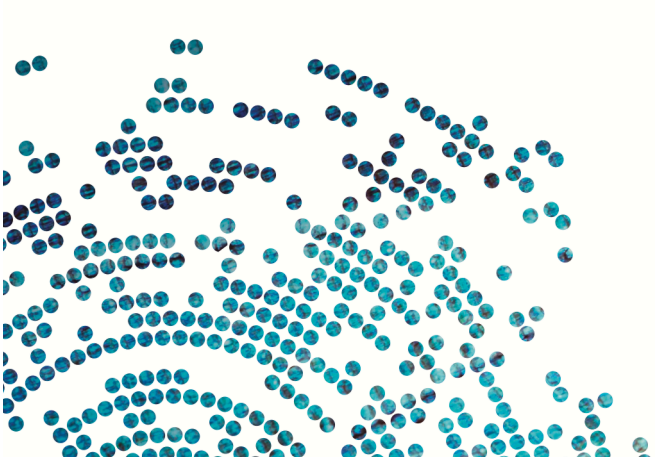
PMC PERSONALIZED MEDICINE COALITION



Personalized Medicine at FDA

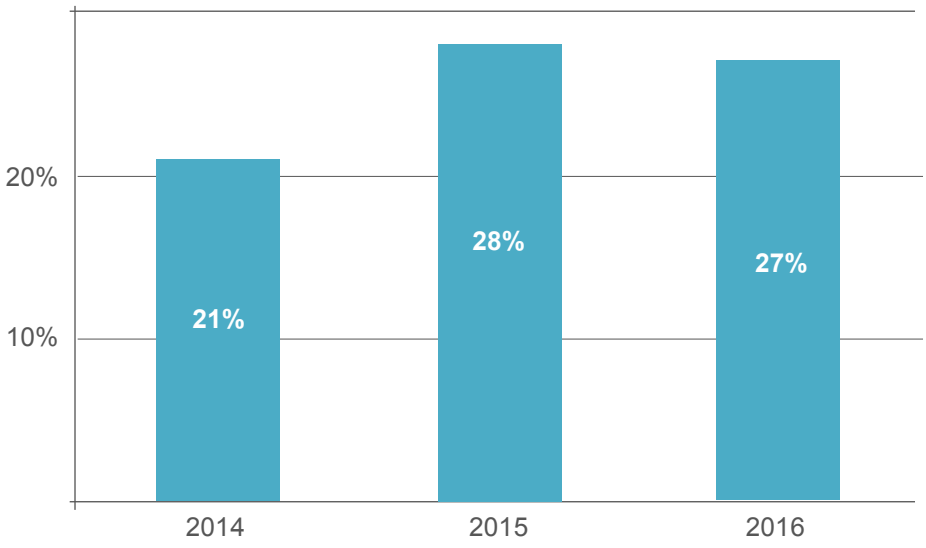
2016 Progress Report

A Three-Year Trend

In 2016, for the third year in a row, personalized medicines accounted for more than 20 percent of all new molecular entities (NMEs) approved by the U.S. Food and Drug Administration (FDA).

FDA's Center for Drug Evaluation and Research (CDER) approved 22 NMEs, new drugs, agents or therapeutic biologics, in 2016. Of the 22, the Personalized Medicine Coalition (PMC) classified six of them — more than 25 percent — as personalized medicines, continuing a trend that PMC first documented in 2014 when it pointed out that nine of 41 NMEs approved that year are personalized medicines.

More Than 20 Percent of New Molecular Entities Approved by FDA in Each of Past Three Years are Personalized Medicines



The analysis underlines that nearly one of every four drugs the agency approved from 2014 to 2016 is a personalized medicine. That ratio is a sharp increase from 2005, when personalized medicines accounted for just 5 percent of NME approvals.

Methodology

PMC defines personalized medicine as an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient. By combining the data from those tests with an individual's medical history, circumstances and values, health care providers can develop targeted treatment and prevention plans.

When evaluating NMEs, PMC categorizes personalized medicines as those therapeutic products for which the label includes reference to specific biological markers, identified by diagnostic tools, that help guide decisions and/or procedures for their use in individual patients.

Newly Approved Medicines

The six personalized medicines approved in 2016 include:

1. Rubraca (rucaparib) for the treatment of advanced ovarian cancer. The decision to use this product is informed by the BRCA1/2 biomarker status in patients.

2. Exondys 51 (eteplirsen) for the treatment of Duchenne muscular dystrophy. The decision to use this product is informed by the DMD mutation biomarker status in patients.

3. Epclusa (sofosbuvir and velpatasvir) for the treatment of chronic hepatitis C infection. The decision to use this product is informed by the HCV genotype status of the viral infection in patients.

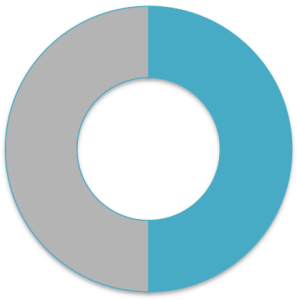
4. Tecentriq (atezolizumab) for the treatment of advanced or metastatic urothelial cancer and metastatic non-small cell lung cancer. The decision to use this product is informed by PD-L1 expression levels in the tumors of patients.

5. Venclexta (venetoclax) for the treatment of chronic lymphocytic leukemia. The decision to use this product is informed by the chromosome 17p deletion biomarker status in patients.

6. Zepatier (elbasvir and grazoprevir) for the treatment of chronic hepatitis C infection. The decision to use this product is informed by the HCV genotype 1 and 4 biomarker status of the viral infection in patients.

Personalized Medicine in Oncology

Nowhere is the transformation of health care toward personalized medicine more clear than in oncology. Of the six personalized NMEs approved in 2016, three are oncology drugs. FDA approved a total of four therapeutic oncology agents in 2016.



50% of the personalized medicines approved by FDA in 2016 are oncology drugs

Newly Approved Indications

FDA also approved several significant new personalized medicine indications for previously approved drugs in 2016. These approvals redefine the drugs' intended populations and provide patients with effective personalized treatment options. The list of new personalized medicines in 2016 should therefore be complemented with reference to newly approved indications for Imbruvica (ibrutinib), Opdivo (nivolumab), Keytruda (pembrolizumab) and Tecentriq (atezolizumab) for new molecularly defined subsets of patients.

Conclusion

FDA's new personalized medicine approvals and expanded indications reflect the extraordinary pace of scientific innovation in the field, which FDA has publicly encouraged.[1] That progress is largely due to an extraordinary commitment to personalized medicine in the pharmaceutical and biotechnology industries.

"We are pleased to see substantial progress and look forward to continuing our efforts to advance biomarkers, which will help bring additional important new therapies to patients in need."

Janet Woodcock, M.D., Director, Center for Drug Evaluation and Research, FDA (March 2015)

Despite ongoing challenges in the areas of scientific discovery, diagnostic regulatory policy, reimbursement and integration of new technologies into clinical practice, that commitment anticipates a shift away from one-size-fits-all, trial-and-error medicine and toward a health care system that utilizes molecular information to improve outcomes and make the health care system more efficient.

[1] Woodcock, J. FDA continues to lead in precision medicine. *FDA Voice* (March 2015). Accessed January 18, 2017 from http://blogs.fda.gov/fdavoice/index.php/2015/03/fda-continues-to-lead-in-precision-medicine/.