme retention and testing its potential in an animal model of depression. If successful, RAID could provide a noninvasive, long-lasting, targeted treatment for neurological and psychiatric disorders with fewer side effects.

Lisa Volpatti, MS, MPhil, PhD

Northwestern University

Turning Bad Cholesterol Into a Good Drug Delivery Vehicle

Obesity-related diseases are rising at an alarming rate, and many are fueled by chronic inflammation that current treatments are unable to fully control. Some drugs that modulate the immune system to reduce inflammation can help, but they often cause serious side effects that limit their long-term use. To address this challenge, we are creating a new kind of precision therapy that works with the body's natural systems. Our approach uses "bad cholesterol" particles as vehicles to deliver medicine directly to inflamed tissues. By designing proteins that attach to these particles, we can build nanoscale delivery systems that travel to diseased areas while sparing healthy ones. In this project, we will explore how different versions of these delivery systems behave in the body and identify the safest and most effective ways to administer them. This innovative platform could transform how we treat inflammation and unlock new therapies for a wide range of diseases.

> **"As an early career scientist, I don't have the track record or the preliminary data to compete with very well established labs. I'm very grateful for the PhRMA Foundation funding to enable me to build that track record in my independent lab and gain that preliminary data to allow me to compete for more grants in the future. It's truly a starting off point that will really enable a successful career."**
>
> **Lisa Volpatti, MS, MPhil, PhD**

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 predoctoral students, postdoctoral trainees, and early-career faculty working in collaboration with clinicians to develop new diagnostic, experimental, and computational approaches and technologies to improve patient care and management.

Eleanor Agosta
Rutgers University

Identifying a Novel Biomarker for Cervical Cancer Progression

While most human papillomavirus (HPV) infections resolve on their own, a small percentage can lead to cancers like cervical cancer. HPV is responsible for over 90% of cervical cancers. In HPV-related tumors, some genes are amplified, meaning that cancer cells have extra copies of those genes. These amplified genes are an important puzzle piece to understanding how HPV causes cancer. My project focuses on a highly amplified gene in cervical tumors, *TP63*, which can produce multiple versions, called isoforms, of its protein. I found that a subset of *TP63* isoforms is highly expressed in cervical tumors, and I am testing whether introducing these isoforms into healthy cells makes them behave like cancer cells. This work could reveal a key mechanism of HPV-driven tumor development and lead to biomarkers that identify high-risk patients, enabling earlier and more targeted prevention strategies.

> **Funding young scientists is so important, especially now when the value of scientific research is being questioned in a very public way. The Foundation award is a much needed vote of confidence that the work I'm doing as a young scientist is valuable.**
>
> **Eleanor Agosta**

Finding Therapeutic Targets for Cerebral Malaria

Alan Ardito
Brown University

Plasmodium falciparum malaria claimed over 600,000 lives in 2023, with over 90% of deaths occurring in children under the age of five in sub-Saharan Africa. While many patients experience mild symptoms, severe disease can manifest when parasite-infected red blood cells cluster in major organs. Cerebral malaria, the deadliest form of malaria, occurs when parasite-infected red blood cells sequester within the brain, causing serious harm to the circulatory system that nourishes the brain. Even with treatment, 15-30% of cases are fatal. Surviving patients often experience long-term neurological defects, like speech disorders, learning disabilities, and blindness. Using lab-created models of the brain's blood vessel system, my research aims to understand the parasite's machinery responsible for damaging the cerebral vascular system. Identifying these mechanisms will highlight new therapeutic targets to prevent the worst outcomes of cerebral malaria.

Boosting the Efficacy of CAR T Cell Therapy for Colorectal Cancer

Miao Cao
Thomas Jefferson University

Chimeric antigen receptor (CAR) T cell therapies have revolutionized the treatment of blood cancers. Yet none have been successful in clinical trials for "solid" tumors, such as colorectal cancer (CRC), the second leading cause of all cancer deaths. CAR T cell therapy reprograms a patient's own T cells to find and kill cancer cells by targeting specific surface markers (antigens). Guanylate cyclase C (GCC) is a membrane receptor expressed by nearly all CRCs and is a leading target for CAR T cell therapy development in CRC. Preclinical studies have shown mixed success. Failure of CAR T cell therapy in solid tumors reflects in part the loss of target antigens on cancer cells, but the mechanisms underlying this target loss are not fully understood. We have identified a novel mechanism by which CAR T cell therapy shapes the tumor environment (we call this CAR-TIME), causing cancer cells to lose the target antigen, GCC. Our project will further define the mechanisms underlying CAR-TIME and explore approaches to overcome it and improve CAR T cell therapy for CRC.

