More than 20 percent of the Novel New Drugs Approved by FDA's Center for Drug Evaluation and Research in 2014 are Personalized Medicines

The availability of new therapeutic products often means new treatment options for patients, and advances in health care for the American public. The U.S. Food and Drug Administration (FDA)'s Center for Drug Evaluation and Research (CDER) approved 41 novel new drugs (NNDs), either new molecular entities or new therapeutic biologics, in 2014.

Of these 41 NNDs, nine of them --- more than 20 percent --- were personalized medicines as classified by the Personalized Medicine Coalition (PMC).

Personalized medicine is an emerging field that uses diagnostic tools to identify specific biological markers, often genetic, that help determine which medical treatments and/or procedures will be best for each patient. By combining this information with an individual's medical records and circumstances, personalized medicine allows health care professionals and patients to make more informed prevention and treatment plans.

The high proportion of new approvals that are personalized medicines demonstrates the progress researchers have made in advancing the field from an emerging idea a decade ago to an established approach to treating many diseases today. This progress has come as our scientific understanding of the genetic and molecular causes of disease has grown and as researchers have translated this knowledge into new diagnostic and therapeutic products.

Methodology: In its evaluation of the 41 NND approvals, the PMC defined personalized medicines as those therapeutic products[1] for which the label includes reference to specific biological markers, identified by diagnostic tools, that help guide decisions and/or procedures for the product's use in individual patients.

The nine newly approved personalized medicines include:

1. Lynparza (olaparib) for the treatment of advanced ovarian cancer. The decision to treat with this product is affected by the BRCA biomarker status in patients.

2. Vimizim (elosulfase alpha) for the treatment of Mucopolysaccharidosis Type IV (Morquio Syndrome). The decision to treat with this product is affected by the type A or B biomarker status in patients.

3. Cyrazma (ramucirumab) for the treatment of advanced gastric or gastro-esophageal junction adenocarcinoma or non-small cell lung cancer (NSCLC). Treatment procedures are influenced by the EGFR or ALK biomarker status in patients.

4. Zykadia (ceritinib) for the treatment of NSCLC. The decision to treat with this product is affected by the ALK biomarker status in patients.

5. Beleodaq (belinostat) for the treatment of peripheral T-cell lymphoma. Treatment procedures are influenced by the UGT1A1 biomarker status in patients.

6. Cerdelga (eliglustat) for the long-term treatment of Gaucher disease type 1. Treatment procedures are influenced by the CYP2D6 biomarker status in patients.

[1] Of the 41 novel new drug approvals, 39 were for therapeutic products, while two were for diagnostic agents. One of these drugs (Neuraceq - florbetaben F 18 injection) is an adjunct to other diagnostic evaluations to identify a specific biomarker used in evaluating patients with cognitive impairment. Although it is not a therapeutic product, this drug also represents an important tool for personalized medicine.

7. Harvoni (ledipasvir and sofosbuvir) for the treatment of chronic hepatitis C infection. The decision to treat with this product is affected by the genotype 1 biomarker status of the viral infection in patients.

8. Viekira Pak (ombitasvir, paritaprevir, and ritonavir; dasabuvir) for the treatment of chronic hepatitis C infection. The decision to treat with this product is affected by the genotype 1 biomarker status of the viral infection in patients.

9. Blincyto (blinatumomab) for the treatment of B-cell precursor acute lymphoblastic leukemia (ALL). The decision to treat with this product is affected by the Philadelphia chromosome biomarker status in patients.

Despite these remarkable successes, there remain many challenges to advancing personalized medicine, particularly in the areas of scientific discovery, regulatory policy, reimbursement, and integration of new technologies into clinical practice. The biomedical community continues to address these challenges. With an environment that supports progress in personalized medicine, the approvals we have seen in 2014 will be just the beginning of many advances for years to come.