PERSONALIZED MEDICINE AT FDA

The Scope & Significance of Progress in 2023

PMC

MILESTONES

1. Approval of 20 personalized medicines representing approximately 38 percent of all newly approved therapeutic molecular entities. Personalized medicines have now accounted for at least a quarter of new drug approvals for each of the last nine years.

2. Approval of six new gene or cell-based therapies. Gene and cell-based therapies promise to dramatically improve care for certain patients by genetically re-engineering a patient's own cells to combat disease. The therapies approved in 2023 extend the benefits of these personalized treatment approaches to patients with rare genetic diseases including dystrophic epidermolysis bullosa, Duchenne muscular dystrophy, and severe hemophilia A, as well as those with sickle cell anemia or hematologic cancers with planned cord blood transplantation.

3. Clearance or approval of significant new or expanded indications for 12 diagnostic testing systems that can help target treatments to only those who will benefit, sparing expenses and side effects for those who will not. The newly cleared and approved tests and indications include:

   - The first companion diagnostic to identify patients who may benefit from treatment with a gene therapy; and

   - Five new tests that will expand the frontiers of liquid biopsy testing, which can be an alternative to more invasive tissue biopsies for some cancer patients. The newly approved liquid biopsy tests include four new blood-based biomarker tests and one new test to detect mutations in matched blood or saliva. The tests will help guide personalized oncology treatment decisions.

4. Approval of a pair of new cell-based gene therapies for sickle cell disease, a rare, debilitating, and life-threatening blood disorder with significant unmet need. The approval is for patients 12 years and older who suffer from recurrent blood flow blockages that deprive tissues of oxygen.

   - One of the therapies represents the first FDA-approved treatment to utilize CRISPR/Cas9 genome editing technology.

5. The publication of a proposed rule to amend the regulatory framework with respect to laboratory-developed tests (LDTs). Under the proposed rule, FDA would add language to the definition of "*in vitro* diagnostic products" (IVDs) to assert that IVDs are considered devices under the *Food, Drug and Cosmetic Act*, "including when the manufacturer of these products is a laboratory." Treating LDTs as IVDs subject to regulation as medical devices would have a significant regulatory impact on personalized medicine.

6. The publication of a final guidance document titled *Oncology Drug Products Used with Certain* In Vitro *Diagnostic Tests: Pilot Program*. This new guidance document outlines a voluntary pilot program that is designed to provide greater transparency regarding the performance characteristics of certain oncology biomarker tests used with targeted therapies.

7. An accelerated approval decision for a new personalized therapy for the treatment of Alzheimer's disease that targets the fundamental pathophysiology of the disease instead of only treating the symptoms. The approval comes with a recommendation for apolipoprotein E $_{\varepsilon}$4 testing, which is associated with a higher risk for amyloid-related imaging abnormalities (ARIAs). ARIAs are commonly associated with temporary swelling in the brain with little or no symptoms, although serious and life-threatening events rarely may occur. The approval provides a disease mechanism and pharmacogenetic-based targeted approach for patients with few alternative treatment options.

INTRODUCTION

The transformation of health care from one-size-fits-all, trial-and-error medicine to a more targeted approach utilizing each patient's molecular information continues to accelerate as the U.S. Food and Drug Administration more regularly and rapidly approves new diagnostic tools and treatments that will expand the field with implications for patients with rare genetic diseases, cancers, and some common and infectious diseases.

Personalized medicine, sometimes called individualized or precision medicine, is an evolving field in which physicians use diagnostic tests to determine which medical treatments will work best for each patient or use medical interventions to alter molecular mechanisms that impact health. By combining data from diagnostic tests with an individual's medical history, circumstances, and values, health care providers can develop targeted treatment and prevention plans with their patients.

With the approval of 20 new personalized medicines in 2023, personalized medicines have now accounted for at least a quarter of new drug approvals for each of the last nine years. This figure represents a sharp recent increase. Just over a decade ago, personalized medicines accounted for less than 10 percent of the new therapies approved each year.

In 2023, FDA also expanded the indications for many existing personalized therapies; approved six new gene or cell-based therapies; issued a proposed rule on the regulatory oversight of laboratory-developed tests; and approved many new diagnostic indications that will allow for targeted treatment decisions for various health conditions. The newly approved products will help innovators and physicians provide safer and more efficacious treatments and prevention regimens based on the principles of patient-centered care.