Annalisa Ferrotta

Weill Medical College of Cornell University

Understanding Failure of PI3K Inhibitors in Treating Glioblastoma and Evaluating New Therapeutic Options

Glioblastoma (GBM) is the most common brain tumor in adults and is known to be highly aggressive. The five-year survival of GBM patients is only 5%, due in part to ineffective therapies that have remained unchanged for two decades. The phosphoinositide 3-kinase (PI3K) pathway, which promotes cell growth and proliferation, is overactivated in nearly all GBM patients. This led to the belief that drugs that inhibit PI3K would be effective treatments for GBM. Unfortunately, clinical trials investigating PI3K inhibitors in GBM have all failed, despite PI3K inhibitors being effective treatments for other cancers, such as lymphoma. My research seeks to understand and validate two potential biological mechanisms that may cause resistance to these PI3K inhibitors in GBM. I will also evaluate a new generation of PI3K inhibitors in patient-derived models of GBM to identify more effective therapeutic approaches.

Ariel Leyte-Vidal

University of Miami

Understanding and Overriding Resistance to Asciminib in Chronic Myeloid Leukemia

Chronic myeloid leukemia (CML) is a blood cancer that arises from the fusion of two genes, BCR and ABL1. The resulting BCR-ABL1 protein promotes uncontrolled growth of blood cells. CML can be treated with drugs that block the activity of BCR-ABL1, but resistance occurs in some patients. At the molecular level, resistance to these drugs is most commonly due to mutations that prevent their ability to bind BCR-ABL1. In 2021, the FDA approved a drug called asciminib, which binds to a different site on the BCR-ABL1 protein than previous drugs, locking the protein in an inactive state and stopping it from causing uncontrolled cell growth. However, some of the first cases of asciminib resistance are now emerging, and to date, all are associated with new mutations in BCR-ABL1. I hypothesize that most of these mutations will not impact drug binding but rather prevent the changes that asciminib normally causes to inactivate BCR-ABL1. Using assessments of drug binding and protein structures, my work seeks to molecularly dissect the mechanisms responsible for resistance to asciminib.

Noe Mercado

Brown University

Investigating the Role of a Common Human Virus in Brain Tumors

Glioblastoma is a deadly brain tumor with very limited therapies currently available. The current standard of care has not changed since 2005, and patient survival has not significantly improved past 14 months. Recent reports have indicated that pathogens like viruses contribute to glioblastoma growth. Our lab focuses on understanding the role of how a widespread human virus called cytomegalovirus (CMV) may drive glioblastoma growth. CMV persists in the body after primary infection, with 60-90% of the population being infected. Thus, targeting CMV may provide a new therapeutic strategy to treat glioblastoma patients. The goal of my research is to assess the changes in glioblastoma after CMV infection and to determine whether targeting the virus using antiviral drugs may provide therapeutic benefit. Ultimately, my data will provide new insights into glioblastoma tumor biology and identify new therapeutic approaches that previously have not been considered in the clinic.

> **I am incredibly honored to receive the PhRMA Foundation predoctoral fellowship. This award will support my efforts to find novel therapeutic approaches to treat brain tumors like glioblastoma and make a meaningful impact in my field.**
>
> **Noe Mercado**

Nathan Campbell, PhD

University of Mississippi Medical Center

Evaluating the Role of T Helper Cells in Development of Preeclampsia and Related Postpartum Complications

Pregnant people can develop a disease called preeclampsia that is characterized by high blood pressure late in pregnancy and can have life-threatening complications if left untreated. Although the frequency of preeclampsia has increased in the past two decades, there have been no new treatments in 50 years. People with preeclampsia show increased activation of B cells, white blood cells that can produce harmful proteins called angiotensin II type 1 receptor autoantibodies (AT1-AA) that mistakenly target the body's own cells. We have shown that another type of white blood cell, a T helper cell, is crucial in activating B cells producing AT1-AA. Research in animal models shows that AT1-AA exposure during pregnancy causes neurological complications after pregnancy as well as cardiovascular complications for offspring as adults. This project aims to test whether a new drug that lowers AT1-AA activity can mitigate these long-term complications in a novel model of preeclampsia that involves transfer of human T helper cells into pregnant rats. This will help determine how T helper cells from human patients contribute to the development of preeclampsia and disease risk. The goal is to improve maternal outcomes and reduce future disease risk for people who experience preeclampsia and their children.

