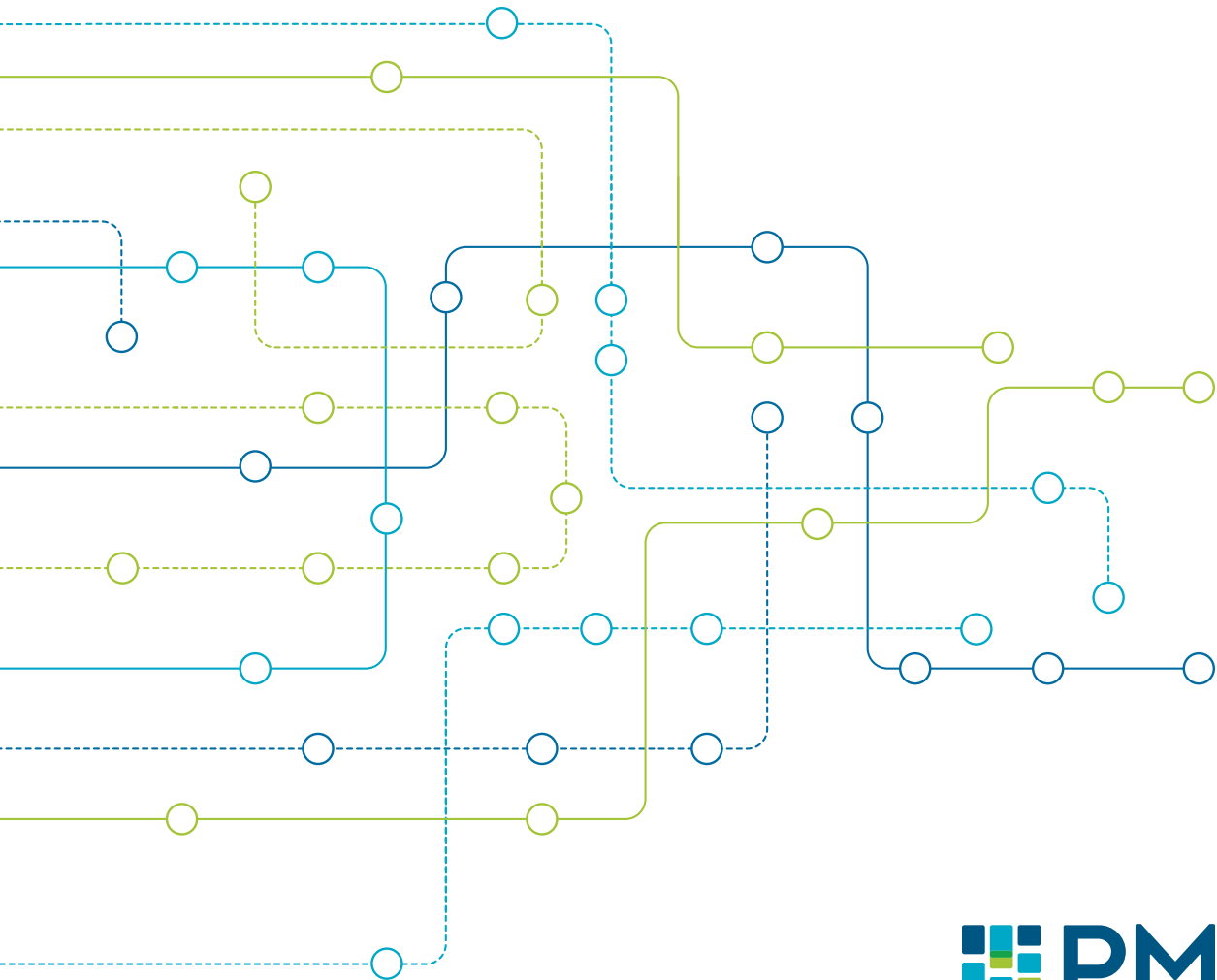


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# PERSONALIZED MEDICINE AT FDA

