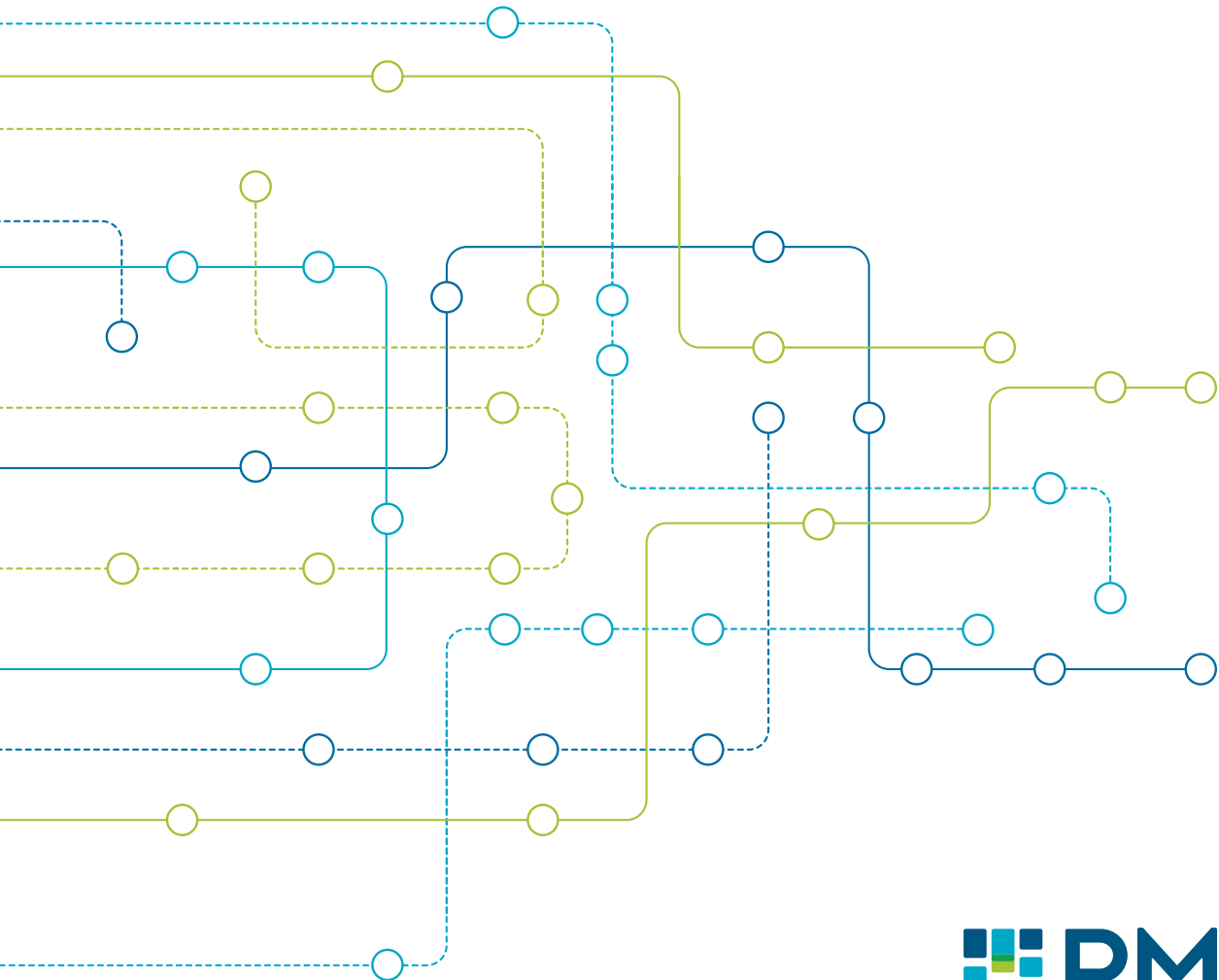

PERSONALIZED MEDICINE AT FDA

The Scope & Significance of Progress in 2025



MILESTONES

1. Approval of 16 personalized medicines representing approximately 36 percent of all newly approved therapeutic molecular entities. Personalized medicines have now accounted for at least a third of new drug approvals for each of the last six years.
2. Approval of five 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 2025 extend the benefits of these personalized treatment approaches to patients with rare genetic diseases including idiopathic macular telangiectasia type 2 (MacTel), recessive dystrophic epidermolysis bullosa (RDEB), spinal muscular atrophy (SMA), and Wiskott-Aldrich syndrome (WAS), as well as to those with hematological cancers who are planning to receive umbilical cord blood transplantation.
3. Expedited authorization of the first bespoke CRISPR gene-editing therapy for an individual patient, enabling treatment of an infant with a rare metabolic disorder. This highlights the application of flexible regulatory pathways for highly customized therapies and signals the advent of novel personalized medicine approaches in which gene-editing treatments can be tailored to a patient's unique genetic mutation.
4. Clearance or approval of significant new or expanded indications for 16 companion diagnostic testing systems that can help target treatments to only those who will benefit, sparing expenses and side effects for those who will not. In addition to the newly cleared and approved companion tests and indications, The FDA cleared or approved:
 - Two new blood-based biomarker tests for Alzheimer's disease, which extend the benefits of personalized medicine to patients with cognitive decline to help rule out Alzheimer's pathology or to measure biomarkers in plasma for early, less invasive Alzheimer's disease detection.
5. Rescinded the 2024 final rule aimed at regulating laboratory-developed tests (LDTs) as medical devices. This action followed a federal court decision vacating the prior rule and maintaining regulation of LDTs under the Clinical Laboratory Improvement Amendments (CLIA) and applicable state law.
6. Released two draft guidance documents to establish frameworks for integrating AI into medical product development. "Considerations for the Use of AI to Support Regulatory Decision-Making for Drug and Biological Products" helps establish a risk-based framework for integrating AI into drug and biologic development, focusing on model credibility, data quality, and, in many cases, human oversight. "Artificial Intelligence-Enabled Device Software Functions: Lifecycle Management and Marketing Submission Recommendations" emphasizes ensuring AI tools used for development and clinical implementation of personalized medicine and other technologies are validated, transparent, and maintain consistent performance throughout their lifecycle.
