Documents for the Record – 07/18/23 Health Hearing

Majority:

- July 18, 2023 Alzheimer's Association statement
- July 18, 2023 American Medical Association statement
- July 18, 2023 GaitBetter statement
- Geneoscopy testimony
- July 18, 2023 AiArthritis statement
- July 17, 2023 LuMind IDSC Foundation statement

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

United States House Committee on Energy and Commerce, Health Subcommittee Hearing on "Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology"

July 18, 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 House Committee on Energy and Commerce, Health Subcommittee hearing on "Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology." The Association and AIM thank the Subcommittee for its continued leadership on issues important to the millions of people living with Alzheimer's and other dementia and their caregivers.

This statement highlights the continued urgency of addressing the Centers for Medicare & Medicaid Services (CMS) coverage decision that unnecessarily limits access to Food and Drug Administration (FDA)-approved Alzheimer's therapies, especially those living in rural and underserved areas. 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 treatments to change the course of Alzheimer's disease. We appreciate the strong bipartisan support in Congress for CMS to immediately open a reconsideration of this decision and provide access without barriers to these breakthrough treatments if patients, along with their clinicians, decide such a treatment is right for them.

Founded in 1980, the Alzheimer's Association is the world's leading voluntary health organization in Alzheimer's care, support, and research. Our mission is to eliminate Alzheimer's and other dementia through the advancement of research, to provide and enhance care and support for all affected, and to reduce the risk of dementia through the promotion of brain health. AIM is the Association's advocacy affiliate, working in a strategic partnership to make Alzheimer's a national priority. Together, the Alzheimer's Association and AIM advocate for policies to fight Alzheimer's disease, including increased investment in research, improved care and support, and the development of approaches to reduce the risk of developing dementia.

Innovation and Breakthrough Treatments

Alzheimer's is one of the most significant health issues facing Medicare beneficiaries and their families, and now, for the first time, treatments have been approved by the FDA that change the course of the disease. 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 (AAIC) 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 disease had an even greater benefit, with 60 percent slowing of decline compared to placebo. According to the company, we anticipate the FDA issuing a traditional approval decision on donanemab before the end of the year. 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 invigorates the field, increases investments in new treatments, and encourages 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 we 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.

Continued Barriers to Accessing FDA-Approved Treatments

In 2022, CMS implemented an unprecedented NCD that not only applies to the two currently approved FDA-approved Alzheimer's therapies but also applies to all future treatments in the same class. Using coverage with evidence development (CED) requirements, CMS only covers mAbs treating Alzheimer's approved through the accelerated approval pathway for individuals enrolled in randomized clinical trials, and treatments approved through the traditional approval pathway when patients are enrolled in a registry. This decision creates an unnecessary barrier to care for older Americans, especially those living in rural and underserved areas.

Following FDA's traditional approval of Leqembi, CMS announced additional details about its low-touch registry. It is clear that CMS leaders listened to experts, members of Congress, people living with Alzheimer's and their families, and advocates across the country in taking action to reduce physician burden in their registry approach. Registries are an important tool to gather much-needed real-world evidence to transform and improve patient care, but should not be a requirement for coverage of a FDA-approved treatment. The Alzheimer's Association and AIM will support implementation of this coverage plan so that doctors can easily navigate the registry process and provide access to their eligible patients. However, we are disappointed that CMS did not take this opportunity to initiate the formal process to reconsider their NCD in order to eliminate all barriers to access and to treat Medicare coverage of Alzheimer's drugs consistent with drugs for all other diseases. We urge CMS to do so immediately.

There continue to be outstanding questions on the scientific need for and implementation of registries as a condition of coverage. CMS has not effectively explained what size and scope of data is needed to end the CED and how long reconsideration may take. It is also unclear how CMS plans to ensure equitable access, particularly for those living in rural and underserved communities, to the treatment via the registry.

The barriers to accessing these mAb treatments led to thousands of Medicare beneficiaries with a progressive, terminal disease, for the first time in history, losing the opportunity to receive FDA-approved treatments. While we welcome the greater access now available to the first traditionally approved drug in this class, this delayed access implicitly concedes that this treatment should have been made available upon FDA's initial approval. It is increasingly evident that CMS' decision to treat this class of mAb treatments differently from all others is out of step with the scientific evidence and, for many who had counted on Medicare's support, it has been a deeply harmful policy.

All individuals, families, and caregivers facing a devastating, fatal disease deserve the opportunity to access FDA-approved treatments. As Alzheimer's Association advocate Sue Wronsky stated in her testimony to the Subcommittee, the benefits of these treatments will only be realized if patients have access. If there had been an FDA-approved treatment in 1991 when her mother was first diagnosed, she may have been able to spend more time with her family and remain independent. Access to treatment that could have slowed her mother's decline would have helped lessen the burdens of caregiving. When facing the diagnosis of a progressive brain disease, the last thing families affected by Alzheimer's need is more roadblocks: they need full access to these treatments.