Yu-Hsiang Chen, MS, PhD

The University of Texas MD Anderson Cancer Center

Detecting and Tracking Residual Cancer Cells After Treatment to Prevent Relapse

Myelodysplastic syndrome (MDS) is a blood cancer that can only be cured with stem cell transplantation, which replaces malignant cells with healthy donor blood and stem cells. However, about half of patients still relapse and die. Sensitive tools have been developed to detect small numbers of residual cancer cells that predict relapse. Yet we still don't fully understand what these cells look like or why they survive. We suspect these residual cancer cells can evade attack by donor immune cells. Our study uses a new single-cell technology not only to detect these cells but also to inspect individual malignant cells for their RNA, protein, and genetic features. This work tracks changes in individual cells over time to show how the residual cancer cells evolve and avoid attack from donor immune cells. By understanding these changes, we can develop better treatments to prevent relapse and improve outcomes for patients.

> **The PhRMA Foundation award provides me with the essential resources to advance my research. This support strengthens our mission to make a meaningful impact on cancer diagnosis and treatment.**
>
> **Yu-Hsiang Chen, MD, PhD**

Samagya Banskota, PhD
Boston University

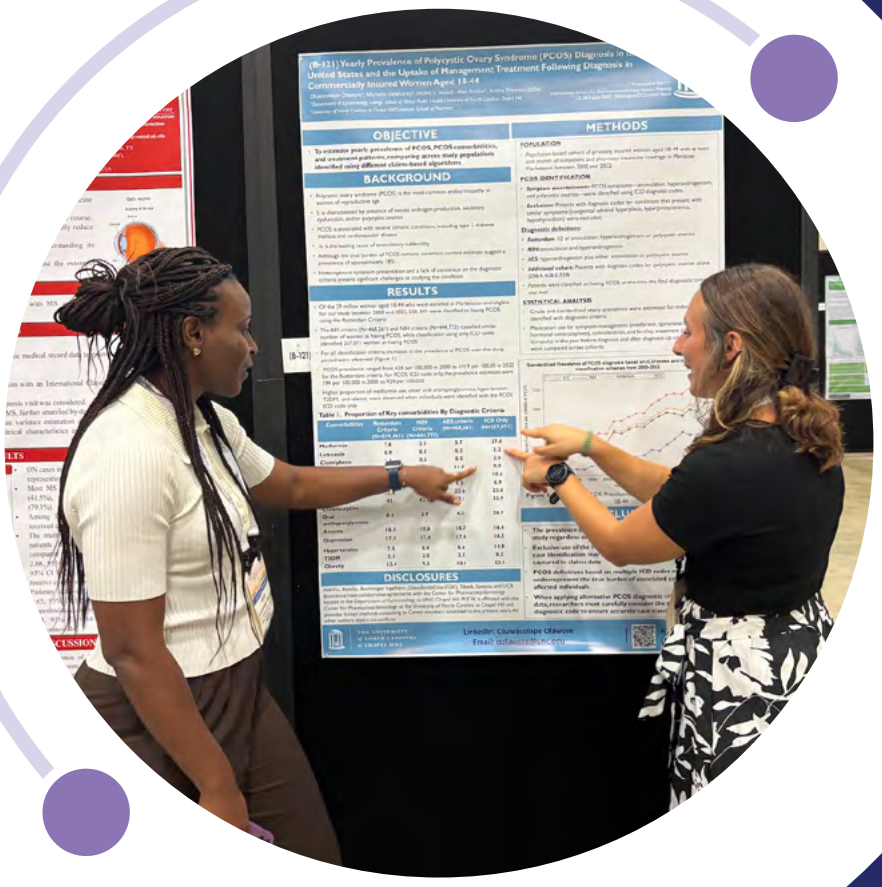
Advancing Precision Genome Editing Therapies for Treatment of Rare Genetic Diseases