7. Qualification of the first AI-based drug development tool, AIM-MASH, for use in assessing liver biopsies in metabolic dysfunction-associated steatohepatitis (MASH). This marked the first qualification of an AI-based tool under FDA's Drug Development Tool Qualification Program and established a new regulatory pathway for integrating AI-driven methods into clinical trials, supporting more precise evaluation of therapies and advancing the development of personalized medicine.
8. Released final guidance, titled "Patient-Focused Drug Development: Selecting, Developing, or Modifying Fit-for-Purpose Clinical Outcome Assessments." This guidance is the third in a series of four methodological patient-centered drug development guidance documents that describe how patients, researchers, medical product developers, and others can acquire and submit patient experience and other relevant patient information to be used for personalized medicine product development and regulatory decision-making.
9. Convened two Rare Disease Innovation, Science, and Exploration (RISE) workshops through its FDA Rare Disease Innovation Hub. "On the Rise: Controls in Rare Disease Trials for Small and Diminishing Populations" and "Individualized Therapies on the RISE" brought together more than 2,400 stakeholders to advance regulatory science for rare disease treatment development, including approaches to individualized therapies.

INTRODUCTION

The shift from a one-size-fits-all model of care toward a more precise, personalized approach continues to gain momentum, driven by sustained advances in science, technology, and clinical application. In 2025, the U.S. Food and Drug Administration expanded its record of approvals for personalized medicine innovations, including targeted therapies, gene and cell-based treatments, and increasingly sophisticated diagnostic tools that help predict disease risk and guide treatment selection. These developments are broadening the reach of personalized care across oncology, rare diseases, and a growing number of other conditions. At the same time, the FDA is making incremental progress in modernizing its regulatory framework to address the integration of artificial intelligence into clinical diagnostics and drug development, helping to ensure that these emerging tools are both safe and effective while supporting continued innovation.