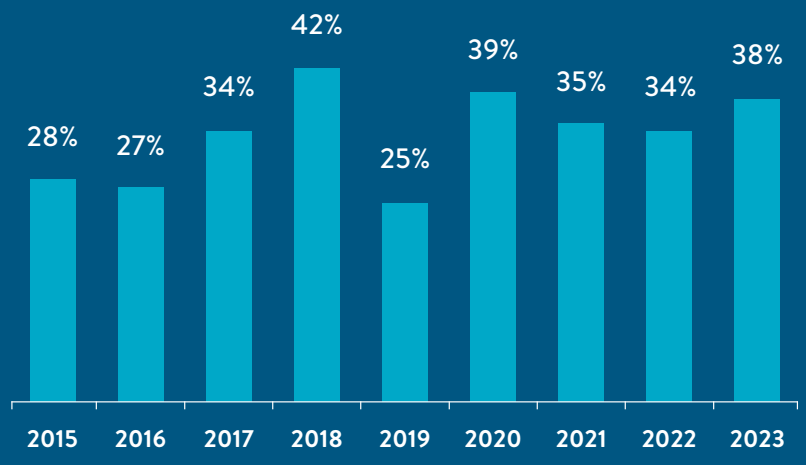
A CONSISTENT TREND

Personalized Medicines Account for More Than a Quarter of All New Therapeutics Approved Since 2015

FDA's Center for Drug Evaluation and Research approved 55 new molecular entities (NMEs) in 2023. All but two of these NMEs are therapeutic products (the others were a diagnostic agent and a hematopoietic stem cell mobilizer). Of the 53 therapeutic NMEs, 20 of them — approximately 38 percent — are personalized medicines as classified by the Personalized Medicine Coalition (PMC). Personalized medicines now account for more than a quarter of the new therapeutics approved since 2015. They have comprised more than a third of new drug approvals for six of the last seven years.

In addition, FDA's Center for Biologics Evaluation and Research approved six new gene or cell-based therapies in 2023. These approvals further underline the potential of this class of personalized treatments, which involve the transplantation of normal genes into a patient's own cells to modify specific cellular functions. FDA has now approved 19 gene or cell-based therapies.

Personalized Medicines Accounted for More Than 25 Percent of FDA Approvals for Each of the Last Nine Years



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

2023 APPROVALS

20 of the 53 new therapeutic molecular entities FDA approved in 2023 — as well as six new gene/cell-based therapies — are personalized medicines.

1. Augtyro (repotrectinib) — for the treatment of metastatic non-small cell lung cancer. The decision to use this product is informed by the status of the ROS1 biomarker in the tumors of patients.
2. Elfabrio (pegunigalsidase alfa-iwx) — for the treatment of Fabry disease. This product provides a recombinant exogenous source of functional alpha-galactosidase A to patients with alpha-galactosidase A deficiency [characterized by accumulated globotriaosylceramide (Gb3) biomarker].
3. Fabhalta (iptacopan) — for the treatment of paroxysmal nocturnal hemoglobinuria. This product selectively targets the Factor B biomarker of the alternative complement pathway and regulates the cleavage of C3 to control both extra- and intra-vascular hemolysis in patients.
4. Filspari (sparsentan) — for the treatment of proteinuria associated with primary immunoglobulin A nephropathy in patients at risk of rapid disease progression. The decision to use this product is informed by the level of the UPCR biomarker in patients.
5. Joenja (leniolisib) — for the treatment of activated phosphoinositide 3-kinase delta (PI3K$_\delta$) syndrome. The decision to use this product is informed by the status of the PI3K$_\delta$ biomarker in patients.
6. Lamzede (velmanase alfa-tycv) — for the treatment of non-central nervous system manifestations of alpha-mannosidosis. This product provides a recombinant exogenous source of functional alpha-mannosidase to patients with alpha-mannosidase deficiency (characterized by certain mannosidase alpha class 2B member 1 biomarkers).

7. Leqembi (lecanemab-irmb) — for the treatment of Alzheimer's disease. The use of this product can be informed by the status of the ApoE $_{\epsilon}$4 biomarker in patients.