Bipartisan Support for Access to Treatments

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 CED policy. Representatives LaHood (R-IL) and Tonko (D-NY) led 72 bipartisan members in February in sending a letter to the US 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 NCD.

Despite this growing momentum, CMS denied the Alzheimer's Association's request for 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 bipartisan legislation to ensure timely Medicare coverage of FDA-approved therapies. As no two treatments are the same, it is important that CMS evaluate each treatment individually and based on their own scientific evidence, rather than as one broad category. The Mandating Exclusive Review of Individual Treatments (MERIT) Act (H.R. 133) would require CMS to evaluate treatments and cures individually and based on their own merits, rather than as a broad class of drugs. We also support the bipartisan Access to Innovative Treatments Act (H.R. 2408) which would create a transparent process for ensuring that CMS responds and reconsiders drugs for Medicare coverage when sufficient data is collected on the drug's effectiveness.

Conclusion

Given the substantial new clinical evidence published since the NCD was developed and the FDA's confirmation of clinical benefit in granting traditional approval of a treatment in this class, CMS should reconsider the policy to provide full access for Medicare beneficiaries to these FDA-approved Alzheimer's treatments. Any barrier — whether cost, coverage, logistics, or knowledge — to accessing FDA-approved treatments is unacceptable and is not patient-focused.

The Alzheimer's Association and AIM appreciate the steadfast support of the Subcommittee and its continued commitment to issues important to the millions of families affected by Alzheimer's and other dementia. We ask that the Subcommittee continue to stress the urgency to HHS and CMS of immediately opening a reconsideration of the NCD to remove the CED requirements for FDA-approved mAbs targeting amyloid for the treatment of Alzheimer's, based on substantial new evidence published since the finalization of the NCD. We look forward to working with the Subcommittee and other members of Congress in a bipartisan way to ensure Medicare beneficiaries living with mild cognitive impairment and early-stage Alzheimer's have access to FDA-approved treatments if the patient and clinician decide it is right for them.

STATEMENT
of the
American Medical Association

to the

U.S. House of Representatives
Committee on Energy and Commerce
Subcommittee on Health

Re: Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology

July 18, 2023

Division of Legislative Counsel

202-789-7426

Statement for the Record
of the
American Medical Association
to the
Committee on Energy and Commerce, Subcommittee on Health

Re: Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology

July 18, 2023

The American Medical Association (AMA) appreciates the opportunity to submit the following Statement for the Record to the U.S. House of Representatives Committee on Energy and Commerce Subcommittee on Health as part of its hearing entitled "Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology." The AMA commends the Subcommittee for focusing on creating clear and predictable pathways to payment for innovative technologies that are supported by high quality, clinically validated data that help advance the quadruple aim, and are delivered in a manner that promotes health equity for all.

Support for Innovative Approaches to Coverage and Payment for Emerging Technologies

The AMA supports efforts to develop innovative and novel approaches to promoting adoption of emerging technologies that meet the goals of the quadruple aim[1] and serve to promote health equity. New technologies, especially those that are digitally or augmented intelligence-enabled, present unique challenges to traditional coverage and payment paradigms. We are encouraged to see new ways of thinking, such as the Centers for Medicare and Medicaid's (CMS) recent notice of the Transitional Coverage for Emerging Technologies (TCET) pathway, that seeks to reduce the burdens of bringing beneficial technologies to consumers while ensuring those technologies are safe, effective, and add value to the health care system. We encourage Congress to continue to view the AMA as a resource as it evolves its thinking around payment for innovative technology. The AMA, for its part, is committed to realizing the full potential of digitally enabled care and will continue to expand its digital health resources, federal and state advocacy efforts, and stakeholder engagement initiatives to meet the challenge of bridging the digital health disconnect. We have long supported the development and use of digital tools, in the least burdensome pathways for introduction to market, so long as those tools are met with the conditions that aim to ensure safe and effective use by its end users.

Blueprint for Optimizing Digitally Enabled Care

The AMA supports a digitally enabled hybrid health care system that offers expanded access to care options, and a variety that can fit the needs and preferences of patients and their physicians or other health care providers. In our Future of Health report, we discuss how to close the digital health disconnect with our blueprint for optimizing digitally enabled care. We have entered this era of fully integrated in-person and virtual care models that hybridize care delivery based on clinical appropriateness and other factors, such as convenience and cost. Realizing the full potential of digitally enabled care will require a fundamental rethinking of how care models are designed, implemented, and scaled to solve many of the challenges of today's health care system. The world's thinking of digitally enabled care has been

[1] Principles that meet the goal of the "quadruple aim," should enhance the patient experience of care and outcomes, improve population health, reduce overall costs for the health care system while increasing value, and support the professional satisfaction of physicians and the health care team.

transformed by the COVID-19 pandemic, and we believe the time is now to build on how we think about accessing, providing, managing, and paying for health care.