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 16 new personalized medicines in 2025, personalized medicines have accounted for at least a quarter of new drug approvals for the past eleven years, while for the last six years they have accounted for over a third. This figure represents an upward trend. Just over a decade ago, personalized medicines accounted for less than 10 percent of the new therapies approved each year.

In 2025, FDA also expanded the indications for many existing personalized therapies; approved five new gene and cell-based therapies; made several advancements in developing frameworks for integrating AI into medical product development and clinical diagnosis; and approved many new diagnostic indications that will allow for targeted treatment decisions for cancer and other health conditions. The newly approved products will provide patient access to safer and more efficacious treatments and prevention regimens based on the principles of more precise personalized medicine.

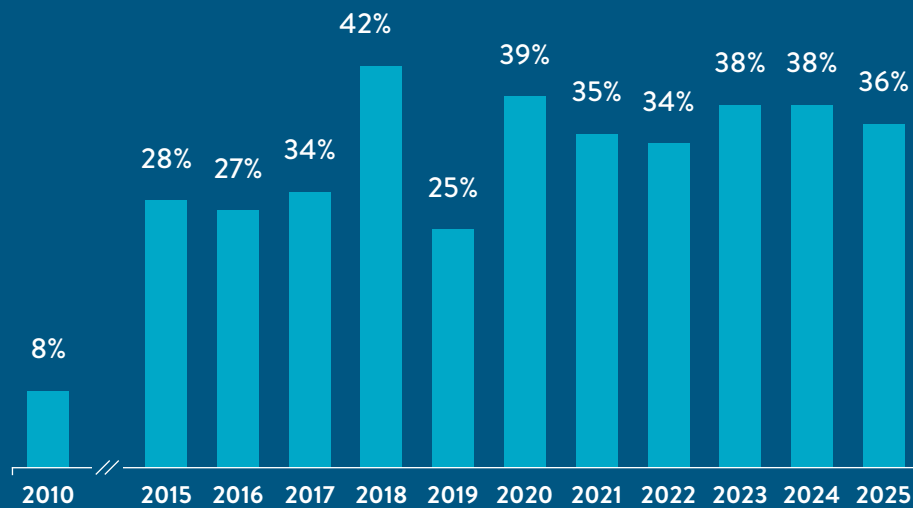
A CONTINUED UPWARD TREND IN PERSONALIZED MEDICINE DEVELOPMENT

Personalized Medicines Account for More Than a Third of All New Therapeutics Approved Since 2020

FDA's Center for Drug Evaluation and Research (CDER) approved 46 new molecular entities (NMEs) in 2025. All but one of these NMEs are therapeutic products (one non-therapeutic NME included a preparative agent for stem cell transplantation). Of the 45 therapeutic NMEs, 16 of them — approximately 36 percent — are personalized medicines as classified by the Personalized Medicine Coalition (PMC). Personalized medicines now account for more than a third of the new therapeutics approved since 2020. They have comprised more than a quarter of new drug approvals for the last eleven years.

In addition, FDA's Center for Biologics Evaluation and Research (CBER) approved five new gene or cell-based therapies in 2025. 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 34 gene or cell-based therapies.

Percentage of Newly Approved Drugs That Are Personalized Medicines, by Year



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.

2025 APPROVALS

16 of the 45 new therapeutic molecular entities FDA approved in 2025 — as well as five new gene/cell-based therapies — are personalized medicines.

