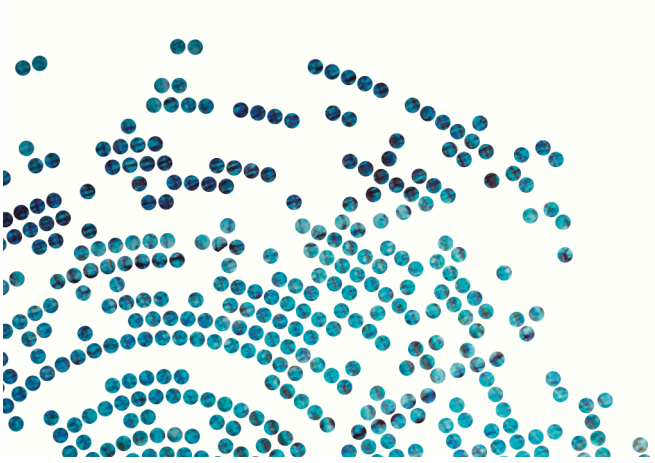
PMC PERSONALIZED MEDICINE COALITION



2015 Progress Report

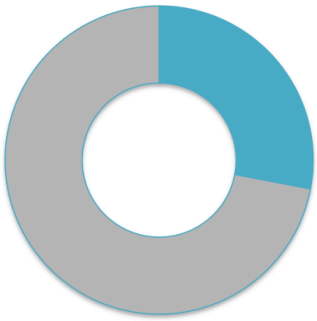
Personalized Medicine at FDA

More Than 25 Percent of the Novel New Drugs Approved by FDA in 2015 are Personalized Medicines

The transformation of health care from one-size-fits-all, trial-and-error medicine to a targeted approach utilizing an individual patient's molecular information continues to accelerate as the U.S. Food and Drug Administration (FDA) more regularly and rapidly approves new personalized medicines.

FDA's Center for Drug Evaluation and Research (CDER) approved 45 novel new drugs (NNDs), either new molecular entities or new therapeutic biologics, in 2015. Of these 45 NNDs, 13 of them — more than 25 percent — were personalized medicines as classified by the Personalized Medicine Coalition (PMC), thus continuing a trend that began last year when nine of 41 NNDs were classified as personalized medicines.

Personalized medicine is 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.



28% of the novel new drugs approved by FDA in 2015 are personalized medicines

Methodology

In its evaluation of the 45 NND approvals, PMC defined 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 13 newly approved personalized medicines include:

1. Alecensa (alectinib) for the treatment of non-small cell lung cancer (NSCLC). The decision to treat with this product is affected by the ALK biomarker status in patients.
2. Tagrisso (osimertinib) for the treatment of NSCLC. The decision to treat with this product is affected by the EGFR biomarker status in patients.
3. Cotellic (cobimetinib) to be used in combination with vemurafenib for the treatment of advanced melanoma. The decision to treat with this product is affected by the BRAF biomarker status in patients.
4. Nucala (mepolizumab) for the maintenance treatment of asthma. The decision to treat with this product is affected by the eosinophil level in patients.
5. Aristada (aripiprazole lauroxil) for the treatment of schizophrenia. Treatment procedures are influenced by the CYP2D6 biomarker status in patients.
6. Lonsurf (trifluridine and tipiracil) for the treatment of advanced colorectal cancer. Treatment procedures are influenced by the VEGF, RAS and EGFR biomarker statuses in patients.
7. Repatha (evolocumab) for the treatment of high cholesterol. The decision to treat with this product is affected by biomarkers that indicate familial hypercholesterolemia.
8. Daklinza (daclatasvir) for the treatment of chronic hepatitis C infection. The decision to treat with this product is affected by the genotype 3 biomarker status of the viral infection in patients.
9. Praluent (alirocumab) for the treatment of high cholesterol. The decision to treat with this product is affected by biomarkers that indicate familial hypercholesterolemia.
10. Rexulti (brexpiprazole) for the treatment of schizophrenia and major depressive disorder. Treatment procedures are influenced by the CYP2D6 biomarker status in patients.
11. Orkambi (lumacaftor and ivacaftor) for the treatment of cystic fibrosis. The decision to treat with this product is affected by the F508del/CFTR biomarker status in patients.
12. Cholbam (cholic acid) for the treatment of bile acid synthesis disorders. The decision to treat with this product is affected by the various single enzyme defect biomarker statuses in patients.
13. Ibrance (palbociclib) for the treatment of advanced breast cancer. The decision to treat with this product is affected by the ER and HER2 biomarker statuses in patients.

Personalized Medicine in Oncology

Nowhere is the transformation of health care toward personalized medicine more clear than in oncology. Of the 13 personalized NNDs in 2015, five are oncology drugs. These drugs account for 35 percent of the 14 oncology NNDs approved in 2015.

35% of the novel new oncology drugs approved by FDA in 2015 are personalized medicines

Newly Approved Indications

Even the high number of 2015 personalized NND approvals does not adequately capture the whole picture, however, of the growing list of personalized medicines available to doctors and their patients. In addition to the 13 personalized NNDs in 2015, FDA approved a number of significant new personalized medicine indications for previously existing drugs that redefine their intended populations and provide patients with more effective personalized treatment options.

The list of new personalized medicines in 2015 would therefore not be complete without mention of newly approved indications for Iressa (gefitinib), Technivie (ombitasvir, paritaprevir, ritonavir, ribavirin), Opdivo (nivolumab) and Keytruda (pembrolizumab) for new molecularly defined subsets of patients.

Rare Genetic Disorders

FDA also approved three new drugs for the treatment of rare genetic disorders. These drugs include Xuriden (uridine triacetate) for hereditary orotic aciduria, Strensiq (asfotase alfa) for perinatal-, infantile-, and juvenile-onset hypophosphatasia (HPP), and Kanuma (sebelipase alfa) for lysosomal acid lipase deficiency.

An Established Approach

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.

While all of these approvals demonstrate the increasing prominence of personalized medicine in health care, there remain many challenges, particularly in the areas of scientific discovery, diagnostic regulatory policy, reimbursement and integration of new technologies into clinical practice.

Substantial progress has been made, but there is still much work to do.