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PhRMA Foundation and Personalized Medicine Coalition Announce

2018 Value Assessment Challenge Award Recipients

\$85,000 in funding supports research that helps define and measure value in personalized medicine

(October 10, 2018) The PhRMA Foundation and the Personalized Medicine Coalition (PMC) have announced the recipients of the 2018 Value Assessment Challenge Awards – designed to encourage innovative approaches in defining and measuring value in health care. This year's award recipients were recognized for submitting research proposals that focus specifically on novel methods for assessing the value of personalized medicine.

A total of \$85,000 in funding was provided for the researchers, who answered the 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 Award recipients include:

First place: \$50,000

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.

Second Place: \$25,000

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 genomic data on children with a range of clinical phenotypes.

Third Place: \$10,000

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 Challenge Awards, first offered in 2017, are one component of the PhRMA Foundation's multi-faceted Value Assessment Initiative, which supports a variety of research and innovation projects to help the United States make a successful transition to a value-based health care system.

Concern over rising U.S. health care costs in recent years has increased interest in promoting high-quality care while avoiding low-value or inefficient care. In response, various efforts aimed at driving value in health care have emerged, but few have offered transformative solutions that reflect patient preferences and real-world clinical practice.

PMC, a 501(c)3 education and advocacy organization, 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 patient outcomes and make the health system more efficient by targeting treatments to only those patients who will benefit from them.

The Foundation's Value Assessment Initiative is aimed at encouraging more evidence-based research on how the true value of medical treatments can be accurately defined and quantified. To date, the Foundation has awarded more than \$1.4 million to support a variety of research projects on that topic.

Recipients of Challenge Awards will be acknowledged during PMC's 14th Annual Personalized Medicine Conference at Harvard Medical School on Thursday, November 15th. They will be asked to present their winning papers at a public forum, to be announced later, where they will be honored for their work. Award recipients will also 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.

"Working with PMC, the PhRMA Foundation is delighted to recognize and reward the innovative approaches to value assessment demonstrated in these outstanding research proposals," said PhRMA Foundation President Eileen Cannon. "The work of these outstanding researchers will make a significant contribution to the effort to create truly value-based health care in the United States."

To learn more about the 2018 Challenge Awards, please visit www.phrmafoundation.org or call 202-572-7756.

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About 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. Since its founding, it has distributed more than \$90 million to support scientific research. To learn more, please visit www.phrmafoundation.org.

About the Personalized Medicine Coalition

The Personalized Medicine Coalition (PMC), representing innovators, scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system. To learn more, please visit www.personalizedmedicinecoalition.org.