FDA Clears First Blood Test Used in Diagnosing Alzheimer's Disease

New Test Provides Less Invasive Option, Reduces Reliance on PET Scans and Increases Diagnosis Accessibility

The U.S. Food and Drug Administration today cleared for marketing the first in vitro diagnostic device that tests blood to aid in diagnosing Alzheimer's disease. The Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio is for the early detection of amyloid plaques associated with Alzheimer's disease in adult patients, aged 55 years and older, exhibiting signs and symptoms of the disease.

"Alzheimer's disease impacts too many people, more than breast cancer and prostate cancer combined," **said FDA Commissioner Martin A. Makary, M.D., M.P.H.** "Knowing that 10% of people aged 65 and older have Alzheimer's, and that by 2050 that number is expected to double, I am hopeful that new medical products such as this one will help patients."

Alzheimer's disease, a brain disorder known to slowly destroy memory and thinking skills, and, eventually, the ability to carry out the simplest tasks, is progressive, meaning that the disease gets worse over time. In most people with Alzheimer's disease, clinical symptoms first appear later in life. Amyloid plaques in a patient's brain are a hallmark sign of Alzheimer's disease. While amyloid plaques can occur in other diseases, being able to detect the presence of plaque, along with other evaluations, helps the doctor determine the probable cause of the patient's symptoms and findings. These plaques can be detected and visualized using amyloid positron emission tomography (PET) brain scans, often years before clinical symptom onset, to aid in diagnosing Alzheimer's disease. PET scans, however, are a costly and time-consuming option and expose patients to radiation.

The Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio measures two proteins, pTau217 and ß-amyloid 1-42, found in human plasma, a component of blood, and calculates the numerical ratio of the levels of the two proteins. This ratio is correlated to the presence or absence of amyloid plaques in the patient's brain, reducing the need for a PET scan. Similar FDA-authorized/cleared tests, one from the same company as this new test, are used with cerebrospinal fluid (CSF) samples, which are collected through an invasive lumbar puncture, also called a spinal tap. This new Lumipulse test only requires a simple blood draw, making it less invasive and much easier for patients to access.

"Nearly 7 million Americans are living with Alzheimer's disease and this number is projected to rise to nearly 13 million," **said Center for Devices and Radiological Health Director Michelle Tarver, M.D., Ph.D.** "Today's clearance is an important step for Alzheimer's disease diagnosis, making it easier and potentially more accessible for U.S. patients earlier in the disease."

During review of the Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio, the FDA evaluated data from a multi-center clinical study of 499 individual plasma samples from adults who were cognitively impaired. The samples were tested by the Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio and compared with amyloid PET scan or CSF test results.

In this clinical study, 91.7% of individuals with Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio positive results had the presence of amyloid plaques by PET scan or CSF test result, and 97.3 % of individuals with negative results had a negative amyloid PET scan or CSF test result. Less than 20% of the 499 patients tested received an indeterminate Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio result.These findings indicate that the new blood test can reliably predict the presence or absence of amyloid pathology associated with Alzheimer's disease at the time of the test in patients who are cognitively impaired. The test is intended for patients presenting at a specialized care setting with signs and symptoms of cognitive decline. The results must be interpreted in conjunction with other patient clinical information.

The risks associated with the Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio are mainly the possibility of false positive and false negative test results.

False positive results, in conjunction with other clinical information, could lead to an inappropriate diagnosis of, and unnecessary treatment for, Alzheimer's disease. This could lead to psychological distress, delay in receiving a correct diagnosis as well as expense and the risk for side effects from unnecessary treatment.

False negative results could result in additional unnecessary diagnostic tests and potential delay in effective treatment. Importantly, the Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio is not intended as a screening or stand-alone diagnostic test and other clinical evaluations or additional tests should be used for determining treatment options.

The FDA reviewed the Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio through the 510(k) premarket notification pathway. A 510(k) notification is a premarket submission made to the FDA to demonstrate that a new device is substantially equivalent to a legally marketed predicate device. The FDA found that the Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio is substantially equivalent to the Lumipulse G ß-amyloid Ratio (1-42/1-40), which is the previously authorized test that uses CSF samples.

The Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio was granted Breakthrough Device designation, a process designed to expedite the development and review of devices that provide for more effective treatment or diagnosis of life-threatening or irreversibly debilitating diseases or conditions.

The FDA issued clearance of the Lumipulse G pTau217/ß-Amyloid 1-42 Plasma Ratio to Fujirebio Diagnostics, Inc.

FDA News released May 16, 2025. www.fda.gov.

FDA Eliminates Risk Evaluation and Mitigation Strategies (REMS) for Autologous Chimeric Antigen Receptor CAR T cell Immunotherapies

Agency determines the safety and effectiveness of these immunotherapies can be assured without a REMS

The U.S. Food and Drug Administration announced today that it has eliminated the Risk Evaluation and Mitigation Strategies (REMS) for currently approved BCMA- and CD19-directed autologous chimeric antigen receptor CAR T cell immunotherapies.