8. Loqtorzi (toripalimab-tpzi) — for the treatment of metastatic advanced naso-pharyngeal carcinoma. The use of this product can be informed by the status of the programmed death receptor-1 biomarker in the tumors of patients.

9. Orserdu (elacestrant) — for the treatment of metastatic breast cancer. The decision to use this product is informed by the status of the ESR1, HER2, and ER biomarkers in the tumors of patients.

10. Pombiliti (cipaglucosidase alfa-atga) — for the treatment of Pompe disease [lysosomal acid alpha-glucosidase (GAA) deficiency]. This product provides an exogenous source of functional GAA enzyme to patients with GAA deficiency.

11. Qalsody (tofersen) — for the treatment of amyotrophic lateral sclerosis (ALS). The decision to use this product is informed by the status of the SOD1 biomarker in patients.

12. Rivfloza (nedosiran) — for the treatment of high urinary oxalate levels in patients with primary hyperoxaluria type 1. This product is a small interfering RNA that selectively targets the hepatic lactate dehydrogenase biomarker in patients.

13. Rystiggo (rozanolixizumab-noli) — for the treatment of generalized myasthenia gravis. The decision to use this product is informed by the status of the AChR or MuSK Ab biomarkers in patients.

14. Sohonos (palovarotene) — for the reduction in volume of new heterotopic ossification in patients with fibrodysplasia ossificans progressiva. This product selectively targets the BMP/ALK2 biomarker, which reduces chondrogenesis and osteocyte differentiation resulting in reduced endochondral bone formation in patients.

15. Truqap (capivasertib) — for the treatment, in combination with fulvestrant, of metastatic breast cancer. The decision to use this product is informed by the status of the HR and HER2 biomarkers as well as one or more PIK3CA/AKT1/PTEN alterations in the tumors of patients.

16. Vanflyta (quizartinib) — for the treatment, in combination with cytarabine and anthracycline, of newly diagnosed acute myeloid leukemia following consolidation chemotherapy. The decision to use this product is informed by the FLT3 internal tandem duplication biomarker in the tumors of patients.
17. Veopoz (pozelimab-bbfg) — for the treatment of CHAPLE disease. This product selectively targets the terminal complement protein C5 biomarker that inhibits terminal complement activation in patients.
18. Wainua (eplontersen) — for the treatment of polyneuropathy of hereditary transthyretin-mediated amyloidosis. This product is an antisense oligonucleotide that selectively targets the transthyretin biomarker in patients.
19. Zilbrysq (zilucoplan) — for the treatment of generalized myasthenia gravis. The decision to use this product is informed by the status of the AChR Ab biomarker in patients.
20. Zynyz (retifanlimab-dlwr) — for the treatment of metastatic Merkel cell carcinoma. The use of this product can be informed by the status of the programmed death receptor-1 biomarker in the tumors of patients.

Methodology: When evaluating new molecular entities (NMEs), PMC defined personalized medicines as those therapeutic products for which the label includes reference to specific biological markers, often identified by diagnostic tools, that help guide decisions and/or procedures for their use in individual patients.

Six Newly Approved Gene or Cell-Based Therapies

1. Omisirge (omidubicel) — for use in patients with hematologic malignancies who are planned for umbilical cord blood transplantation following myeloablative conditioning to reduce the time to neutrophil recovery and the incidence of infection. The treatment is a nicotinamide modified haematopoietic progenitor cell-based therapy derived from cord blood used as an allogeneic stem cell donor source.
2. Vyjuvek (beremagene geperpavec-svdt) — for the treatment of wounds in patients with dystrophic epidermolysis bullosa with mutation(s) in the collagen type VII alpha 1 chain gene. The treatment is a live, replication defective HSV-1 vector-based gene therapy containing a transgene encoding the human type VII collagen protein.
3. Elevidys (delandistrogene moxeparvovec-rokl) — for the treatment of ambulatory pediatric patients with Duchenne muscular dystrophy (DMD) with a confirmed mutation in the DMD gene. The treatment is an adeno-associated viral vector-based gene therapy containing a transgene encoding the engineered Elevidys micro-dystrophin protein under the control of the MHCK7 promoter.
4. Roctavian (valoctocogene roxaparvovec-rvox) — for the treatment of adults with severe hemophilia A (congenital factor VIII deficiency with factor VIII activity <1 IU/dL) without pre-existing antibodies to adeno-associated virus serotype 5. The treatment is an adeno-associated viral vector-based gene therapy containing a transgene encoding the B-domain deleted SQ form of human coagulation factor VIII.