The Scope & Significance of Progress in 2024



# MILESTONES

1. Approval of 18 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 ten 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 2024 extend the benefits of these personalized treatment approaches to patients with rare genetic diseases including metachromatic leukodystrophy, aromatic L amino acid decarboxylase (AADC) deficiency, and moderate to severe hemophilia B (congenital factor IX deficiency), as well as those with cancers such as metastatic melanoma, metastatic synovial sarcoma, and B-cell precursor acute lymphoblastic leukemia.
3. Clearance or approval of significant new or expanded indications for 11 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:
  - A newly approved tumor-agnostic testing indication, which extends the benefits of personalized medicine to more cancer patients by directing personalized medicines to all patients whose tumors express certain biomarkers, regardless of where in the body those tumors are located.
  - Three newly approved companion diagnostics to identify patients who may benefit from treatment with gene therapies; and
  - Six 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 six new blood-based biomarker tests that will help guide personalized oncology treatment decisions.
4. Approval of safety label changes for fluorouracil injection products based on pharmacogenomic (PGx)-based biomarker testing to determine additional information regarding the risk of serious adverse effects in patients with dihydropyrimidine dehydrogenase (DPD) deficiency. The updates demonstrate the potential and value of PGx testing for helping inform safe and effective treatment decision-making.
5. Began a process for the reclassification of many in vitro diagnostics (IVDs) that are Class III (high-risk devices) into Class II (moderate-risk devices) in advance of enforcement of the FDA final rule to end enforcement discretion of laboratory developed tests (LDTs). Most of these Class III diagnostics are infectious disease and companion diagnostic IVDs, and their reclassification would enable manufacturers of these tests to seek marketing authorization through the 510(k) clearance pathway rather than the more rigorous pre-market approval (PMA) pathway.
6. Released a draft guidance document, titled Diversity Action Plans to Improve Enrollment of Participants from Underrepresented Populations in Clinical Studies, outlining FDA's expectations for Diversity Action Plans (DAPs), as mandated by the Food and Drug Omnibus Reform Act of 2022, to improve the participation of historically underrepresented populations in clinical trials and to improve the strength of evidence on the use of drugs and medical devices approved by the FDA for their intended use in these populations.
7. Established the Center for Drug Evaluation and Research (CDER) AI Council to provide oversight, coordination, and consolidation of CDER activities around AI use. The CDER AI Council consolidates and continues AI efforts started by the CDER AI Steering Committee, AI Policy Working Group, and CDER AI Community of Practice to address the advent of novel AI applications, such as generative AI and large language models in personalized medicine.
8. Established the new [CDER Center for Real-World Evidence Innovation \(CCRI\)](#) which aims to coordinate, advance, and promote the use of real-world evidence (RWE) in regulatory decision-making across CDER. CCRI is meant to create a focal point within FDA to identify ways to utilize RWE to streamline the development of effective and safe medicines that address the needs of individual patients.

# INTRODUCTION

The transformation of health care from one-size-fits-all, trial-and-error medicine to a personalized medicine approach utilizing each patient's molecular information continues to accelerate. 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 18 new personalized medicines in 2024, personalized medicines have now accounted for at least a quarter of new drug approvals for each of the last ten 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 2024, FDA also expanded the indications for many existing personalized therapies; approved six new gene and cell-based therapies; began reclassifying many class III (high-risk devices) into Class II (moderate-risk devices) in advance of pending regulatory reform of laboratory developed tests (LDTs); 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 clinicians provide safer and more efficacious treatments and prevention regimens based on the principles of patient-centered care.

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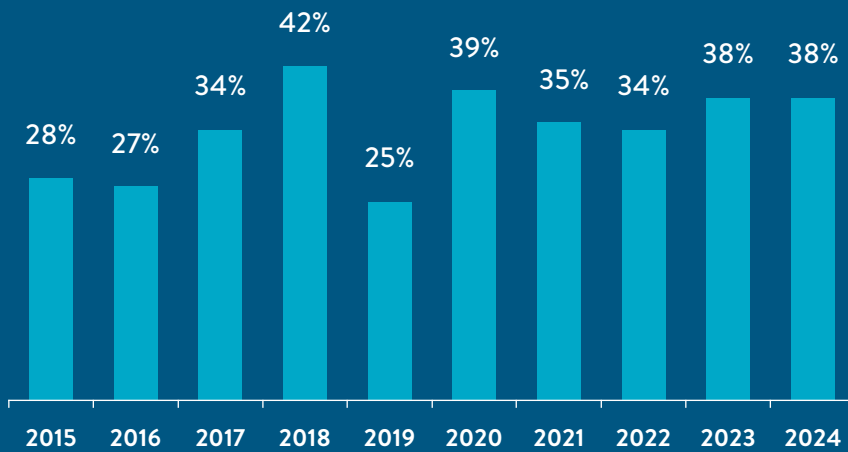
# A DECADE OF CONSISTENT PERSONALIZED MEDICINE DEVELOPMENT

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

FDA's Center for Drug Evaluation and Research (CDER) approved 50 new molecular entities (NMEs) in 2024. All but three of these NMEs are therapeutic products (non-therapeutic NMEs included radio graphic contrast or optical imaging agents). Of the 47 therapeutic NMEs, 18 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 by the FDA since 2015. They have comprised more than a third of new drug approvals for seven of the last eight years.

