

Comment Letter from Patient Advocacy Groups

in response to

[Centers for Medicare and Medicaid Services' Proposed Decision Memo: Monoclonal Antibodies Directed Against Amyloid for the Treatment of Alzheimer's Disease](#)

February 9, 2022

Dear Secretary Becerra:

We, the undersigned, write to express our concern that the recent proposed National Coverage Determination (NCD) on anti-amyloid monoclonal antibodies (mAbs) sets a precedent that could have far-ranging and damaging impacts on approval and access to new therapeutics across all disease areas. The draft NCD creates the potential for a new paradigm in which U.S. Food and Drug Administration (FDA) approval is not sufficient for access. It suggests that drug approvals, whether based on Accelerated Approval or on traditional approval to market, will be second-guessed by the Centers for Medicare and Medicaid Services (CMS) and made subject to a new set of requirements that may preclude patients from having access to an FDA-approved product for which they qualify.

The draft NCD sends a signal that the CMS can effectively set aside the FDA's approval of a new therapy and require a duplicative system for its own re-evaluation of the evidence upon which an approval is based. This proposed approach disregards the primacy of the FDA's jurisdiction as therapy regulator, its critical role in overseeing trial design (endpoint selection, methodology, target product profile, and biomarker selection, to name a few areas), and its benefit/risk determination based on significant scientific expertise in the relevant disease state. The unfortunate effect of the decision will be to prevent access for patients who meet the label criteria, especially those from racially, ethnically, or geographically diverse populations that have disproportionate challenges to accessing clinical trials. It creates a system that will thwart the very goal for diversity espoused by the agency.

We urge you to consider the implications of this draft decision. The NCD raises a number of questions that stand unanswered, including:

- 1. Does this determination create a paradigm where FDA approval is not sufficient for patient access?** While CMS states that, "Generally, an intervention is not reasonable and necessary if its risks outweigh its benefits,"¹ based on the proposed NCD, we are left unclear whether the converse remains the standard. *Specifically, when the benefits outweigh the risks, as determined by the FDA, should this not be tantamount to a finding that the intervention is reasonable and necessary?*
- 2. What is the role of Coverage with Evidence development?** As stated by CMS, is it a tool that can be a bridge for patient access while additional evidence is generated? Further CMS's own guidance provides, "CED will not duplicate or replace the FDA's authority in assuring the safety, efficacy, and security of drugs, biological products, and devices."² How does this decision abide by and advance these principles for a CED?

¹ [Centers for Medicare and Medicaid Services. Proposed Decision Memo: Monoclonal Antibodies Directed Against Amyloid for the Treatment of Alzheimer's Disease. January 11, 2022.](#)

² <https://www.cms.gov/medicare-coverage-database/view/medicare-coverage-document.aspx?MCDId=27>

The significant urgency, unmet need, and public health impact of our communities is matched by the potential for scientific discoveries that will transform health outcomes for millions of Americans. We must protect and preserve the processes enabling this progress, while also working towards collaborative solutions to address identified gaps in evidence.

On behalf of millions of patients and caregivers living with different acute, chronic, common, and rare diseases, we ask you exercise the overarching jurisdictional authority inherent in your office as Secretary of Health and Human Services (HHS) to:

- Take steps to address the sweeping, negative impacts of this draft decision;
- Guide CMS and FDA to work in collaboration, not contradiction; and
- Direct CMS to issue a substantially revised NCD that:
 - Respects and relies upon FDA's review authorities; and
 - Assures coverage for beneficiaries who meet the FDA label requirements coupled with a robust post-market evidence generation strategy from various sources including the sponsors' post-market commitments that will deliver real-time, longitudinal data on the safety and efficacy profile across diverse populations for each drug in this class of disease-modifying therapeutics as these may come to market.

We are anxious to work with you toward these important and urgent goals.

Sincerely,

Adult Polyglucosan Body Disease Research
Foundation

Advocacy & Awareness for Immune Disorders
Association (AAIDA)

Alliance for Aging Research

Alpha-1 Foundation

ALS Association

American Behcet's Disease Association

Amyloidosis Research Consortium

Angelman Syndrome Foundation

Avery's Hope

The Balm in Gilead, Inc.

Batten Disease Support and Research Association

BrightFocus Foundation

Cancer Support Community

Caregiver Action Network

Caring Ambassadors Program, Inc.

Color of Crohn's and Chronic Illness

Congenital Adrenal Hyperplasia Research,
Education & Support Foundation (DBA:
CARES Foundation)

Cure GM1 Foundation

Cure Mito Foundation

Cure Sanfilippo Foundation

Cure SMA

Dusty Joy Foundation

EveryLife Foundation for Rare Diseases

Fabry Support and Information Group

Friends of Cancer Research

The Global Foundation of Peroxisomal Disorders

Global Liver Institute

Haystack Project

HealthyWomen

Histiocytosis Association

HIV + Hepatitis Policy Institute

HypoPara Support & Advocacy, Inc.

International Pemphigus Pemphigoid Foundation

Little Hercules Foundation
LUNGeivity Foundation
Marilyn's Legacy
MarylandRARE
MEPAN Foundation
Mission: Cure
National Fabry Disease Foundation
National PKU Alliance
NTM Info & Research
One Rare
The Oral Cancer Foundation
Parent Project Muscular Dystrophy

Partnership to Fight Chronic Disease
Pompe Alliance
Powerful Patient, Inc.
Sarcoidosis of Long Island
Sudden Arrhythmia Death Syndromes (SADS)
Foundation
SynGAP Research Fund (SRF) 501(c)(3)
Texas Rare Alliance
Undiagnosed Diseases Network Foundation
(UNDF)
UsAgainstAlzheimer's
Voices of Alzheimer's