1. **Datroway (datopotamab deruxtecan-dlnk)** – for the treatment of breast cancer. The decision to use this product is informed by the status of the hormone receptor (HR-) and human epidermal growth factor receptor 2 (HER2) (IHC 0, IHC 1+ or IHC 2+/ISH-) biomarkers in the tumors of patients.
2. **Vanrafia (atrasentan)** – for the treatment of proteinuria in adults with primary immunoglobulin A nephropathy (IgAN). The decision to use this product is informed by the status of the UPCR (urine protein-to-creatinine ratio (UPCR) ≥ 1.5 g/g) biomarker in patients.
3. **Penpulimab-kcqx (penpuliimab-kcqx)** – for the treatment of metastatic nonkeratinizing nasopharyngeal carcinoma (NPC). 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.
4. **Imaavy (nipocalimab-aahu)** – for the treatment of generalized myasthenia gravis (gMG). The decision to use this product is informed by the status of the anti-acetylcholine receptor (AChR) or anti-musclespecific tyrosine kinase (MuSK) biomarkers in patients.
5. **Avmapki Fakzynja Co-Pack (avutometinib and defactinib)** – for the treatment of recurrent low-grade serous ovarian cancer (LGSOC). The decision to use this product is informed by the KRAS biomarker status in the tumors of patients.

6. **Emrelis (telisotuzumab vedotin-tllv)** – for the treatment of non-squamous non-small cell lung cancer (NSCLC). The decision to use this product is informed by the C-Met biomarker status in the tumors of patients.
7. **Ibtrozi (taletrectinib)** – for the treatment of non-small cell lung cancer (NSCLC). The decision to use this product is informed by the ROS1 biomarker status in the tumors of patients.
8. **Zegfrovy (sunvozertinib)** – for the treatment of non-small cell lung cancer (NSCLC). The decision to use this product is informed by the epidermal growth factor receptor (EGFR) exon 20 biomarker status in the tumors of patients.
9. **Modeyso (dordaviprone)** – for the treatment of diffuse midline glioma. The decision to use this product is informed by the H3 K27M biomarker status in the tumors of patients.
10. **Hernexeos (zongertinib)** – for the treatment of non-squamous non-small cell lung cancer (NSCLC). The decision to use this product is informed by the HER2 (ERBB2) biomarker status in the tumors of patients.
11. **Keytruda Qlex (pembrolizumab and berahyaluronidase alfa-pmph)** – for the treatment of solid tumor cancers. The decision to use this product can be informed by the status of multiple biomarkers in the tumors of patients depending on indication, including PD-L1 expression, EGFR and ALK, metastatic microsatellite instability high (MSI-H), mismatch repair deficient (dMMR), mismatch repair proficient (pMMR), tumor mutational burden high (TMB-H), and HR and HER2.
12. **Inluriyo (imlunestrant)** – for the treatment of advanced or metastatic breast cancer. The decision to use this product is informed by the ER, HER2, and ESR1 biomarkers status in the tumors of patients.

Methodology: When evaluating new molecular entities, 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.

13. **Kygevi (doxectine and doxribtimine)** – for the treatment of thymidine kinase 2 deficiency (TK2d). The use of this product is informed by the TK2 gene variant status in patients, and its use can be informed by the ALT and AST biomarkers status in patients.
14. **Komzifti (ziftomenib)** – for the treatment of acute myeloid leukemia (AML). The decision to use this product is informed by the NPM1 biomarker status in the tumors of patients.
15. **Redempro (plozasiran)** – for the adjunct treatment of familial chylomicronemia syndrome (FCS). 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.
16. **Hyrnuo (sevabertinib)** – for the treatment of non-squamous non-small cell lung cancer (NSCLC). The decision to use this product is informed by the HER2 (ERBB2) biomarker status in the tumors of patients.

Five Newly Approved Gene or Cell-Based Therapies

1. **Encelto (revakinagene taroretcel-lwey)** – for the treatment of adults with idiopathic macular telangiectasia type 2 (MacTel), a rare neurodegenerative disease of the retina that leads to progressive loss of central vision. The treatment is an allogeneic encapsulated cell-based gene therapy. The therapy consists of genetically modified cells that continuously secrete recombinant human ciliary neurotrophic factor (CNTF), a protein that supports photoreceptor survival and slows retinal degeneration.
2. **Zevaskyn (prademagene zamikeracel)** – for the treatment of wounds in adult and pediatric patients with recessive dystrophic epidermolysis bullosa (RDEB). The treatment is an autologous cell-based gene therapy consisting of a patient's own keratinocytes that are genetically modified using a viral vector to express functional type VII collagen (COL7A1). The modified cells promote durable wound closure and restore skin integrity.