5. Casgevy (exgamglogene autotemcel) — for the treatment of sickle cell disease in patients 12 years and older with recurrent vaso occlusive crises. The treatment is a cell-based gene therapy created using the patient's own CD34+ hematopoietic stem cells edited by CRISPR/Cas9 technology within the BCL11A gene to reduce BCL11A expression, leading to increased fetal hemoglobin protein production.
6. Lyfgenia (lovotibeglogene autotemcel) — for the treatment of patients 12 years of age or older with sickle cell disease and a history of vaso occlusive events. The treatment is a β globin-based gene therapy consisting of autologous CD34+ cells from patients with sickle cell disease containing hematopoietic stem cells transduced with BB305 LVV encoding β globin, providing a one-time administration to add functional copies of a modified form of the β-globin gene (βA-T87Q-globin gene) into the patient's own hematopoietic stem cells.

Other Important Trends in Drug Approvals

Expanding Indications

Even the large number of new therapies classified as personalized medicines in 2023 does not provide the whole picture of the growing list of personalized medicines available to doctors and their patients. In addition to the 20 newly approved personalized NMEs and the six newly approved gene or cell-based therapies, FDA approved many significant new personalized medicine indications for existing drugs and combinations of drugs in 2023. These approvals redefine the drugs' intended populations and often provide patients with more effective personalized treatment options.

The list of new personalized medicines in 2023 should therefore be complemented with reference to newly approved indications and combinations for Brukinsa (zanubrutinib), Tukysa (tucatinib) in combination with Herceptin (trastuzumab), Trodelvy (sacituzumab govitecan-hziy), Verzenio (abemaciclib), Taflinar (dabrafenib) in combination with Mekenist (trametinib), Keytruda (pembrolizumab), Pedcev (enfortumab vedotin-ejfv) in combination with Keytruda, Polivy (polatuzumab vedotin-piiq), Lynparza (olaparib), Talzenna (talazoparib) in combination with Xtandi (enzalutamide), Jemperli (dostarlimab-gxly), Lonsurf (trifluridine/tipiracil), Lonsurf in combination with Avastin (bevacizumab), Talvey (talquetamab), Bosulif (bosutinib), Braftovi (encorafenib) in combination with Mektovi (binimetinib), Rozlytrek (entrectinib), Tibsovo (ivosidenib), and Xtandi (enzalutamide).

Significant among these expanded indications are the expanded labels for Keytruda and the combination of Talzenna and Xtandi. Keytruda, previously approved as an immunotherapy for many types of cancers and recently approved in combination with chemotherapy for neoadjuvant treatment following surgery for patients with high-risk, early-stage triple-negative breast cancer, is now indicated with chemotherapy as a neoadjuvant treatment, and with continuation as a single-agent for post-surgical adjuvant treatment for non-small cell lung cancer (NSCLC). This provides the first non-chemotherapeutic adjuvant treatment for NSCLC patients, providing a more effective treatment to prevent recurrence in patients expressing threshold levels of the programmed cell death ligand 1 biomarker while reducing adverse side effects. Talzenna, previously approved for breast cancer indications, and Xtandi, previously approved for prostate cancer, now have expanded indications when used in combination for the treatment of metastatic prostate cancer patients who no longer respond to standard hormone-blocking treatments and who are homologous recombination repair deficient. The combination therapy regimen provides a far more effective treatment option for eligible patients based on two mechanisms of drug targeting. About a quarter of prostate cancer patients have an alteration in a DNA repair gene.

