PhRMA Foundation
Value Assessment Initiative
2018 Challenge Award Winners

Recently, the Pharmaceutical Research and Manufacturers of America (PhRMA) Foundation launched its Value Assessment Initiative, a three-part award program that to date has provided more than $1.5 million in funding aimed at advancing the ongoing shift toward a value-driven health care system through better evidence and value frameworks. The Foundation partnered with the Personalized Medicine Coalition in administering the initiative's Challenge Awards for 2018.

PROGRAM OVERVIEW

Concern over rising health care costs has increased interest in promoting high-quality care, while avoiding low value or inefficient care. As the United States health care system encourages value-driven decision-making, it is important that information on the value of health care interventions be robust, transparent and address the needs of all health care stakeholders, including patients, payers and providers.

A number of initiatives aiming to drive value in health care have emerged in recent years, but few offer transformative solutions that reflect patient preferences and real-world clinical practice. Recognizing these concerns, the PhRMA Foundation aims to encourage the development of innovative, multi-stakeholder-based solutions to current challenges in assessing and measuring value through its new Value Assessment Initiative.

The initiative includes three award programs:

- **Challenge Awards** for papers that provide transformative strategies to measure or evaluate value of health care interventions that could help advance a value-driven health care system in the United States.

- **Research Awards** for proposals to identify and address challenges in approaches to assess the value of medicines and health care services.

- **Center of Excellence Awards** for proposals to establish and sustain new collaborative, multi- disciplinary centers that will undertake activities to build evidence and partnerships that can inform value assessment strategies and value-driven decision-making.

VALUE IN PERSONALIZED MEDICINE

Personalized medicine – in which prevention and treatment strategies are guided by genetic tests, other biomarkers, and patient preference – is taking hold as a significant element of clinical care, particularly in the field of oncology. In 2017, U.S. Food and Drug Administration approved a record number of targeted medicines. The field of precision medicine has given rise to, and been enabled by, increasingly sophisticated electronic health data systems capable of capturing and analyzing large volumes of genetic, clinical, and patient-generated data. Thought leaders are seeking to harness this capacity to rethink health care and drive unprecedented transparency around the value of health care. Yet, conventional methods for value assessment remain rooted in conventional, population-level methods and evidence hierarchies. New, innovative strategies for determining value in personalized medicine are needed.

THE 2018 CHALLENGE AWARDS

The Value Assessment Initiative Challenge Awards are designed to encourage innovative approaches in defining and measuring value in health care. This year's Challenge Award recipients were recognized for submitting research proposals that focus specifically on advancing value in personalized medicine. A total of $85,000 in funding was provided for the researchers, who answered this question:

> *What are potentially transformative strategies and methods to define and measure value at all levels of decision making that are aligned with personalized/precision medicine?*

2018 CHALLENGE AWARD WINNERS

Three diverse research proposals received Challenge Awards in 2018. In addition to receiving funding, recipients of Challenge Awards are given the opportunity to present their winning papers at a public forum and to become part of the Value Assessment Research Network, which has been established to encourage collaboration and dissemination of findings that emerge from various projects the Foundation is supporting through its Value Assessment Initiative.

1ST — University of Washington and Office of Health Economics
$50,000 Award

Research Proposal: A Strategy to Support the Efficient Development and Use of Innovations in Personalized and Precision Medicine.

Award recipients: Louis Garrison, PhD, University of Washington; Adrian Towse, MPhil, MA, Office of Health Economics, London, England.

In their research proposal, Dr. Garrison and Mr. Towse call for a broadening of the concepts of value in personalized/precision medicine, laying out six basic policy principles as pathways to help determine value. These range from the need for flexible, value-based pricing to real-world evidence generation in personalized/precision medicine and the challenging implications for assessing and rewarding value.

2ND — The Hospital for Sick Children
$25,000 Award

Research Proposal: Genome diagnostics: Novel strategies for measuring value.

Award recipients: Robin Z. Hayeems, ScM, PhD, the Hospital for Sick Children, Toronto; Stephanie Luca, the Hospital for Sick Children, Toronto; M. Stephen Meyn, MD, University of Wisconsin; Eleanor Pullenayegum, PhD, the Hospital for Sick Children, Toronto; Wendy J. Ungar, PhD, the Hospital for Sick Children, Toronto.

In their research proposal, Dr. Hayeems and colleagues discuss the substantial medical and economic benefits of genome–wide sequencing (GWS) as a means to enhance personalized medicine across a broad range of therapeutic areas, noting that assessing the full value of these technologies requires a set of metrics that extend beyond laboratory-based performance parameters. The authors summarize their progress in developing a methodology for measuring the clinical and personal value of genome diagnostics, building on preliminary findings from the Hospital for Sick Children's Genome Clinic, a translational genomics research platform that routinely generates sequence data on children with a range of clinical phenotypes.

3RD — Western University of Health Sciences
$10,000 Award

Research Proposal: Discrete-Event Simulation – An Alternative Patient-Level Modeling Approach for Value Assessment: A Cost-Effectiveness Study of Current Treatment Guidelines for Women with Postmenopausal Osteoporosis.

Award recipient: Quang A. Le, PharmD, PhD, Western University of Health Sciences, College of Pharmacy.

In his research proposal, Dr. Le discussed discrete-event simulation (DES), an event-driven, continuous time, patient-level modeling method for health economic evaluations that addresses some limitations of other common modeling techniques. Flexibility, the ability to reflect patient heterogeneity, increased precision, and better characterization of modeling uncertainty are advantages in the DES model. Dr. Le's proposal aims to describe and demonstrate an application of the DES model to evaluate the cost-effectiveness of the current treatment guidelines for women with postmenopausal osteoporosis.

THE PhRMA FOUNDATION

The mission of the PhRMA Foundation is to support young scientists in disciplines important to the pharmaceutical industry by awarding them competitive research fellowships and grants at a critical decision point at the outset of their careers. For more than 50 years, the Foundation has been helping to build a larger pool of highly-trained, top-quality scientists to help meet the growing needs of scientific and academic institutions, government, and the research-intensive pharmaceutical industry. It has distributed more than $84 million to support scientific research. To learn more, please visit www.phrmafoundation.org.

THE PERSONALIZED MEDICINE COALITION

The Personalized Medicine Coalition (PMC), representing innovators, scientists, patients, providers and payers, is an education and advocacy organization that promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system. PMC has defined personalized medicine as an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient. As such, personalized medicine presents unique opportunities to improve the value of health care. To learn more, please visit www.personalizedmedicinecoalition.org.

For more information on our Value Assessment programs, please visit *http://www.phrmafoundation.org/2018-awards/value-assessment-initiative/*