3. **Itvisma (onasemnogene abeparvovec-brve)** – for the treatment of spinal muscular atrophy (SMA) in pediatric and adult patients 2 years of age and older with a confirmed mutation in the survival motor neuron 1 (SMN1) gene. The treatment is an adeno-associated virus vector-based gene therapy designed to deliver a functional copy of the SMN1 gene to motor neurons, enabling production of the SMN protein that is necessary for motor neuron survival and function.
4. **Omisirge (omidubicel-only)** – for the treatment of hematologic cancer patients with severe aplastic anemia (SAA) who require hematopoietic stem cell transplantation. The treatment is an allogeneic hematopoietic progenitor cell therapy derived from umbilical cord blood. The therapy is designed to enhance engraftment and accelerate the recovery of neutrophils and other blood and immune cells following transplantation.
5. **Waskyra (etuvetidigene autotemcel)** – for the treatment of patients with Wiskott-Aldrich syndrome (WAS) who have a mutation in the WAS gene and for whom hematopoietic stem cell transplantation (HSCT) is appropriate. The treatment is a lentiviral vector-based gene therapy consisting of autologous CD34+ hematopoietic stem cells that are genetically modified to introduce a functional copy of the WAS gene, enabling restoration of immune and hematologic function.

Other Important Trends in Drug Approvals

Expanding Indications

The new therapies classified as personalized medicines in 2025 do not provide the whole picture of the growing list of personalized medicines available to doctors and their patients. In addition to the 16 newly approved personalized NMEs and the five newly approved gene and cell-based therapies, FDA approved many significant new personalized medicine indications for existing drugs and combinations of drugs in 2025. These approvals redefine the drugs' intended populations and often provide patients with more effective personalized treatment options.

The list of new personalized medicines in 2025 should therefore be complemented with reference to newly approved indications and combinations for Keytruda (pembrolizumab), Calquence (acalabrutinib), Vectibix (panitumumab) in combination with Lumakras (sotorasib), Adcetris (Brentuximab vedotin), Pluvicto (lutetium Lu 177 vipivotide tetraxetan), Imfinzi (durvalumab) in combination with gemcitabine and cisplatin, Opdivo (nivolumab) in combination with Yervoy (ipilimumab), Keytruda Qlex (pembrolizumab and berahyaluronidase alfa-pmph), Darzalex Faspro (daratumumab and hyaluronidase-fihj), Koselugo (selumetinib), Breyanzi (lisocabtagene maraleucel), Jaypirca (pirtobrutinib), Akeega (niraparib/abiraterone acetate), Enhertu (fam-trastuzumab deruxtecan-nxki) in combination with Perjeta (Pertuzumab), and Rubraca (rucaparib).

The expanded indication for Keytruda Qlex, a formulation of pembrolizumab, which was previously approved for treatment of several distinct cancer types with use often guided by PD-L1 biomarker expression, is now indicated for patients with all solid tumor types. This expanded, tumor-agnostic indication reinforces the growing role of biomarker-driven immunotherapy, enabling more patients to access treatment options informed by the molecular characteristics of their disease rather than tumor origin. It further advances a personalized medicine approach by broadening the applicability of immunotherapies across diverse patient populations.

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

Also significant among these expanded indications is the newly approved use of Koselugo (selumetinib), which was previously approved for pediatric patients with neurofibromatosis type 1 (NF1) and symptomatic, inoperable plexiform neurofibromas. This expanded indication addresses an unmet need, as Koselugo is now indicated for patients as young as 1 year of age, extending the benefits of a targeted therapy to an even younger pediatric population with a rare genetic condition. It supports earlier intervention based on underlying disease biology, providing a treatment option earlier in disease progression with great potential to improve patient outcomes.

