Proposals from the 2018 PhRMA Challenge Award Program

Eileen Cannon, and Daryl Pritchard, PhD

The use of value assessment frameworks and the application of personalized medicine are 2 emerging managed care strategies that attempt to consistently identify which treatments provide the most benefits for patients and the health system. Through the 2018 PhRMA Foundation Challenge Award Program, the PhRMA Foundation and the Personalized Medicine Coalition partnered to facilitate research that aligns these 2 driving issues.

Personalized medicine—in which prevention and treatment strategies are guided by diagnostic tests that identify specific biological markers, along with the consideration of patient circumstances and preferences—is taking hold as a significant element of clinical care. At its center is the individual patient and the realization that optimizing treatment at the patient level can translate to system-level value.

The PhRMA Foundation's emphasis on evidence-based research that determines the true value of medicines is supported by its ongoing Value Assessment Initiative, which is dedicated to promoting innovative and groundbreaking research to better the lives of patients. Recognizing that traditional value measurement methods are limited, our objective is to drive research that develops and assesses value frameworks through an open and transparent process. That process includes meaningful input from patients and providers, consideration of patient differences and preferences, and the delivery of reliable and relevant information to inform health care decision making.

Our request for proposals posed this challenging question: *What are potentially transformative strategies and methods to define and to measure value at all levels of decision making that are aligned with personalized/precision medicine?* We were very pleased to recognize and award researchers whose work provides different perspectives and exemplifies new approaches to value assessment.

In the following pages of this issue of the *Journal of Managed Care & Specialty Pharmacy* (JMCP), three 2018 award-winning proposals are summarized. These proposals address the question with different objectives and strategies, but they are complementary and consistent in recognizing the critical need for decision makers to take into account individual patient heterogeneity. In “Genome Diagnostics: Novel Strategies for Measuring Value,” Hayeems et al. focus on the development of a tool to

measure the “informational value” of new genomic diagnostics to patients. They aim to develop, validate, apply, and disseminate a novel, stakeholder-informed measure of utility. In “Patient-Level Modeling Approach Using Discrete-Event Simulation: A Cost-Effectiveness Study of Current Treatment Guidelines for Women with Postmenopausal Osteoporosis,” Le illustrates the value of using discrete-event simulation modeling to incorporate individual characteristics and clinical profiles into value assessment at the patient level. Le evaluates the cost-effectiveness of the current treatment guidelines for women with postmenopausal osteoporosis. Garrison and Towse approached the question at a health system level in their proposal “A Strategy to Support Efficient Development and Use of Innovations in Personalized Medicine and Precision Medicine,” by suggesting policies that promote dynamic efficiency in research and development to support personalized/precision medicine globally. They lay out 6 basic cross-cutting policy principles that include the need for flexible value-based pricing, the need for real-world evidence generation, and the complementary nature of the inputs in personalized/precision medicine.

The PhRMA Foundation and the Personalized Medicine Coalition are committed to driving real change in health care delivery and recognize the benefit of partnerships that encourage scientific progress that directly affects patient care.

We thank JMCP for highlighting the exemplary work produced by these researchers through the 2018 Challenge Award Program.

Eileen Cannon

President, PhRMA Foundation
ecannon@phrma.org

Daryl Pritchard, PhD

Senior Vice President, Science Policy
Personalized Medicine Coalition
dpritchard@personalizedmedicinecoalition.org

DISCLOSURES

This introduction was written under the sponsorship of the PhRMA Foundation Award Program. The authors have nothing to disclose.