



Alzheimer's Association and Alzheimer's Impact Movement Statement for the Record

United States Senate Special Committee on Aging Hearing on “Unlocking Hope: Access to Therapies for People with Rare, Progressive, and Serious Diseases”

October 26, 2023

The Alzheimer's Association and Alzheimer's Impact Movement (AIM) appreciate the opportunity to submit this statement for the record for the United States Special Committee on Aging hearing on “Unlocking Hope: Access to Therapies for People with Rare, Progressive, and Serious Diseases.” The Association and AIM thank the Committee for holding this hearing to discuss the importance of ensuring access to life-changing therapies for the millions of people living with rare, progressive, and serious diseases, such as Alzheimer's and other dementia.

As the Committee is well aware, there has been much controversy around the Centers for Medicare & Medicaid Services' (CMS) coverage of new and innovative Alzheimer's treatments. Last year, CMS unnecessarily limited access to Food and Drug Administration (FDA)-approved Alzheimer's therapies. Specifically, the CMS National Coverage Determination (NCD) on Monoclonal Antibodies Directed Against Amyloid (mAbs) for the Treatment of Alzheimer's Disease continues to limit the abilities of people living with mild cognitive impairment (MCI) and early-stage Alzheimer's disease to access the first class of FDA-approved treatments to change the course of Alzheimer's disease through the use of the coverage with evidence development (CED) registry requirement. We appreciate the strong bipartisan support in Congress and the Committee calling on CMS to open a reconsideration of this decision and provide access without barriers to these breakthrough treatments if patients, along with their clinicians, decide such treatment is right for them.

While we continue to call on CMS to reconsider their decision, we are committed to working with them to ensure widespread, appropriate access to these treatments under the registry requirements and have submitted our own registry, Alzheimer's Network for Treatment and Diagnostics (ALZ-NET), to be an approved CED registry. ALZ-NET is a voluntary provider-enrolled patient registry that collects information on treatments for Alzheimer's disease from patients being evaluated for or treated with novel FDA-approved Alzheimer's therapies. ALZ-NET is also a platform for patients to access resources about Alzheimer's.

Innovation and Breakthrough: Treatment Advances

As with the first drugs in any class, additional therapies build upon initial breakthroughs to deliver more efficacious treatments. Aducanumab (marketed as Aduhelm) received FDA accelerated approval on June 7, 2021. Lecanemab (marketed as Leqembi) received accelerated approval on January 6, 2023, and traditional approval on July 6, 2023. Lecanemab is the first Alzheimer's treatment to receive traditional FDA approval that changes the underlying

biology of the disease, slowing cognitive and functional decline over 18 months, and significantly improving biological markers of Alzheimer's disease. In a study of 1,800 individuals in the early stages of Alzheimer's, lecanemab reduced the rate of cognitive decline by 27 percent. On well-established measures to assess the quality of life for dementia patients and caregivers, it slowed the decline by half. The peer-reviewed, published results show lecanemab will provide individuals with more time to participate in daily life and live independently. This will mean they have more months of recognizing their spouses, children, and grandchildren. This will also mean more time for people to drive safely, take care of family finances, and participate fully in hobbies and interests.

Adding to the strength of evidence around mAbs, on July 17, 2023, full results of the Phase 3 trial of donanemab were released at the Alzheimer's Association International Conference in Amsterdam, Netherlands, and simultaneously published in the *Journal of the American Medical Association*. These results clearly show that donanemab significantly slowed cognitive and functional decline in people with amyloid-positive early symptomatic Alzheimer's disease, confirming the May 2023 topline data release. Study participants at the earliest stage of the disease had an even greater benefit, with a 60 percent slowing of decline compared to placebo. According to the manufacturer, the FDA is expected to issue a traditional approval decision on donanemab before the end of 2023. Additional clinical trials are underway and offer the hope of additional treatments.

This is just the beginning of meaningful treatment advancements. History has shown that approvals of the first drugs in a new category invigorate the field, increase investments in new treatments, and encourage greater innovation. The progress we have seen in this class of treatments and in the diversification of treatment types and targets over the past few years provides hope to those impacted by this devastating disease. While researchers continue efforts to discover new targets and test new treatments, people living with this fatal disease deserve the opportunity to discuss and make the choice with their doctors if an FDA-approved treatment is right for them.

Innovation and Breakthrough: Early Access to Treatments

The Alzheimer's Association and AIM are grateful that the Consolidated Appropriations Act, 2023 included key provisions of the bipartisan Modernizing the Accelerated Approval Pathway Act (S. 4446), and encourages the FDA to strengthen and better utilize the accelerated approval pathway at the FDA. Strengthening the accelerated approval pathway further ensures that all patients living with critical unmet needs have timely access to FDA-approved treatments.

The Association and AIM brought together other stakeholders in this space, including the Juvenile Diabetes Research Foundation (JDRF), to advocate for policies to preserve and strengthen this important regulatory tool. We were glad to support the Modernizing Accelerated Approval Act, and we believe it will preserve the accelerated approval pathway and ensure that all patients living with unmet needs, regardless of their specific condition, can be assured of the FDA's appropriate utilization of the pathway. This important policy will help improve the lives of

families across the United States that have been affected by Alzheimer's and other dementia as well as other progressive, serious and rare diseases, and ensure they have access to innovative therapies as soon as possible.