IMPACT

First Time Approval of a Custom Targeted Therapy for a Single Patient and Further Progress in Targeting RAS mutations

Expedited FDA Approval of a Bespoke CRISPR Therapy

In 2025, the FDA played a pivotal role in one of the most significant personalized medicine milestones to date by rapidly authorizing an individualized CRISPR-based gene-editing therapy for an infant with severe carbamoyl phosphate synthetase 1 (CPS1) deficiency, a rare and often fatal metabolic disorder. After clinicians at the University of Pennsylvania and Children's Hospital of Philadelphia developed a therapy tailored to a pediatric patient's (Baby KJ) unique mutation, they submitted an expanded-access Investigational New Drug (IND) application on February 14, 2025. The FDA approved the request within one week, enabling treatment to begin later that month. This expedited review demonstrated the agency's ability to apply regulatory flexibility in urgent, life-threatening cases where no approved treatment options exist, while maintaining appropriate standards for safety and oversight.

The impact on the patient was profound, underscoring the promise of precision therapeutics. Following treatment, Baby KJ showed meaningful clinical improvement, including greater tolerance for dietary protein, reduced need for supportive medications, and improved resilience during routine childhood illnesses. **By the end of 2025, he had taken his first steps and was later reported to be walking, talking, growing, and thriving.**

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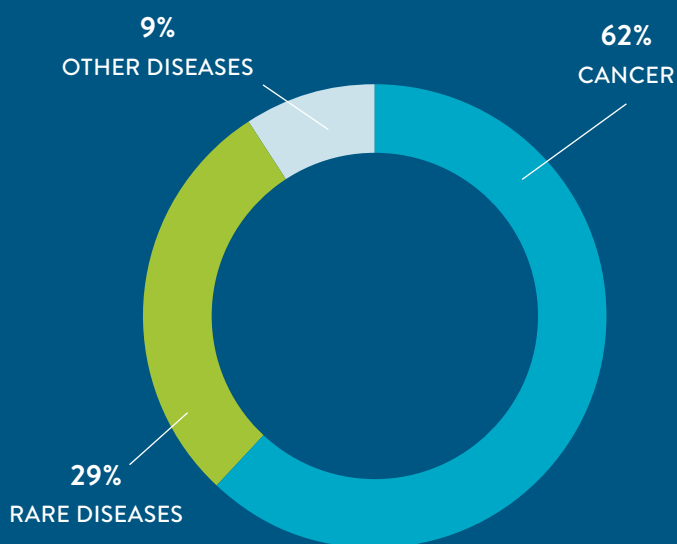
Approval of KRAS-Directed Treatment of Ovarian Cancer

The approval of Avmapki Fakzynja Co-Pack (avutometinib and defactinib) for patients with recurrent low-grade serous ovarian cancer (LGSOC) marked an important advance for a rare ovarian cancer subtype that has historically had limited treatment options and relative resistance to conventional chemotherapy. Use of the therapy is informed by the KRAS biomarker status, helping identify patients whose tumors are more likely to benefit from this targeted approach. For patients with recurrent disease, the approval introduced a new precision medicine option that can improve tumor response and disease control while offering an alternative to repeated courses of less effective systemic therapy.

The approval also reflects broader progress in oncology drug development by demonstrating that KRAS-driven cancers, once widely considered “undruggable,” can be successfully targeted through biomarker-guided strategies, and this may encourage further development of targeted therapies for additional KRAS-altered solid tumors.

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

Includes 16 New Molecular Entities and Five Newly Approved Gene and Cell-Based Therapies



■ Cancer

1. Datroway
2. Penpulimab-kcqx
3. Avmapki Fakzynja Co-Pack
4. Emrelis
5. Ibtrozi
6. Zegfrovy
7. Modeyso

■ Rare Diseases

8. Hernexeos
9. Keytruda Qlex
10. Inluriyo
11. Komzifti
12. Hyrnuo
13. Omisirge

■ Other Diseases

1. Imaavy
2. Vanrafia

NEW DIAGNOSTICS

Newly Approved, Authorized, and Cleared Diagnostic Indications Guide Personalized Treatment Decisions and Enable Earlier Detection

The use of diagnostics to discern biomarker statuses to guide drug use and prevention strategies is the cornerstone of personalized medicine. In 2025, FDA's Center for Devices and Radiological Health (CDRH) approved or cleared several significant new or expanded indications within 16 in vitro diagnostic testing applications that underpin personalized medicine strategies. New approvals and expanded indications associated with the 16 companion diagnostic testing platforms listed below will help inform targeted treatment decisions to improve drug safety and efficacy.