IMPACT

A Record Number of New Treatments for Rare Genetic Diseases and Several Firsts in Cancer and Alzheimer's Disease

Reversing the Root Causes of Rare Genetic Diseases

Eleven of the 20 new molecular entities (NMEs) approved in 2023 and five of the six newly approved gene or cell-based therapies are designed to reverse the root causes of certain rare genetic diseases. Many patients with alpha-mannosidosis, immunoglobulin A nephropathy, activated phosphoinositide 3-kinase delta syndrome, amyotrophic lateral sclerosis (ALS), Fabry disease, fibrodysplasia ossificans progressiva, CHAPLE disease, Pompe disease, primary hyperoxaluria type 1, paroxysmal nocturnal hemoglobinuria, hereditary transthyretin-mediated amyloidosis, dystrophic epidermolysis bullosa, Duchenne muscular dystrophy, severe hemophilia A, and sickle cell disease now have treatments available that target the underlying molecular mechanisms of their diseases.

Casgevy is one of two newly approved cell-based and gene therapies for the treatment of sickle cell disease (SCD) in patients 12 years and older with recurrent vaso occlusive crises. SCD is a group of inherited blood disorders causing debilitating pain and potentially life-threatening disabilities. The disease affects approximately 100,000 people in the United States. SCD primarily affects African Americans. Very few treatments, none of which address the root causes of the disease, have been historically available for SCD patients. Casgevy is a cell-based gene therapy created using the patient's own CD34+ hematopoietic (blood) stem cells that have been modified to correct the underlying mechanism of disease. Casgevy is the first FDA-approved therapy utilizing the novel genome editing technology known as CRISPR/Cas9 gene editing. The approval paves the way for this advanced technology to be employed across other disease states going forward.

Combatting Cancer

Six of the 20 NMEs and one of the six newly approved gene or cell-based therapies provide new treatment options for cancer patients, including two rare forms of cancer for which few treatment options are available. These treatments can significantly improve the outlook for many patients, reducing disease progression and extending survival.

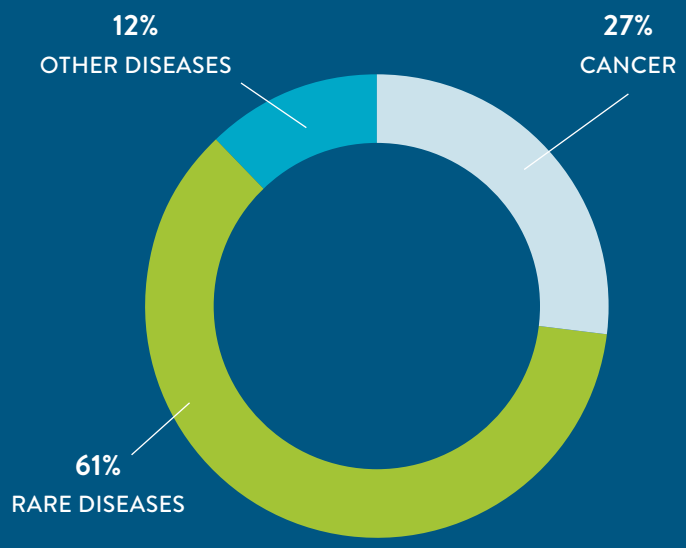
The approval of Truqap in combination with Faslodex for the treatment of metastatic breast cancer marks the first AKT inhibitor to secure approval. It provides a targeted treatment option for patients with tumors with one or more alterations in the PIK3CA, AKT1, or PTEN biomarkers. Up to 50 percent of hormone receptor-positive breast cancer patients may have one or more of these biomarker mutations. The approval thus provides a more effective targeted treatment option for a large population of breast cancer patients who previously may have had to endure multiple rounds of less effective chemotherapy and radiation therapy. As indicated below, FDA also expanded the approved indications for the FoundationOne CDx Assay as a companion diagnostic to select patients with PIK3CA, AKT1, or PTEN alterations that are eligible to receive Truqap.

A New Possibility for Alzheimer's Disease

Leqembi is a newly approved personalized therapy for the treatment of Alzheimer's disease that targets the expected fundamental pathophysiology of the disease instead of only treating the symptoms. Alzheimer's disease is an irreversible, progressive brain disorder affecting more than 6.5 million Americans. The disease slowly destroys memory and thinking skills and, eventually, the ability to carry out simple tasks. Very few effective treatments are available for Alzheimer's patients, highlighting a massive unmet healthcare need. Patients receiving the treatment have significant dose- and time-dependent reduction of amyloid beta plaque, widely believed to affect Alzheimer's disease pathology and progression. The new approval comes with a recommendation for pharmacogenetic testing to prevent rare but potentially serious side effects. The approval provides a disease mechanism and pharmacogenetic-based targeted approach for patients with few other treatment options.