In addition, FDA's Center for Biologics Evaluation and Research (CBER) approved six new gene or cell-based therapies in 2024. 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 25 gene or cell-based therapies.

## Newly Approved Personalized Medicines Over the Last Ten Years (% of All Newly Approved Drugs)



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.

# 2024 APPROVALS

**18 of the 47 new therapeutic molecular entities FDA approved in 2024 – as well as six new gene/cell-based therapies – are personalized medicines.**

1. **Tevimbra (tislezumab-jsgf)** – for the treatment of metastatic esophageal squamous cell carcinoma. The use of this product can be informed by the status of the programmed death ligand-1 (PD-L1) biomarker in the tumors of patients.
2. **Ojemda (tovorafenib)** – for the treatment of pediatric low-grade glioma. The decision to use this product is informed by the status of a BRAF fusion or rearrangement, or a BRAF V600 mutation, in the tumors of patients.
3. **Kisunla (donanemab-azbt)** – for the treatment of Alzheimer’s disease. The use of this product can be informed by the ApoE ε4 biomarker status in patients.
4. **Leqselvi (deuruxolitinib)** – for the treatment of alopecia areata. The decision to use this product is informed by the CYP2C9 biomarker status in patients.
5. **Voranigo (vorasidenib)** – for the treatment of Grade 2 astrocytoma or oligodendroglioma. The decision to use this product is informed by the status of the IDH1 or IDH2 biomarkers in the tumors of patients.
6. **Livdelzi (seladelpar)** – for the treatment of primary biliary cholangitis. The use of this product can be informed by the CYP2C9 biomarker status in patients.
7. **Lazcluze (Lazertinib)** – for the treatment of non-small cell lung cancer. The decision to use this product is informed by the status of the EGFR exon19 or exon21 L858R biomarkers in the tumors of patients.
8. **Miplyffa (arimocomol)** – for the treatment of Niemann-Pick disease type C. The decision to use this product is informed by the status of the NPC1 and NPC2 biomarkers in patients.
9. **Aqneursa (levacetylleucine)** – for the treatment of Niemann-Pick disease type C. The decision to use this product is informed by the status of the NPC1 and NPC2 biomarkers in patients.

10. [Itovebi \(inavolisib\)](#) – for the treatment of metastatic breast cancer. The decision to use this product is informed by the status of the PIK3CA, HR, and HER2 biomarkers in the tumors of patients.
11. [Vyloy \(zolbetuximab-clzb\)](#) – for the treatment of gastric or gastroesophageal junction adenocarcinoma. The decision to use this product is determined by the status of the HER2 and claudin (CLDN) 18.2 biomarkers in the tumors of patients.
12. [Revufori \(revumenib\)](#) – for the treatment of acute leukemia. The decision to use this product is informed by the status of the lysine methyltransferase 2A (KMT2A) biomarker in the tumors of patients.
13. [Zilhera \(zanidatamab-hrii\)](#) – for the treatment of metastatic biliary tract cancer. The decision to use this product is informed by the status of the HER2 biomarker in the tumors of patients.
14. [Bizengri \(zenocutuzumab-zbco\)](#) – for the treatment of non-small cell lung cancer and pancreatic adenocarcinoma. The decision to use this product is informed by the status of the NRG1 gene fusion biomarker in the tumors of patients.
15. [Unloxcyt \(cosibelimab-ipdl\)](#) – for the treatment of cutaneous squamous cell carcinoma. The use of this product can be informed by the status of the programmed death ligand-1 (PD-L1) biomarker in the tumors of patients.
16. [Ensacove \(ensartinib\)](#) – for the treatment of non-small cell lung cancer. The decision to use this product is informed by the status of the ALK biomarker in the tumors of patients.
17. [Tryngolza \(olezarsen\)](#) – for the adjunct treatment of familial chylomicronemia syndrome. The decision to use this product can be informed by the status of the LPL gene in patients. This product is an antisense oligonucleotide that selectively targets apoC-III mRNA in patients.
18. [Alyftrek \(vanzacaftor, tezacaftor, and deutivacaftor\)](#) – for the treatment of cystic fibrosis. The decision to use this product is informed by the status of the F508 biomarker or other responsive mutational biomarkers in patients.