Significant New Approvals/Indication Expansions

1. **Therascreen KRAS RGQ PCR Kit:** Indications expanded to include detection of *KRAS G12C mutations* to guide treatment decisions regarding the use of Lumakras (sotorasib) in combination with Vectibix (panitumumab) for patients with colorectal cancer.
2. **FoundationOne CDx:** Indications expanded to include detection of *BRAF V600 mutations and BRAF gene fusions* to guide treatment decisions regarding the use of Ojemda (tovorafenib) for patients with low-grade glioma.
3. **PATHWAY anti-HER2/neu (4B5) Rabbit Monoclonal Primary Antibody:** Approved the biomarker testing platform as a device for two indications, as follows:
 - Indications approved for the detection of *HER2 (ERBB2) ultralow protein expression* to guide treatment decisions regarding the use of Enhertu (fam-trastuzumab deruxtecan-nxki) for patients with breast cancer.
 - Indications approved for the detection of *HER2 (ERBB2) overexpression* to guide treatment decisions regarding the use of Enhertu (fam-trastuzumab deruxtecan-nxki) in combination with Perjeta (pertuzumab) in patients with breast cancer.

4. **Oncomine Dx Express Test:** Approved for the detection of *EGFR exon 20 insertion mutations* to guide treatment decisions regarding the use of Zegfrovy (sunvozertinib) for patients with non-small cell lung cancer (NSCLC).
5. **MMR IHC Panel pharmDx (Dako Omnis):** Approved for the detection of *deficient mismatch repair (dMMR) protein expression* to guide treatment decisions regarding the use of Opdivo (nivolumab), alone or in combination with Yervoy (ipilimumab), for patients with colorectal cancer.
6. **Idylla CDx MSI Test:** Approved for the detection of *microsatellite instability-high (MSI-H) status* to guide treatment decisions regarding the use of Opdivo (nivolumab), alone or in combination with Yervoy (ipilimumab), for patients with colorectal cancer.
7. **Oncomine Dx Target Test:** Two significant indication expansions, as follows:
 - Indications expanded to include detection of *ERBB2 (HER2) activating mutations* to guide treatment decisions regarding the use of Hernexeos (zongertinib) for patients with non-small cell lung cancer (NSCLC).
 - Indications expansion to include the detection of *ERBB2 (HER2) activating mutations* to guide treatment decisions regarding use of Hyrnuo (sevabertinib) in patients with NSCLC.
8. **Vysis ALK Break Apart FISH Probe Kit:** Approved for the detection of *ALK gene rearrangements* to guide treatment decisions regarding the use of Ensacove (ensartinib) for patients with non-small cell lung cancer (NSCLC).
9. **VENTANA MET (SP44) Rx Dx Assay:** Approved for the detection of *MET protein expression* to guide treatment decisions regarding the use of Emrelis (telisotuzumab vedotin-tlv) for patients with non-small cell lung cancer (NSCLC).
10. **INNOVANCE Antithrombin:** Indications expanded to include the measurement of functionally active *antithrombin* to guide treatment decisions regarding the use of Qfitlia (fitusiran) for patients with hemophilia A or B, with or without inhibitors.
11. **ConcizuTrace ELISA:** Authorized for the measurement of *tissue factor pathway inhibitor (TFPI) levels* to guide treatment decisions regarding the use of Alhemo (concizumab) for patients with hemophilia A or B, with or without inhibitors.

