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GUEST COMMENTARY

CAPACITY FOR PATIENT ENGAGEMENT

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Patient engagement in drug and device development and approval has become a cornerstone in broader efforts to reshape how innovation comes to market. However, in order to fulfill the vision for patient engagement, we will need to expand the capacity for the patient community to be ready and able to partner with regulators, industry and researchers.

For industry, patient engagement is at the core of strategies to align and lower the uncertainty that the value of products in the pipeline will come to fruition in the market.

For the patient advocacy community, what began as a relatively innocuous provision in PDUFA V has become a wedge issue for those who are challenging FDA to rethink how to standardize its benefit-risk framework to reflect the trade-offs acceptable to patients. Finally, whether symbolic or intentional, the 21st Century Cures draft legislation issued by the House Energy and Commerce Committee on Jan. 27 began with a provision entitled "Putting Patients First by Incorporating Their Perspectives Into the Regulatory Process and Addressing Unmet Needs."

Type I diabetes advocacy group JDRF provides a pioneering example. Today, private insurers broadly cover continuous glucose monitoring (CGM) for patients with Type I diabetes, due in large part to the efforts of JDRF.

CGM provides people living with diabetes and their caregivers access to real-time data to effectively manage blood sugar and avoid extreme highs and lows that pose serious risks to patients. To support patient access to CGM, JDRF designed and funded a clinical trial to demonstrate the technology's benefits and then worked with payers to translate the research findings into coverage policies.

What makes JDRF a pioneering model is, since its founding 45 years ago, JDRF has placed patients and their families at the center of its long- and short-term priority setting, funding decisions and evaluation of the research portfolio. In addition, the organization directly funds over $568 million in research each year and employs over 26 Ph.D. scientists.

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The group has established a robust and state-of-the-art infrastructure to infuse board and research council decisions with "the broadest spectrum of views, concerns, and perspectives" of Type I patients and their families. In the new age of patient engagement in drug discovery and healthcare, JDRF's investment and infrastructure is exceptional and will be closely watched as a model for how to integrate patients into R&D and patient access.

DEEPENING THE BENCH

Investment is necessary to deepen the bench within patient advocacy and research organizations with clinicians, scientists and researchers to build a strong bridge to regulatory agencies, industry and the research community.

According to a survey conducted by the European Patients' Academy on Therapeutic Innovation (EUPATI), patient experts, patient advocates and the public at large have low levels of participation in research, and low understanding of

drug development, the regulatory process or market access. EUPATI is funded by the Innovative Medicines Initiative (IMI).

In the U.S., the Patient-Centered Outcomes Research Institute (PCORI) has established five research priorities, including to accelerate patient-centered outcomes research and methodological research. PCORI plans to allocate $2.6 billion to research projects out of $3.5 billion it expects to receive over 2010-19. Although engagement permeates PCORI's research funding, in 2015 only $15.5 million is dedicated to improve the capacity of patients and other stakeholders to participate in the research process.

This is a drop in the bucket of the type of capacity needed to support robust patient engagement in product R&D, regulatory approval and market access.

Absent patient advocacy organizations creating this capacity and the federal government sponsoring such work, the life sciences industry is left to support engagement as a part of its investment in R&D of new products. The potential concentration of funding for these efforts by industry is likely to raise questions of objectivity and stunt the credibility of these efforts for regulators, payers and others. What is needed is the creation of a diverse funding base for these efforts, as well as well recognized best practices for patient engagement and robust methodological approaches.

A public-private partnership is a potentially logical approach to supply the infrastructure, support and systemic focus on patient engagement in research and drug development, and this policy option has been proposed and supported by BIO, the National Health Council (NHC), the Critical Path Institute (C-Path), IMI and other stakeholders. According to these organizations, such a partnership could enhance collaboration with FDA to further patient-focused drug development efforts, serve as a resource to industry and researchers seeking to identify and engage patients, refine methods for obtaining genuine and high-quality engagement, create a support network for patients and advocates involved in R&D, and create an opportunity for centralized/sustained funding for patient engagement.

C-Path provides a model. As a non-profit, public-private partnership with FDA, C-Path was created under the auspices of FDA's Critical Path program to bring patients, industry, clinicians and FDA together on key hurdles to innovation. Once supported by philanthropic and FDA seed funding, C-Path has implemented a diversified funding model that will ensure its longevity and ability to scale to meet the evolving needs of its mission.

The Drug Information Association's Patient Advocate Fellowship Program provides another possible approach to deepening capacity. This program aims to develop and reinforce the collaboration of patients and patient advocates with stakeholders involved in policy development, research and the delivery of healthcare.

The draft 21st Century Cures legislation and PDUFA VI on the horizon opens a window of opportunity to build a sustained capacity for patient engagement. Now it's up to policy-makers to prioritize human capital investments along with more traditional proposals that will lead to a more patient-centric innovation environment. bc

COMPANIES AND INSTITUTIONS MENTIONED

Biotechnology Industry Organization (BIO), Washington, D.C.
Critical Path Institute (C-Path), Tucson, Ariz.
Drug Information Association (DIA), Washington, D.C.
European Patients' Academy on Therapeutic Innovation (EUPATI), Riemerling, Germany
JDRF, New York, N.Y.
Innovative Medicines Initiative (IMI), Brussels, Belgium
National Health Council, Washington, D.C.
Patient-Centered Outcomes Research Institute (PCORI), Washington, D.C.
U.S. Food and Drug Administration (FDA), Silver Spring, Md.

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