Newly Approved Personalized Medicines Are Indicated for Treatment of Rare Diseases, Cancers, and Common/ Infectious Diseases

Includes 20 New Molecular Entities and Six Newly Approved Gene or Cell-Based Therapies



Cancer

1. Orserdu
2. Zynyz*
3. Vanflyta
4. Loqtorzi*
5. Augtyro
6. Truqap
7. Omisirge

Rare Diseases

1. Lamzede
2. Filspari
3. Joenja
4. Qalsody
5. Elfabrio
6. Sohonos
7. Veopoz
8. Pombiliti
9. Rivfloza
10. Fabhalta
11. Wainua
12. Vyjuvek
13. Elevidys
14. Roctavian
15. Casgevy
16. Lyfgenia

Other Diseases

1. Leqembi
2. Rystiggio
3. Zilbrysq

*indicates rare cancer

NEW DIAGNOSTICS

Newly Approved/Cleared Diagnostic Indications Will Help Target Personalized Medicines to Those Most Likely to Benefit

An important consideration for personalized medicine is the use of diagnostics to discern biomarker statuses to guide drug use. In 2023, FDA's Centers for Devices and Radiological Health and Biologics Evaluation and Research approved or cleared several significant new or expanded indications within 12 *in vitro* diagnostic testing applications that underpin personalized medicine strategies. New approvals and expanded indications associated with the 12 tests listed below will help inform targeted treatment decisions to improve drug safety and efficacy.

Significant New Approvals/Indication Expansions

1. Ventana PD-L1 (SP263) Assay — Approved for the detection of the programmed cell death ligand 1 (PD-L1) protein to guide decisions regarding the use of Libtayo (cemiplimab-rwlc) for the treatment of non-small cell lung cancer.
2. AAV5 Detect CDx — Approved for the detection of antibodies to adeno-associated virus serotype 5. The test can help guide decisions regarding the use of the gene therapy Roctavian for the treatment of severe hemophilia A.
3. CRCdx RAS Mutation Detection Kit — Approved for the detection of certain RAS mutations to help guide decisions regarding the use of Vectibix (panitumumab) for the treatment of colorectal cancer.
4. Abbott RealTime IDH1 — Indications expanded to include detection of certain IDH1 mutations to guide decisions regarding the use of Tibsovo for the treatment of myelodysplastic syndromes.

5. FoundationOne CDx — Four significant indication expansions, as follows:
   - Indications expanded to include detection of BRAF V600 mutations to help guide decisions regarding the use of Braftovi in combination with Mektovi for the treatment of non-small cell lung cancer;
   - Indications expanded to include detection of RET fusions to help guide decisions regarding the use of Retevmo (selpercatinib) for the treatment of solid tumor cancers;
   - Indications expanded to include detection of PIK3CA, AKT1, and PTEN alterations to help guide decisions regarding the use of Truqap in combination with Faslodex for the treatment of breast cancer; and
   - Indications expanded to include detection of BRCA1 and BRCA2 mutations to help guide decisions regarding the use of Akeega (niraparib and abiraterone acetate) for the treatment of prostate cancer.

6. FoundationOne Liquid CDx — Three significant indication expansions, as follows:
   - Indications expanded to include detection of EGFR (HER1) Exon 20 insertion mutations from plasma samples to guide decisions regarding the use of Exkivity (mobocertinib) for the treatment of non-small cell lung cancer;
   - Indications expanded to include detection of BRAF V600E alterations from plasma samples to guide decisions regarding the use of Braftovi in combination with cetuximab for the treatment of metastatic colorectal cancer; and
   - Indications expanded to include detection of BRAF V600E alterations from plasma samples to guide decisions regarding the use of Braftovi in combination with Mektovi for the treatment of non-small cell lung cancer.

7. Guardant360 CDx — Indications expanded to include detection of certain ESR1 missense mutations from plasma samples to guide decisions regarding the use of Orserdu for the treatment of breast cancer.

8. LeukoStrat CDx FLT3 Mutation Assay — Approved for the detection of certain FLT3 IDT and TKD mutations to guide decisions regarding the use of Vanflyta for the treatment of acute myelogenous leukemia.

