Value & Access

Value of Medicines and Affordable Access: Global Dilemma, Local Debates

Sandra Blumenrath
DIA Science Writer

This article was developed with input from and discussion with DIA Senior Scientist Inka Heikkinen.

The question of how to expedite patient access to emerging technologies and reduce costs has been on nearly everyone's radar in 2017, including governments and the private sector.

Value frameworks to assess health technologies, more frequent pricing revisions, and the use of Real World Evidence (RWE) were among the most commonly discussed topics. In the US and Canada, approval and access to generic drugs was also high on the list.

Americas

In 2017, Canada took another step forward in its commitment to bringing down healthcare cost:

- After consultations with various stakeholders, the Canadian government proposed to amend drug pricing regulations that govern the Patented Medicine Prices Review Board (PMPRB), which protects consumers from excessive drug prices. The proposed amendments reduce the regulatory burden for generic drugmakers and equip the PMPRB with new tools to evaluate drug prices, including a much longer list of country comparators.
- The pan-Canadian Pharmaceutical Alliance (pCPA) moved towards establishing a policy framework for biosimilars that would enable more consistent price negotiations.

In the US, stakeholders focused their attention primarily on the implications of PDUFA VI, 21st Century Cures, the "Right to Try" Act, and the US generic drug program for patient access to the medicines they need. Other prominent topics were value frameworks for health technology assessment (HTA), the definition and application of RWE, and outcomes-based drug reimbursement agreements.

Key implications of **PDUFA, 21st Century Cures**, and the **"Right to Try"** Act:

- Both PDUFA VI and the 21st Century Cures Act provide pathways to expedited access to innovative treatments (Patient Access to Therapies and Information clause, Cures Act; Ensuring Sustained Success of Breakthrough Therapy Program clause, PDUFA VI).
- The "Right to Try" gives patients with terminal illnesses the right to access experimental treatments that have demonstrated a level of safety.

Important milestones in the **US generic drug program** include:

- The FDA's Drug Competition Action Plan to increase competition and improve patient access to affordable drugs.
- The reauthorization of the Generic Drug User Fee Amendments (GDUFA), which will provide important funding to the FDA generic drug program, advancing generic drug approvals.

Catering to the need for continued discussions among the different stakeholders, DIA and the Boston Consulting Group (BCG) held several Market Access Roundtables to develop a common set of standards for future market access competencies.

The focus on value frameworks to assess health technologies has led to a surge in **HTA frameworks** by non-governmental actors like ICER (Institute for Clinical and Economic Review), NCCN (National Comprehensive Cancer Network), ASCO (American Society of Clinical Oncology), the Memorial Sloan Kettering Cancer Center, and the American Heart Association. There has also been an increased push for using **RWE** to inform the development and implementation of these frameworks.

Arguably one of the most remarkable **outcomes-based agreements** for treatments with hefty price-tags was the deal struck between Novartis and the Centers for Medicare and Medicaid Services (CMS), offering patients a money-back guarantee if the drug maker's new, personalized cancer treatment is unsuccessful.

Asia

Some of the Asian markets echo challenges similar to those in Europe and in the US:

- Intense debates in Japan centered on HTA assessments and more frequent pricing revisions, all matters still awaiting final resolution at the end of the year. The country also uses a R&D cost-based model in line with WHO's Fair Pricing Initiative that does not use any comparators to determine price.
- In China, the Ministry of Human Resources and Social Security (MoHRSS) has updated the China National Reimbursement Drug List (NRDL) as part of the country's wide-ranging health reform, improving both availability and affordability of needed drugs, including those produced by foreign firms. The NRDL also includes a cost negotiation process for 44 nationally and internationally produced high-priced drugs.

Europe

As healthcare systems are increasingly socio-economically vulnerable across the EU, sustaining patient access to high quality care has become a high priority. To that end

- pharmaceutical companies can use scientific advice from HTA Bodies (HTABs) to generate the evidence needed by payers for decisions on treatment costs and reimbursement;
- the European Medicines Agency (EMA), HTA bodies, and European payer organizations have joined forces to address clinical research deficiencies and streamline pricing and reimbursement negotiations to boost timely and affordable access to new healthcare products; and
- ADAPT-SMART, a project by the Innovative Medicines Initiative (IMI) to provide early access to innovative drugs, continued to look into challenges and solutions to early access, including issues around harnessing RWD in Central and Eastern Europe.

WHAT LIES AHEAD?

"The opportunities for generating evidence to substantiate value and access will be greatly enhanced by the use of digital technologies for patient recruitment and data collection, and by data linkage. The ability to conduct randomized trials enabled by follow-up through linked data or direct-to-patient follow-up is just one example of the rigorous approaches we are likely to see. The best thing about these new approaches is that they will support evaluation of a wider variety of real-world outcome assessments which, if the product really works, will be able to quantify its value for various populations of interest."

Nancy Dreyer, MPH, PhD

Head, Center for Advanced Evidence Generation
Global Chief, Scientific Affairs
QuintilesIMS

"The new WHO director general has made access to quality health care one of WHO's main goals during his tenure. Like education and fire and police protection, access to quality healthcare by all is becoming the global mantra. The old mid-20th century reality of regulatory approval being tantamount to "access" no longer exists. Policy makers, purchasers, and payers all have major decision-making impacts on access. Defining a healthcare products' value proposition to the community is becoming as critical as defining its benefit/risk profile and manufacturing quality standards. "Traditional" regulators are no longer the only "gatekeepers" they once were regarding new healthcare products; they are simply one in a series of keepers of a growing number of gates these new products now must go through before patients have access."

Murray Lumpkin, MD

Deputy Director, Integrated Development (Lead for Global Regulatory Systems Initiatives)
Bill and Melinda Gates Foundation

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