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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. **Amtagvi (lifileucel)** – For the treatment of adult patients with unresectable or metastatic melanoma previously treated with a PD-1 blocking antibody, or a BRAF inhibitor with or without a MEK inhibitor. The treatment is a tumor-derived autologous T-cell immunotherapy derived from a patient’s tumors, expanded, and reintroduced by intravenous infusion.
2. **Lenmeldy (atidarsagene autotemcel)** – for the treatment of children with pre-symptomatic late infantile (PSLI), pre-symptomatic early juvenile (PSEJ) or early symptomatic early juvenile (ESEJ) metachromatic leukodystrophy (MLD). The treatment is a lentiviral vector (LVV)-based gene therapy consisting of autologous CD34+ cells containing hematopoietic stem cells (HSCs) containing a transgene encoding the human arylsulfatase A (ARSA) gene, which produces functional ARSA enzyme to prevent the harmful accumulation of sulfatides.
3. **Beqvez (Fidanacogene elaparvovec-dzkt)** – for the treatment of adult patients with moderate to severe hemophilia B (congenital factor IX deficiency) who use factor IX prophylaxis therapy, or have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes, and do not have neutralizing antibodies to adeno-associated virus serotype Rh74var (AAVRh74var) capsid. The treatment is an adeno-associated virus (AAV)-based gene therapy containing a transgene encoding a functional copy of the factor IX gene that is able to transduce hepatocytes to increase circulating factor IX activity.
4. **Tecelra (afamitresgene autoleucel)** – for the treatment of adults with metastatic synovial sarcoma who have received prior chemotherapy, are HLA-A\*02:01P, -A\*02:02P, -A\*02:03P, or -A\*02:06P positive and whose tumor expresses the MAGE-A4 antigen. The treatment is a melanoma-associated antigen A4 (MAGE-A4)-directed genetically modified autologous T-cell immunotherapy consisting of CD4 and CD8 positive T 315 cells transduced with a lentiviral vector (LV) expressing an affinity-enhanced T cell 316 receptor (TCR) that can result in T cell 343 proliferation, cytokine secretion, and killing of MAGE-A4/HLA-A\*02 expressing synovial sarcoma 344 cells.

5. [Aucatzyl \(obecabtagene autoleucel\)](#) – for the treatment of adult patients with relapsed or refractory B-cell precursor acute lymphoblastic leukemia (ALL). The treatment is a CD19-directed chimeric antigen receptor genetically modified autologous T-cell (CAR-T) immunotherapy that allows engagement of anti-CD19 CAR-positive T cells with CD19 expressed on target cells in anti-tumor activity.
6. [Kebilidi \(eladocagene exuparvovec-tneq\)](#) – for the treatment of adult and pediatric patients with aromatic L amino acid decarboxylase (AADC) deficiency. The treatment is a recombinant adeno-associated virus serotype 2 (rAAV2)-based gene therapy containing a transgene which encodes a functional AADC enzyme.

## Other Important Trends in Drug Approvals

### Expanding Indications

The new therapies classified as personalized medicines in 2024 do not provide the whole picture of the growing list of personalized medicines available to doctors and their patients. In addition to the 18 newly approved personalized NMEs and the six newly approved gene and cell-based therapies, FDA approved many significant new personalized medicine indications for existing drugs and combinations of drugs in 2024. These approvals redefine the drugs' intended populations and often provide patients with more effective personalized treatment options.

The list of new personalized medicines in 2024 should therefore be complemented with reference to newly approved indications and combinations for Keytruda (pembrolizumab), Casgevy (exagamglogene autotemcel), Tepmetko (tepotinib), Tagrisso (osimertinib), Opdivo (nivolumab), Brukinsa (zanubrutinib), Breyanzi (lisocabtagene maraleucel), Carvykti (ciltacabtagene autoleucel), Elahere (mirvetuximab soravtansine), Enhertu (fam-trastuzumab - deruxtecan-nxki), Abecma (Idcabtagene vicleucel), Alecensa (alectinib), Lutathera (lutetium Lu 177 dotatate), Retevmo (selpercatinib), Augtyro (repotrectinib), Imfinzi (durvalumab), Blincyto (blinatumomab), Krazati (adagrasib), Vyvgart (efgartigimod alfa-fcab), Darzalex Faspro (daratumumab and hyaluronidase-fihj) in combination with bortezomib and dexamethasone, Jemperli (dostarlimab-gxly), Tecentriq Hybreza (atezolizumab and hyaluronidase-tqjs), Kisqali (ribociclib), Rybrevant (amivantamab-vmjw) in combination with carboplatin and pemetrexed, Sarclisa (isatuximab-irfc) in combination with bortezomib, and Itovebi (inavolisib) in combination with palbociclib and fulvestrant.