12. **Quest Diagnostics AAVrh74 Antibody ELISA CDx:** Approved for the detection of *anti-AAVrh74 antibodies* to guide treatment decisions regarding the use of Elevidys (delandistrogene moxeparvovec-rokl) for patients with Duchenne muscular dystrophy.
13. **Guardant360 CDx:** Indications expansion to include detection of ESR1 mutations to guide treatment decisions regarding use of Inluriyo (imlunestrant) for patients with breast cancer.
14. **KMT2A Breakapart FISH Probe Kit PDx (CDA-LPH013):** Approved for the detection of KMT2A rearrangements to guide treatment decisions regarding the use of Revuforj (revumenib) for patients with acute leukemia.
15. **OncoMate MSI Dx Analysis System:** Approved for the detection of microsatellite instability (MSI) to guide treatment decisions regarding Keytruda (pembrolizumab) in combination with Lenvima (lenvatinib) for patients with endometrial carcinoma.
16. **Ventana HER2 Dual ISH DNA Probe Cocktail:** Indications expansion to include the detection of HER-2/neu (ERBB2) gene amplification to guide treatment decisions regarding Enhertu (fam-trastuzumab deruxtecan-nxki) in combination with Perjeta (pertuzumab) for patients with breast cancer.

New Blood-Based Diagnostics in Alzheimer's Disease

In 2025, the FDA's CDRH cleared or approved several important blood-based diagnostic applications for Alzheimer's disease, marking a significant step toward earlier and less invasive detection. Among these, the **Roche Elecsys pTau181 Assay** can help rule out Alzheimer's pathology in primary care settings for patients presenting with cognitive decline, while the **Fujirebio Lumipulse G pTau217/ β -amyloid Ratio Test** measures plasma biomarkers to support early detection of disease. These advances expand access to scalable, minimally invasive testing approaches that can be more readily integrated into routine clinical care. Together, they represent meaningful progress in personalized medicine by enabling earlier diagnosis and more informed clinical decision-making.

POLICY DEVELOPMENTS

Integration of Artificial Intelligence in Medical Product Development

In 2025, FDA advanced the use of artificial intelligence (AI) in personalized medicine through two draft guidance documents and qualification of the first AI-based drug development tool. The draft guidance “Considerations for the Use of Artificial Intelligence to Support Regulatory Decision-Making for Drug and Biological Products” outlines a risk-based framework for AI in drug and biologic development, while “Artificial Intelligence-Enabled Device Software Functions: Lifecycle Management and Marketing Submission Recommendations” provides recommendations to ensure AI-enabled devices remain safe, effective, and reliable throughout their lifecycle. FDA also qualified AIM-MASH as the first AI-based tool under the Drug Development Tool Qualification Program for assessing liver biopsies in metabolic dysfunction-associated steatohepatitis (MASH). Together, these actions establish new regulatory pathways for trustworthy AI and support more precise diagnostics, improved clinical trials, and broader advancement of personalized medicine.

Laboratory-Developed Test Oversight

FDA withdrew its prior framework that would have phased in medical device requirements for laboratory-developed tests (LDTs) after a federal court ruling set aside the 2024 final rule. As a result, LDT oversight continues primarily through the Clinical Laboratory Improvement Amendments (CLIA) program and applicable state authorities, thereby preserving the existing regulatory structure for many diagnostic tests that support personalized medicine.

FDA advanced the use of artificial intelligence (AI) in personalized medicine through two draft guidance documents and qualification of the first AI-based drug development tool.

Rare Disease Innovation Hub Collaboration

In 2025, FDA convened two Rare Disease Innovation, Science, and Exploration (RISE) workshops through the FDA Rare Disease Innovation Hub: “On the RISE: Controls in Rare Disease Trials for Small and Diminishing Populations” and “Individualized Therapies on the RISE.” The workshops highlighted FDA’s role in building new policy frameworks that can support individualized treatment development and expand the reach of personalized medicine for patients with rare conditions.

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 2025 show that scientific innovation continues to move the health system away from one-size-fits-all, trial-and-error medicine, toward the utilization of individual patient 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.

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.

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

1710 Rhode Island Ave., NW
Suite 700
Washington, DC 20036