Innovation and Breakthrough: Diagnostics

Alzheimer's is one of the most significant health issues facing Medicare beneficiaries and their families. As such, an early and accurate diagnosis of the disease is vital, as it has a positive impact on health outcomes, access to treatments, eligibility for clinical trials, and access to much-needed support and services. An essential tool required for the diagnosis of Alzheimer's disease is brain amyloid positron emission tomography (PET) imaging. Amyloid PET imaging enables clinicians to distinguish Alzheimer's disease from other causes of dementia or memory loss, and help ensure appropriate medical care and treatment. On October 13, 2023, CMS announced a decision to expand coverage of PET scans for the diagnosis of Alzheimer's beyond clinical trials. The new policy will retire the NCD which includes the CED data collection requirements and lifetime one scan limit, which create unnecessary barriers to this important diagnostic and patient safety tool. In issuing its new policy, CMS said the specific details of the coverage will be made by the Medicare Administrative Contractors (MACs). We urge the MACs to quickly implement broad and equitable coverage for all Medicare beneficiaries, and we will continue to work with CMS, the MACs, and other payers to ensure all people living with Alzheimer's disease have easy, affordable access to high quality diagnostic tools, treatments, and care.

Increased access to diagnostic PET scans for the treatment of Alzheimer's disease allows patients to receive treatment while they are still in the early stages of the disease and eligible for innovative disease-modifying treatments. The Alzheimer's Association has led the development and implementation of amyloid PET scans in Alzheimer's research, diagnosis, and care. For example, the Alzheimer's Association and CMS have worked together on both the [Imaging Dementia — Evidence for Amyloid Scanning \(IDEAS\) Study](#) and the [New IDEAS Study](#) to better understand how amyloid PET improves accurate diagnosis and appropriate treatment of Alzheimer's and other dementias in real clinical situations and in diverse and underserved populations. In the IDEAS Study, [patient management changed in 60.2 percent of MCI and 63.5 percent of dementia patients](#). The Association is committed to continuing our work on the New IDEAS Study to further expand our understanding of the importance of accurate diagnosis and PET as a biomarker in traditionally underrepresented individuals.

In addition to PET scans and cerebral spinal fluid (CSF) tests, in the next few months to years, it is anticipated that there will be blood tests available equivalent to the specificity and sensitivity of CSF tests, which are currently some of the most accurate diagnostics. These blood tests have the ability to transform the detection, diagnosis, and treatment of Alzheimer's disease with access and ease of testing. These diagnostic blood tests must be covered by Medicare and other payers, without delay and from the beginning to ensure timely access to new and innovative FDA-approved treatments.

Bipartisan Support for Access to Treatments and Diagnostics

Given the impact on constituents across the country, particularly for rural and underserved populations, there has been strong and consistent bipartisan Congressional support for CMS to reconsider its mAb and PET NCDs. Representatives LaHood (R-IL) and Tonko (D-NY) led 72 bipartisan members in February in sending a [letter](#) to the Department of Health and Human Services (HHS) and CMS and led 44 champions in a [follow-up letter](#) in June, emphasizing the urgency and importance of access to FDA-approved Alzheimer's treatments. Senators Collins (R-ME) and Capito (R-WV) led a similar [letter](#) in the Senate, signed by 20 bipartisan leaders. During the numerous budget and legislative hearings in March, April, and May, over 50 bipartisan members in the House and Senate raised Alzheimer's and [questioned](#) HHS Secretary Becerra and Administrator Brooks-LaSure on why CMS holds Alzheimer's treatments to a different standard than other diseases. Adding to the nationwide support, in April, a [bipartisan group of attorneys general](#) from 26 states and territories sent letters urging HHS and CMS to reverse the mAb NCD.

Despite this growing momentum and ample evidence, CMS did not accept the Alzheimer's Association's [request for mAb NCD reconsideration](#) submitted in December 2022. That request included a [letter signed by more than 200 Alzheimer's researchers and experts](#) expressing their confidence in the lecanemab data, saying there should be "no barriers" to accessing the drug once approved. We continue to urge CMS to reconsider the NCD, especially as we continue to see strong data from FDA-approved treatments, those pending before the FDA, and those in the pipeline.

The Alzheimer's Association and AIM support legislation to further improve access to diagnostic imaging tools, through the Facilitating Innovative Nuclear Diagnostics (FIND) Act of 2023 (S. 1544/H.R. 1199). This bipartisan legislation closes a loophole and allows HHS to pay providers separately for all diagnostic radiopharmaceuticals. In this new era of treatment, multiple amyloid PET scans may be needed to test for eligibility, establish a baseline, track changes in a person's amyloid levels over time, and even determine that treatment should be terminated because lower amyloid levels have been achieved. Revising the currently bundled reimbursement policy will further support physicians who provide diagnostic imaging services, such as PET scans, and have the ability to accurately tailor the course of treatment while receiving adequate reimbursement.

Conclusion

Any barrier to accessing FDA-approved treatments and diagnostics — whether cost, coverage, logistics, or knowledge — is unacceptable and is not patient-focused. Restrictions on access can stifle innovation across the scientific spectrum. In the last decade alone and with substantial investment from Congress through appropriations, the scientific community has made great strides to combat Alzheimer's disease. The next few years are likely to be ripe with breakthroughs and innovation so long as there is coverage and access to diagnostics and treatments.

The Alzheimer's Association and AIM appreciate the steadfast support of the Committee and its continued commitment to issues important to the millions of families affected by Alzheimer's disease and other dementia. We look forward to working with you in a bipartisan way to ensure Medicare beneficiaries living with mild cognitive impairment and early-stage Alzheimer's have access to FDA-approved treatments and diagnostics, including PET scans and blood tests.