The expanded indication for Casgevy (exagamglogene autotemcel), previously approved for treatment of sickle cell disease (SCD), represents the first ever expanded indication for an approved gene therapy treatment. Casgevy is now available as a treatment for patients with transfusion-dependent beta thalassemia (TDT) and provides an opportunity to dramatically improve care for patients by genetically re-engineering their own cells to combat disease.

Also significant among these expanded indications are the newly approved uses for Enhertu (fam-trastuzumab - deruxtecan-nxki), Opdivo Qvantig (nivolumab in combination with hyaluronidase-nvhy), and Augtyro (repotrectinib). Enhertu,

previously approved for patients with HER-2 positive breast cancer and expanded to include patients with (HR)-positive, HER2-low (IHC 1+ or IHC 2+/ISH-) breast cancer and patients with HER-2 positive lung cancer, is now indicated for use in patients with all types of unresectable or metastatic HER2-positive (IHC 3+) solid tumors. This provides a targeted high value treatment option for patients with cancer of all different types. Opdivo previously approved as an immunotherapy for many types of cancers, is now indicated for use in cancer patients with all solid tumor types. This provides an immunotherapeutic approach that may be informed by PD-L1 biomarker testing for many cancer patients where this type of treatment was previously unavailable. Similarly, Augtyro, previously approved for patients with locally advanced or metastatic ROS1-positive non-small cell lung cancer (NSCLC), is now also indicated for treatment of patients with all types of solid tumors that have a neurotrophic tyrosine receptor kinase (NTRK) gene fusion and have progressed following first-line treatment. These newly approved indications build upon the expanding tumor agnostic cancer treatment concept, a personalized medicine approach where drugs are selected based on specific genetic or molecular features of the tumor. This extends the benefits of personalized medicine to more cancer patients by directing targeted treatment to all patients whose tumors express certain biomarkers, regardless of tumor location.

The newly approved expanded indication for Tecentriq Hybreza (atezolizumab and hyaluronidase-tqjs) represents the first PD-L1 immunotherapy treatment indication that is administered using subcutaneous injection. Tecentriq was previously approved as an intravenous medicine for patients with non-small cell lung cancer (NSCLC), small cell lung cancer (SCLC), hepatocellular carcinoma (HCC), melanoma, and alveolar soft part sarcoma (ASPS), and its use can be informed by PD-L1 biomarker expression levels in tumors. The expanded indication for subcutaneous administration allows these patients to receive the treatment with reduced administration time, potential home administration, and most importantly, localized administration that can minimize systemic drug exposure in less severe conditions.

These approvals  
redefine the drugs'  
intended populations  
and often provide  
patients with more  
effective personalized  
treatment options.

# IMPACT

## First-Time Treatments for a Rare Genetic Disease and a Boost for Pharmacogenetic Testing to Help Inform Cancer Treatment

### Reversing the Root Causes of Rare Genetic Diseases

Five of the 18 newly approved NMEs and three of the six newly approved gene and cell-based therapies are designed to provide safer and effective treatment options based on the root causes of certain rare genetic diseases. Patients with Niemann-Pick disease type C, familial chylomicronemia syndrome, cystic fibrosis, metachromatic leukodystrophy (MLD), moderate to severe hemophilia B (congenital factor IX deficiency), and aromatic L amino acid decarboxylase (AADC) deficiency now have new treatments available that target the underlying molecular mechanisms of their diseases. Prior to the approval of Miplyffa (arimoclomol) and Aqneursa (levacetylleucine) in 2024, there had not been any approved treatments for patients with Niemann-Pick disease.

Nieman-Pick Disease is a rare, genetic disorder caused by changes in either the NPC1 or NPC2 gene that impairs the body's ability to transport cholesterol and other lipids, leading to their buildup in tissues, particularly the brain. As a result, these cells do not function as they should, ultimately causing organ damage. On average, individuals affected by this devastating disease only live for about 13 years. These new drug approvals provide a first ever personalized treatment to meet the significant needs of patients with Niemann-Pick Disease Type C (NPC).