Approximately 1 in 10 Americans is affected by a rare genetic disease, half of those are children. Children with rare genetic diseases often have limited, if any, viable treatment options and frequently succumb to disease by mid-adulthood. My research aims to address this unmet clinical need by developing precision genome editing therapeutics that allow scientists to correct the mutations that cause genetic diseases. For example, base editing allows scientists to precisely change one of the four single DNA base letters without breaking the DNA double helix, which can lead to errors or unwanted mutations. Despite their promise, these technologies have not yet been widely used to treat rare genetic diseases. My research seeks to advance the use of base editing therapies for Wolfram syndrome (WS) — a rare and life-threatening genetic disorder. In this project, we aim to develop disease models to better understand the underlying biology of WS and to optimize and evaluate the therapeutic potential of genome editing and delivery modalities. Our long-term goal is to create safe, effective, and novel one-time treatments for rare genetic disorders like WS.

Ying Liu MD, PhD
Johns Hopkins University School of Medicine

Tiny Packages, Big Clues: Exploring Retinal Cell-Derived Particles for Early Detection and Treatment of Vision Loss

Retinal degeneration is a group of eye diseases that gradually damage the retina, the light-sensing layer at the back of the eye that allows us to see. This damage can cause severe vision loss or even blindness. Unfortunately, these diseases are often not detected until vision loss has occurred, and no effective treatments exist. This is mainly because we lack good tools for catching the disease early and do not fully understand how retina cells interact as the disease develops. To address this, we are studying tiny particles called extracellular vesicles (EVs) from diseased retinas. These EVs can act like tiny packages, carrying early warning signs and helping us understand how retina cells communicate during disease. In our research, we grow patient-derived stem cells into mini retinas in a dish to mimic disease. We then study the “EV packages” the retina cells send out, focusing on their ID tags (surface markers), cargo (disease-related molecules), and how they travel between cells (delivery mechanisms). If successful, this study could open the door to earlier diagnosis and new ways to slow or rescue vision loss in people with retinal degeneration.



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.

PREDOCTORAL FELLOWSHIPS

$30,000 per year of stipend support for up to two years

Tim Lai

Auburn University

Leveraging Real-World Data and Patient Preferences for More Effective Treatment of Major Depressive Disorder

Many people with major depressive disorder do not fully recover after their first treatment and continue to experience symptoms. If these symptoms are not treated effectively, they can get worse over time. That is why it is important to identify, as early as possible, which patients may not respond to initial treatment and may need different or more advanced care. Unfortunately, there is not enough research to clearly guide what treatments should come next, making it hard for clinical practitioners to create personalized care plans. In this study, I will first determine which follow-up treatment approaches work best by examining real-world data from claims and self-reported questionnaires. I will survey patients to figure out which treatment approaches they prefer. I will also develop a tool to help identify patients who are at higher risk of not responding to their first-line treatment. These findings will help health care providers offer more effective and patient-centered care.

> **“The award is very valuable for young scientists like me. The entire application process itself is a learning process because we know that, as a scientist, we need to know how to communicate our scientific work to outside audiences. So with this application process, we can have this practice opportunity.”**
>
> **Tim Lai**

Oluwasolape Olawore

University of North Carolina at Chapel Hill

Evaluating the Long-Term Developmental Safety of Antenatal Corticosteroids for Preterm Birth

Antenatal corticosteroids are an important treatment given to pregnant people at imminent risk of preterm birth to accelerate the baby's lung development. Yet there are concerns about how exposure may affect the child's long-term brain development. Results from clinical trials and real-world studies are conflicting, likely because of differences in study populations and systematic errors that can skew findings. My research uses machine learning to identify pregnant people who are at high risk of preterm delivery by analyzing routinely collected clinical and demographic data. I will examine electronic medical records to understand how receiving antenatal corticosteroids influences children's long-term brain development, comparing those who did and did not receive the treatment. By using innovative methods to answer this safety question, my project will supply clear evidence to guide decisions on when and for whom antenatal corticosteroids offer the greatest benefit, helping doctors tailor care and improve outcomes for vulnerable infants.