9. Oncomine Dx Target Test — Indications expanded to include detection of BRAF V600E mutations to guide decisions regarding the use of Tafinlar (dabrafenib) in combination with Mekinist (trametinib) for the treatment of anaplastic thyroid cancer.

10. PD-L1 IHC 22C3 pharmDx — Approved to measure the level of PD-L1 protein expression to guide decisions regarding the use of Keytruda for the treatment of gastric or gastroesophageal junction adenocarcinoma.

11. Therascreen PDGFRA RGQ PCR Kit — Approved for the detection of D842V mutations to guide decisions regarding the use of Ayvakit (avapritinib) for the treatment of gastrointestinal stromal tumors.

12. xT CDx — Approved for the detection of certain RAS mutations from matched plasma or saliva to guide treatment decisions regarding the use of Erbitux (cetuximab) or Vectibix for patients with colorectal cancer.

Direct-to-Consumer Marketing Authorizations

In 2023, FDA's Center for Devices and Radiological Health authorized several significant diagnostic applications that help provide susceptibility and predisposition information directly to consumers, including clearance for marketing of an updated 23andMe Personal Genome Service Genetic Health Risk Report for BRCA1/BRCA2 (selected variants) as a direct-to-consumer test that reports selected BRCA1/BRCA2 genetic variants from human saliva. The test was updated to add 41 BRCA1/BRCA2 variants to the previously authorized test and to include a Predetermined Change Control Plan for adding additional validated BRCA1 and BRCA2 variants and associated cancer risk information without additional premarket review.

FDA also authorized the Invitae Common Hereditary Cancers Panel, the first marketing authorization for a DNA test to assess predisposition for dozens of cancer types. This sequencing-based test can detect hundreds of genetic variants from a blood sample for hereditary cancer predisposition assessment and aid in identifying potentially cancer-associated hereditary genetic variants in individuals who have been diagnosed with cancer. These predisposition tests can provide important personalized medicine information to patients and their families.

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POLICY DEVELOPMENTS

Proposed Rule on Regulation of LDTs

In 2023, FDA issued a proposed rule to amend the regulatory framework with respect to laboratory-developed tests (LDTs). Under the proposed rule, FDA would add language to the definition of "*in vitro* diagnostic products" (IVDs) to assert that IVDs are considered devices under the *Food, Drug and Cosmetic Act*, "including when the manufacturer of these products is a laboratory." Treating LDTs as IVDs subject to regulation as medical devices would be a significant regulatory shift for personalized medicine.

Oncology Drugs Used with Certain *In Vitro* Diagnostic Tests Pilot Program

Policymakers continue to try to provide greater transparency regarding performance characteristics of oncology biomarker tests used with targeted therapies. In 2023, FDA published a final guidance document titled *Oncology Drug Products Used with Certain* In Vitro *Diagnostic Tests: Pilot Program* describing a voluntary program for certain oncology drug products used with corresponding *in vitro* diagnostic tests to help clinicians select appropriate cancer treatments for patients. This voluntary pilot program is designed to provide greater transparency regarding the performance characteristics of certain oncology biomarker tests.

CONCLUSION

Sustaining the Promise of Personalized Medicine

Despite ongoing challenges in the areas of scientific discovery, diagnostic regulatory policy, coverage, reimbursement, and clinical implementation, the personalized medicine developments at FDA in 2023 show that scientific innovation continues to bring the health system away from one-size-fits-all, trial-and-error medicine, toward the utilization of molecular information to improve patient outcomes and make clinical care more efficient. Novel personalized medicine technologies promise to improve outcomes for patients and have a tremendous impact on the efficacy and efficiency of health care delivery.

Continued progress cannot be taken for granted. To ensure that there is sustained progress in the development of groundbreaking personalized medicine tests and treatments for the benefit of patients and health systems, policymakers, as they have in the past, must favor policies that encourage the advancement of the field.

ABOUT US

The Personalized Medicine Coalition (PMC), representing innovators, scientists, patients, providers, and payers, promotes the understanding and adoption of personalized medicine concepts, services, and products to benefit patients and health systems.

PERSONALIZED
MEDICINE COALITION

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