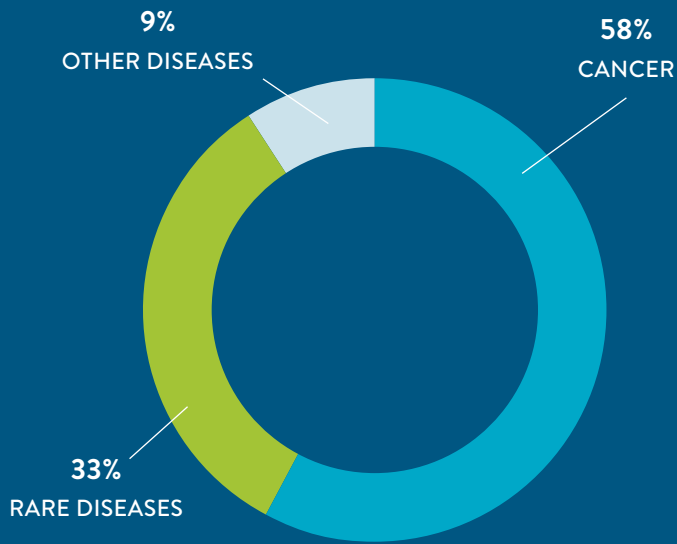
## Drug Labelling Change for an Important Pharmacogenetic Safety Marker

The FDA approved safety label changes for fluorouracil injection products that advise for pharmacogenomic (PGx)-based biomarker testing to determine the risk of serious adverse effects associated with dihydropyrimidine dehydrogenase (DPD) deficiency before administration of the drug. Though the initial approval for fluorouracil injection was granted in 1962 for the treatment of colon and rectum adenocarcinoma, breast adenocarcinoma, gastric adenocarcinoma, and pancreatic adenocarcinoma, the changes to the safety label reflect a growing understanding of the risk of serious adverse events related to fluorouracil use in patients with DPD deficiency and the value of a personalized medicine approach based on PGx testing to inform treatment decisions.

The PGx community had called for broader implementation of DPD/DPYD genetic testing before patients receive fluoropyrimidine chemotherapy such as 5-FU (fluorouracil). This testing helps identify individuals with DPD deficiency who are at higher risk of severe and potentially fatal toxicities. By identifying these individuals, doctors can adjust dosing or avoid the drug class altogether, potentially preventing serious complications.

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

Includes 18 New Molecular Entities and Six Newly Approved Gene or Cell-Based Therapies



### Cancer

1. Tevimbra\*
2. Ojemda
3. Voranigo
4. Lazcluse
5. Itovebi
6. Vyloy
7. Revufori
8. Zilhera\*
9. Bizengri
10. Unloxcyt
11. Ensacove
12. Amtagvi
13. Tecelra\*
14. Aucatzyl

### Rare Diseases

1. Miplyffa
2. Aqneursa
3. Livdelzi
4. Tryngolza
5. Alyftrek
6. Lenmeldy
7. Beqvez
8. Kebilidi

### Other Diseases

1. Kisunla
2. Leqselvi

\*indicates rare cancer

# NEW DIAGNOSTICS

## Newly Approved/Cleared Diagnostic Indications Drive the Delivery of Personalized Medicines

An important consideration for personalized medicine is the use of diagnostics to discern biomarker statuses to guide drug use. In 2024, FDA's Center for Devices and Radiological Health (CDRH) approved or cleared several significant new or expanded indications within 11 in vitro diagnostic testing applications that underpin personalized medicine strategies. New approvals and expanded indications associated with the 11 diagnostic platforms listed below will help inform targeted treatment decisions to improve drug safety and efficacy.

