



The Honorable Cathy McMorris Rodgers
2188 Rayburn House Office Building
Washington, DC 20515

The Honorable Frank Pallone
2107 Rayburn House Office Building
Washington, DC 20515

The Honorable Brett Guthrie
2434 Rayburn House Office Building
Washington, DC 20515

The Honorable Anna Eshoo
272 Cannon House Office Building
Washington, DC 20515

RE: NDSS statement for the record, House Energy and Commerce Committee, Subcommittee on Health

Dear Chairwoman Rodgers, Ranking Member Pallone, Subcommittee Chairman Guthrie, and Subcommittee Ranking Member Eshoo:

The National Down Syndrome Society (NDSS) empowers individuals with Down syndrome and their families by driving policy change, providing resources, engaging with local communities, and shifting public perceptions. We write today in response to the House Energy and Commerce Committee Subcommittee on Health's hearing on "*Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology.*" More specifically, we wish to highlight the impact that Medicare coverage pathways for innovative drugs has on individuals with Down syndrome, specifically as it relates to Alzheimer's disease and related dementia.

Individuals with Down syndrome are uniquely situated in the Alzheimer's landscape because they have an extra copy of chromosome 21. The 21st chromosome carries the amyloid precursor protein (APP) gene, which is strongly associated with the formation of amyloid peptides and plaques, a hallmark of Alzheimer's disease. As a result, individuals with Down syndrome have an elevated lifetime risk for developing Alzheimer's disease, with the onset of symptoms coming earlier and progressing faster than in the general population. In fact, Alzheimer's disease is the number one cause of death for individuals with Down syndrome. Despite being adversely affected by Alzheimer's at a rate that is markedly higher than that of the general population, or any other underserved population, the Down syndrome community continues to face barriers to accessing innovative diagnostic, treatment, and care options.

In the Centers for Medicare and Medicaid Services' (CMS) initial Coverage with Evidence Development (CED) framework for anti-amyloid treatments, individuals with Down syndrome and other developmental disabilities were initially excluded from coverage through any pathway. This discriminatory exclusion could have had a profoundly detrimental effect on the Down syndrome community. While tremendous advocacy on behalf of the community was successful in removing this discriminatory exclusion, individuals with Down syndrome were still not included in any clinical trials for this groundbreaking new class of drugs and therefore, critical safety and efficacy data for the Down syndrome community does not exist. The implications of the recent full FDA approval of Leqembi – a monumental accomplishment in the fight against Alzheimer's disease – illustrates the impact of these exclusions.

Many questions remain about the safety of Leqembi for the Down syndrome community. Given that no individuals with Down syndrome were included in clinical and safety trials, many clinicians advise against the prescription of the drug until safety can be assured. Additionally, lack of transparency around coverage



through CMS's registry requirement and at the state level could mean that individuals with Down syndrome may face additional barriers to access and coverage if and when safety can be assured. Unfortunately, it is clear that innovative new drugs and treatments against Alzheimer's disease are still not accessible to a community that needs it most.

As innovation changes the future of the healthcare system, Congress and this Subcommittee can help ensure that individuals with Down syndrome are not left behind by advocating for the inclusion of individuals with Down syndrome in clinical and safety trials for new drugs, ensuring transparency and accountability from CMS on coverage decisions, and making certain that national coverage decisions do not directly or indirectly exclude those who need access to these drugs and treatments most.

Importantly, the Subcommittee can also take up the NAPA Reauthorization Act (H.R. 619/S. 133, as amended) to support coordination of federal planning, programs, and other efforts to address Alzheimer's disease and related dementias. By incorporating the amendments passed by the Senate Health, Education, Labor, and Pensions committee to more explicitly include the Down syndrome community in the work authorized by the National Alzheimer's Project Act (NAPA), Congress can help ensure that the Down syndrome community has a voice when these critical decisions are being made.

NDSS strives to ensure all individuals with Down syndrome are assured their human rights and valued by a more inclusive society. We applaud the Subcommittee for examining these important issues and look forward to working with Congress to advance bipartisan policies that help us achieve this mission.

Sincerely,

A handwritten signature in black ink that reads "Kandi Pickard". The signature is fluid and cursive, with a large loop at the end of the name.

Kandi Pickard
President & CEO
National Down Syndrome Society