Nha Tran

University of Michigan

Reducing Diabetes Overtreatment in Older Adults: A Patient-Centered Approach to Deprescribing

Overtreatment of diabetes in complex older adults is prevalent and can increase the risk of harmful side effects, falls, cognitive impairment, and hospitalization. Complex older adults with diabetes (COADs) are individuals with multiple chronic conditions, functional limitations, or cognitive impairment. Deprescribing, the process of reducing or stopping inappropriate medications, is key to addressing overtreatment. Successful deprescribing efforts depend on shared decision-making between patients and their clinicians. My research aims to understand what factors influence older adults when deciding whether to discuss deprescribing with clinicians. Using a nationally representative survey linked with claims data, I will examine how patient characteristics relate to willingness to deprescribe among COADs. I will also conduct a survey and interviews with overtreated COADs to explore what motivates or prevents them from initiating deprescribing conversations with clinicians. By leveraging real-world data, my research will identify patients more open to deprescribing and improve understanding of the barriers faced by those less likely to engage. My research will promote patient-centered conversations and reduce the risks associated with overtreatment in diabetes.

POSTDOCTORAL FELLOWSHIP

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

Maksat Jumamyradov, PhD

Auburn University

Novel Methods to Overcome Underrepresentation in Preference-Based Value Assessment

Neglecting to capture the preferences of underrepresented or small populations in health care value assessment research can lead to a mismatch between available treatments and what patients truly want. A key methodological challenge is how to elicit more preference evidence with fewer respondents. This project aims to elicit the preferences of underrepresented populations for type 2 diabetes mellitus (T2DM) treatments by using two novel survey methods that are well-suited for small samples: kaizen tasks and multidimensional thresholding. The kaizen tasks method presents a hypothetical T2DM treatment option with different attributes (e.g., side effects, dosing frequency) and asks patients which attributes they would want to improve first, second, third, etc. The multidimensional thresholding method asks patients to rank the largest possible improvement in all attributes by their importance and then has them answer a series of questions to elicit acceptable tradeoffs among different attributes. These methods aim to gather more detailed preference information per respondent, allowing for more effective and efficient inclusion of underrepresented populations in preference-based value assessment.

FACULTY STARTER GRANT

$100,000 for one year of research project support

Maja Kuharic, MPharm, MSc, PhD

Northwestern University

Care Recipient Self-Perceived Burden: How Feeling Like a Burden Affects Health Care Decisions

Over 1 in 5 Americans provide unpaid care to a loved one. Many patients who rely on this support feel guilty or worry they are a burden — a feeling known as Care Recipient Self-Perceived Burden (CR-SPB). This feeling can affect how patients make treatment decisions and impact the well-being of both the patient and the caregiver. However, little is known about how CR-SPB influences health care decisions. This project aims to better understand these feelings and their impact on care choices and quality of life. By combining interviews, surveys, and input from patients, caregivers, and health care professionals, the study will help identify ways to improve how we measure value in health care and ensure that patient experiences are better reflected in treatment planning.

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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
Dept. of Pharmaceutical Sciences
Director, Kildsig Center for Pharmaceutical Processing Research
University of Connecticut

Tonglei Li, PhD (Co-Chair)
Chair & Professor
Industrial & Physical Pharmacy
College of Pharmacy
Purdue University

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

Maureen D. Donovan, PhD
Professor
Pharmaceutical Sciences & Experimental Therapeutics
College of Pharmacy
University of Iowa

Kelly Forney-Stevens, PhD
Biologics Drug Product Leader (MSAT)
GSK

Darin Y. Furgeson, PhD
Biotech Portfolio Lead
Associate Director, Drug Development
Thermo Fisher Scientific

Metin Nafi Gurcan, PhD
Professor, General Internal Medicine
School of Medicine
Wake Forest University

Arash Hatefi, PharmD, PhD
Professor, Pharmaceutics
Ernest Mario School of Pharmacy
Rutgers, the State University of New Jersey

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

Jeffrey Hughes, PhD
Head of Discovery Formulations
Invaio

Margaret "Meg" Landis, PhD
Director
Molecular Pharmaceutics
Pfizer

Steven Langston, PhD
Senior Scientist
Takeda

Frank Lee, PhD
Founder
IOPharm Consulting

Rushit N. Lodaya, PhD
Associate Director
RNA Delivery Sciences
GSK

Heidi M. Mansour, PhD, RPh, FAIMBE, SMNAI
Professor & Program Leader
Center for Translational Science
College of Public Health & Social Work
College of Medicine
College of Engineering
Florida International University

Ajit Narang, PhD
Vice President, Head of CMC
ORIC Pharmaceuticals

Serkan Oray, PhD
Vice President
Head of Devices, Artwork & Packaging
UCB

Greg Sacha, PhD
Global Senior Scientist
Development & Pre-Commercial Services
Simtra Biopharma Solutions