### Significant New Approvals/Indication Expansions

1. **VENTANA CLDN18 (43-14A) RxDx Assay:** Approved for the detection of Claudin 18 (CLDN18) protein expression to guide decisions regarding the use of Vyloy (zolbetuximab) for the treatment of gastroesophageal junction (GEJ) adenocarcinoma.
2. **VENTANA Pathway anti-HER-2/neu (4B5) Rabbit Monoclonal Primary Antibody:** Approved for the detection of HER-2 protein overexpression to guide decisions regarding the use of Ziihera (zanidatamab-hrii) for the treatment of Biliary Tract Cancer (BTC).
3. **SeCore CDx HLA Sequencing System:** Approved for the detection of human leukocyte antigen A-locus (HLA-A) alleles (HLA-A\*02:01, HLA-A\*02:02, HLA-A\*02:03 or HLA-A\*02:06 and their P-group alleles.) from plasma samples to guide decisions regarding the use of Tecelra (afamitresgene autoleucel) for the treatment of Synovial sarcoma.
4. **nAbCyte Anti-AAVRh74var HB-FE Assay:** Approved for the detection of AAVRh74var capsid neutralizing antibodies from plasma samples to guide decisions regarding the use of Beqvez (fidanacogene elaparvovec) for the treatment of moderate to severe Hemophilia B patients.

5. **MAGE-A4 IHC 1F9 pharmDx:** Approved for the detection of Melanoma-associated antigen 4 (MAGE-A4) protein overexpression to guide decisions regarding the use of Tecelra (afamitresgene autoleucel) for the treatment of Synovial sarcoma.
6. **Therascreen KRAS RGQ PCR Kit:** Approved for the detection of KRAS G12C mutations to guide decisions regarding the use of Krazati (adagrasib) in combination with Erbitux (cetuximab) for the treatment of Colorectal Cancer.
7. **MI Cancer Seek (MCS):** Approved the biomarker testing platform as a device for three specific groups of oncology products and six additional indications, as follows:
  - Indications approved for the detection of BRAF V600E mutations to guide decisions regarding the use of a specific group of oncology products including Tafinlar (dabrafenib) and Zelboraf (vemurafenib) for the treatment of Melanoma.
  - Indications approved for the detection of BRAF V600E and V600K to guide decisions regarding the use of a specific group of BRAF/MEK Inhibitor combinations including Cotellic (cobimetinib) in combination with Zelboraf (vemurafenib), Braftovi (encorafenib) in combination with Mektovi (Binimetinib), and Tafinlar (dabrafenib) in combination with Mekinist (trametinib) for the treatment of Melanoma.
  - Indications approved for the detection of EGFR exon 19 deletions or exon 21 (L858R) substitution mutations to guide decisions regarding the use of a specific group of tyrosine kinase inhibitors including Tarceva (erlotinib), Tagrisso (osimertinib), Iressa (gefitinib), Gilotrif (afatinib), Vizimpro (dacomitinib), and Lazcluze (Lazertinib) for the treatment of Non-Small Cell Lung Cancer (NSCLC).
  - Indication approved to include detection of PIK3CA alterations to guide decisions regarding the use of Piqray (alpelisib) for the treatment of Breast Cancer.
  - Indication approved to include the detection of KRAS and NRAS wild-type biomarkers to guide decisions regarding the use of Vectibix (panitumumab) for the treatment of Colorectal Cancer
  - Indication approved to include the detection of BRAF V600E to guide decisions regarding the use of Braftovi (encorafenib) in combination with Erbitux (cetuximab) for the treatment of Colorectal Cancer.

- Indication approved to include the detection of Microsatellite instability – High (MSI-H) to guide decisions regarding the use of Keytruda (pembrolizumab) for the treatment of Solid Tissue tumors.
  - Indication approved to include the detection of Microsatellite instability – High (MSI-H) to guide decisions regarding the use of Jemperli (dostarlimab-gxly) for the treatment of Solid Tissue tumors.
  - Indication approved to include the detection of Not Microsatellite instability – High (Not MSI-H) to guide decisions regarding the use of Keytruda (pembrolizumab) in combination with Lenvima (lenvatinib) for the treatment of Endometrial Carcinoma (EC).
8. **FoundationOne® CDx:** Indications expanded to include detection of BRCA1 and BRCA2 alterations to guide decisions regarding the use of Lynparza (olaparib) in combination with abiraterone for the treatment of Metastatic Castrate Resistant Prostate Cancer (mCRPC).
9. **FoundationOne® Liquid CDx:** Four significant indication expansions, as follows:
- Indications expanded to include detection of BRCA1 and BRCA2 alterations from plasma samples to guide decisions regarding the use of Akeega (niraparib + abiraterone acetate) for the treatment of Metastatic Castrate Resistant Prostate Cancer (mCRPC).
  - Indications expanded to include detection of BRCA1 and BRCA2 alterations from plasma samples to guide decisions regarding the use of Lynparza (olaparib) in combination with abiraterone for the treatment of Metastatic Castrate Resistant Prostate Cancer (mCRPC).
  - Indications expanded to include detection of PIK3CA mutations from plasma samples to guide decisions regarding the use of Itovebi (inavolisib) in combination with palbociclib and fulvestrant for the treatment of Breast Cancer.
  - Indications expanded to include detection of MET alterations from plasma samples to guide decisions regarding the use of Tepmetko (tepotinib) for the treatment of Non-Small Cell Lung Cancer (NSCLC).