These products are gene therapies that are currently approved to treat blood cancers, such as multiple myeloma and certain types of leukemia and lymphoma.

"The FDA has taken the bold step to remove the Risk Evaluation and Mitigation Strategy requirement from giving CAR T therapies. REMS is a useful safety system, but reevaluation over time helps inform whether a REMS is still needed to ensure that the benefits of a product outweigh its risks," **said FDA Vinay Prasad, M.D., M.P.H., Chief Medical and Scientific Officer and Director, Center for Biologics Evaluation and Research.** "Eliminating the REMS that is no longer needed also expedites the delivery of potentially curative treatments to patients and reduces burden on providers."

A REMS is a safety program that the FDA can require for certain medications with serious safety concerns to help ensure the benefits of the medication outweigh its risks.

The FDA determined that the approved REMS for the following products should be eliminated because a REMS is no longer necessary to ensure that the benefits of the autologous CAR T cell immunotherapies outweigh their risks.

- Abecma (idecabtagene vicleucel)
- Breyanzi (lisocabtagene maraleucel)
- Carvykti (ciltacabtagene autoleucel)
- Kymriah (tisagenlecleucel)
- Tecartus (brexucabtagene autoleucel)
- Yescarta (axicabtagene ciloleucel)

The elimination of REMS for the above products removes the requirements that hospitals and their associated clinics that dispense products must be specially certified and have on-site, immediate access to tocilizumab. The information regarding the risks for these CAR T cell immunotherapies can be conveyed adequately via the current product labeling, which includes a boxed warning for the risks of cytokine release syndrome and neurological toxicities, and medication guides.

"Physicians and institutions now have greater experience identifying and managing toxicities with the currently approved CAR T products," said Richard Pazdur, M.D., FDA Oncology Center of Excellence Director. "This approach will potentially facilitate patient access to these treatments while continuing to prioritize safety."

Continuous monitoring and assessment of the safety of all biological products, including the CAR T cell immunotherapies, is an FDA priority and we remain committed to informing the public when we learn new information about these products.

These products will continue to be subject to safety monitoring, through adverse event reporting requirements in accordance with regulations (21 CFR 600.80). The elimination of the REMS for these products does not change FDA requirements for manufacturers to conduct post marketing observational safety studies to assess the risk of secondary malignancies and long-term safety with follow up of patients for 15 years after product administration.

FDA News released June 27, 2025. www.fda.gov.

FDA Approves First Immunotherapy for Recurrent Respiratory Papillomatosis

Today, the U.S. Food and Drug Administration approved Papzimeos (zopapogene imadenovec-drba), a first-of-its-kind non-replicating adenoviral vector-based immunotherapy for the treatment of adult patients with recurrent respiratory papillomatosis (RRP).

RRP is a rare, chronic disease caused by persistent human papillomavirus (HPV) 6 or 11 infection, leading to the growth of benign tumors in the respiratory tract, most commonly the larynx. The disease is associated with significant morbidity, including voice changes, breathing difficulties, and airway obstruction. There are currently no approved medical therapies that eliminate the need for repeated surgical procedures.

"Randomized trials are not always needed to approve medical products and this approval is proof of that philosophy," **said Vinay Prasad, M.D., M.P.H., Director of the FDA's Center for Biologics Evaluation and Research (CBER)**. "The FDA will always demand the correct clinical study for the specific medical product and disease. Our requirements for products given to tens of millions of healthy people will be different than products given to at most hundreds or thousands of patients with unique diseases."

With an estimated 1,000 new cases diagnosed annually in the U.S., RRP represents a rare disease with significant unmet medical need. Until today, no therapies have been approved for RRP. Papzimeos is administered via subcutaneous injection and is designed to stimulate an immune response against cells infected with HPV types 6 and 11—the causative agents in RRP. The therapy offers a novel mechanism of action distinct from traditional treatments, which have relied primarily on repeated surgical interventions.

"This approval has the potential to transform the treatment landscape for RRP and offer lasting relief for patients who previously faced repeated surgeries to control symptoms of their disease," **said Vijay Kumar, M.D., Acting Director of the Office of Therapeutic Products in CBER**.

Data Supporting Papzimeos

The approval is based on results from a single-arm, open-label trial evaluating Papzimeos in adult patients with RRP who required three or more surgeries per year. Patients received four subcutaneous injections of Papzimeos over 12 weeks following surgical debulking (reduction) procedures.

In the pivotal portion of the study, 51.4% of patients (18/35) achieved a complete response—defined as no need for surgical intervention in the 12 months following treatment. Follow-up data showed that durable responses were maintained in most patients through two years, with a strong correlation between clinical benefit and the induction of HPV 6/11-specific T cells.

The safety profile was favorable, with most treatment-emergent adverse events being mild to moderate. No dose-limiting toxicities were observed, and no treatment-related serious adverse events were reported.

This approval was completed under Priority Review and the product received both Orphan Drug designation and Breakthrough Therapy designation.

The FDA granted approval of Papzimeos to Precigen, Inc.

FDA News released Aug 14, 2025. www.fda.gov.

Source: FDA