Srini Sridharan, PhD
Head
Drug Delivery & Product Strategy
Bristol Myers Squibb

Changquan "Calvin" Sun, PhD, FRSC
Professor
Dept. of Industrial & Molecular Pharmaceutics
Purdue University

Adam Swick, PhD
Director
INT MFG Strategic Operations
Moderna Therapeutics

Oleh Taratula, PhD
Professor
College of Pharmacy
Oregon State University

Huan Xie, PhD
Professor, Pharmaceutical Sciences
Founding Director,
Institute of Drug Discovery & Development
Program Director,
Graduate Program of Pharmaceutical Sciences
Director,
RCMI Center for Biomedical & Minority Health Research
Texas Southern University

Translational Medicine

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

Danyelle M. Townsend, PhD (Co-Chair)
Professor
Drug Discovery & Biomedical Sciences
College of Pharmacy
Medical University of South Carolina

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

Charmaine Demanuele, PhD
Vice President, R&D Data Science
& Digital Health – Neuroscience
Johnson & Johnson

Peter L. Elkin, MD, MACP, FACMI, FNYAM, FAMIA, FIAHSI
Distinguished Professor & Chair
Dept. of Biomedical Informatics
Professor, Internal Medicine
University of Buffalo, SUNY

Vicki L. Ellingrod, PharmD, FCCP, FACNP
Dean & Professor of Pharmacy
College of Pharmacy
Professor, Psychiatry
Adjunct Professor, Psychology
University of Michigan

Raymond J. Hohl, MD, PhD
Professor
Dept. of Molecular & Precision Medicine
Dept. of Medicine
Director, Penn State Cancer Institute
Penn State University

Ritika Jaini, PhD
Formerly Senior Scientist
Immunology
Life Edit Therapeutics

Anastasia Khoury Christianson, PhD
Consultant
Formerly at Pfizer

Donald E. Mager, PharmD, PhD
Professor & Chair
Dept. of Pharmaceutical Sciences
University at Buffalo, SUNY

Emanuela Ricciotti, PharmD, PhD
Research Assistant Professor
Dept. of Systems Pharmacology
& Translational Therapeutics
Perelman School of Medicine
University of Pennsylvania

Adam Snook, PhD
Associate Professor
Dept. of Pharmacology, Physiology & Cancer Biology
Thomas Jefferson University

Jonathan Sockolosky, PhD
Principal
Sockolosky and Associates LLC

Maria Wilson
Executive Director
Project Team Leadership
Genentech

Yong (Tiger) Zhang, PhD
Associate Professor
Dept. of Pharmacology & Pharmaceutical Sciences
Dept. of Chemistry
School of Pharmacy & Pharmaceutical Sciences
University of Southern California

Value Assessment and Health Outcomes Research

Eberechukwu Onukwugha, MS, PhD (Chair)
Professor
Dept. of Practice, Sciences & Health Outcomes Research
Executive Director, Pharmaceutical Research Computing
School of Pharmacy
University of Maryland

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

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

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

Marianne Laouri, PhD
Global Asset Team Leader
Retinal Health
Boehringer Ingelheim Pharmaceuticals, Inc.

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
Retired as Senior Vice President
Value Evidence & Outcomes
Eli Lilly and Company

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

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

FINANCIALS

Statement of Financial Position

As of December 31, 2025

ASSETS		2025
Cash and Cash Equivalents	$	2,405,817
Investments	$	26,348,573
Other Assets	$	46,799
Total Assets	**$**	**28,801,189**

LIABILITIES AND NET ASSETS		
Accounts Payable	$	1,515,326
Net Assets Without Donor Restrictions	$	27,283,863
Total Liabilities and Net Assets	**$**	**28,801,189**

Statement of Activities

For the year ended December 31, 2025

REVENUE AND SUPPORT		2025
Contributions Received	$	2,829,510
Contributed Non-Financial Assets*	$	56,244
Interest and Dividends	$	555,640
Realized and Unrealized Investment Gains	$	2,946,411
Total Revenue and Support	**$**	**6,387,805**

EXPENSES**		
Grants and Awards	$	4,564,221
Program Services	$	14,497
Supporting Services	$	552,658
Total Expenses	**$**	**5,131,376**

**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, 2025.

PhRMA Foundation

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