10. **OncoPrint Dx Target Test:** Indications expanded to include detection of IDH1 and IDH2 variants (IDH1 R132C, IDH1 R132G, IDH1 R132H, IDH1 R132L, IDH1 R132S, IDH2 R172M, IDH2 R172K, IDH2 R172W, IDH2 R172S, and IDH2 R172G) to guide decisions regarding the use of Voranigo (vorasidenib) for the treatment of Astrocytoma and Oligodendroglioma.
11. **TruSight Oncology Comprehensive:** Two significant indication expansions, as follows:
  - Indications expanded to include detection of NTRK1, NTRK2, and NTRK3 fusions to guide decisions regarding the use of Vitrakvi (larotrectinib) for the treatment of solid tissue tumors.
  - Indications expanded to include detection of RET fusions to guide decisions regarding the use of Retevmo (selpercatinib) for the treatment of Non-Small Cell Lung Cancer (NSCLC).

## Early Detection Cancer Screening Approval

In 2024, CDRH approved the Cologuard Plus updated test platform from Exact Sciences, the company's next generation multitarget stool DNA test. The Cologuard Plus test is now approved for adults ages 45 and older who have average risk for colorectal cancer (CRC). The updated test platform improves upon sensitivity and specificity of the previously approved test. This provides access to an improved personalized medicine screening tool that will lead to early detection and improved outcomes in colorectal cancer.

# POLICY DEVELOPMENTS

## Diversity Action Plans

In 2024, FDA issued a draft guidance document, titled Diversity Action Plans to Improve Enrollment of Participants from Underrepresented Populations in Clinical Studies, outlining FDA's expectations for Diversity Action Plans (DAPs), as mandated by the Food and Drug Omnibus Reform Act of 2022. The draft guidance describes the form, content, and process for including diversity action plans in clinical studies that are part of new drug applications. The FDA DAPs are meant to help ensure that clinical trials are more inclusive and representative of the entire population so that research findings can help in the development of personalized health care interventions with potential improved outcomes that apply to diverse patient populations.

## CDER Artificial Intelligence Council

Artificial Intelligence (AI) is poised to play a pivotal role in advancing personalized medicine. By integrating and analyzing varied biomedical data, including molecular data, electronic health records, and patient-generated health information, new AI technologies are poised to help drive personalized medicine into clinical practice. In an effort to ensure the safety and security of novel machine learning health-related software and AI tools, and to better plan for the oversight of AI within pharmaceutical research, development and applications, the Center for Drug Evaluation and Research (CDER) established a new AI Council. The council replaces the Center's separate AI-related steering committee and policy working groups and will continue its work within the federal government-wide AI Community of Practice to help develop appropriate oversight mechanisms.

## Center for Real-World Evidence Innovation

In 2024, the FDA established the new [CDER Center for Real-World Evidence Innovation \(CCRI\)](#) which aims to coordinate, advance, and promote the use of real-world evidence (RWE) in regulatory decision-making across CDER. CCRI is meant to create a focal point within FDA to identify ways to utilize RWE to streamline the development of effective and safe medicines that address the needs of individual patients.

## Rare Disease Innovation Hub

With the goal of improving outcomes for rare disease patients, the FDA also created the [Rare Disease Innovation Hub](#) in 2024 to serve as a point of collaboration and connectivity between the Center for Biologics Evaluation and Research (CBER) and the Center for Drug Evaluation and Research (CDER). The Hub aims to enhance collaboration across the FDA to address common scientific, clinical, and policy issues related to rare disease product development. The Hub plans to work across all rare diseases, with a focus on challenges within smaller populations, with dedicated workstreams that can help advance personalized medicines by considering novel endpoints, biomarker development and assays, innovative trial design, real world evidence, and statistical methods.

# CONCLUSION

## Realizing 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-related developments at FDA in 2024 show that scientific innovation continues to move 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 safety, 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.

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# 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.





**PMC**

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