Evolution, Progress, and Status of Innovative Technology in Health care

It goes without saying that despite potential prior reservations associated with remote delivery of care, evidence will show that with exposure and greater experience, impactful and positive outcomes have resulted for both physicians and their patients. Evidence consistently demonstrates support for telehealth and remote patient monitoring, and we urge Congress to continue to encourage the Administration to believe in the power of these tools and technological capabilities, to increase access to quality care for all populations. The AMA advocates tirelessly to stress the importance of payment for tools and devices to each payor. It is critical that poor coverage or non-coverage determinations do not set a precedent that unravels all the progress that has been made to this point.

AMA and CPT® (Current Procedural Terminology®) as a Leader in Innovation -- The CPT Editorial Panel and Digital Medicine Payment Advisory Group

The AMA has long supported innovative care models and payment reforms that achieve the goals of higher quality patient care.

CPT is consistently utilized today within existing Alternative Payment Models and Value Based Care Models. Through the transparent, rigorous CPT Editorial Panel process, CPT codes can adapt to meet the needs of physicians, qualified health professionals, health systems, policymakers and payers as new models are developed, tested, and implemented. Very pertinent to this specific discussion is the existence of CPT Category III Codes. CPT Category III codes are a set of temporary codes that allow data collection for emerging technologies, services, procedures, and service paradigms. These codes are intended to be used for data collection to substantiate widespread usage or to provide documentation for the U.S. Food and Drug Administration (FDA) approval process. The AMA offers a host of educational resources on CPT Category III Codes to inform, educate, and promote the proper usage of these unique codes.

Another example of CPT guiding the future of payment of technology in health care is the addition of Appendix S to assist CPT stakeholders, including innovators, payors, regulators, medical professionals and patient organizations in the development of CPT code descriptors for artificial intelligence/augmented intelligence medical services and procedures. The AI taxonomy for medical services and procedures provides guidance to classify various AI applications (e.g., expert systems, machine learning, algorithm-based services) into one of three categories: assistive, augmentative, or autonomous. Using terminology for assistive, augmentative, or autonomous in code descriptors differentiates the work done by the physician and the work done by the machine on behalf of the physician to facilitate appropriate valuation and payment of AI.

In January 2017, the AMA established the Digital Medicine Payment Advisory Group (DMPAG), comprised primarily of nationally recognized telehealth physicians practicing at leading health systems around the country, physicians who are subject matter experts in coding and valuation, as well as industry experts with knowledge of expected technology advancements. Consistent with the AMA's goals and policy, the DMPAG provides regular advice and counsel on a clear pathway to clinical integration of digital medicine to ensure equitable access to and availability of high quality and safe clinical care for patients and their physicians that achieve improved health outcomes. The advisory group addresses:

- The creation and dissemination of data supporting the use of digital medicine technologies and services in clinical practice.

- Existing code sets (with an emphasis on CPT and HCPCS) and the level to which they appropriately capture these services and technologies.
- Factors that impact the fair and accurate valuation for services delivered via telehealth.
- Widespread coverage of digital medicine (including telemedicine and remote patient monitoring), including greater transparency of services covered by covered by payers and advocacy for enforcement of parity coverage laws.
- Program integrity concerns of payers including, but not limited to, appropriate code use, and other perceived risks unique to digital medicine.

In short, the AMA strongly emphasizes how critical it is that CPT codes are foundational to the evolution of coverage pathways to innovative technology that have the potential to enhance the physician's health care practice and the overall patient experience.

Conclusion

The AMA remains steadfastly supportive of clear, predictable, least burdensome pathways to coverage for innovative technologies. However, we must ensure that the technologies we provide to our nations' Medicare patients are equitably accessible, safe, effective, high quality, and meet the goals of the quadruple aim. The AMA is committed to continuing to develop resources and insights, advocating for policy changes that support the future of health care, and bringing diverse perspectives across industry to the table to keep building on this work.

July 18, 2023

The Honorable Cathy McMorris Rodgers
Chairman
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Frank Pallone
Ranking Member
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Brett Guthrie
Chairman
Health Subcommittee, Ways & Means Committee
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

The Honorable Anna Eshoo
Ranking Member
Health Subcommittee, Ways & Means Committee
Ways & Means Committee
1139 Longworth House Office Building
Washington, DC 20515

Dear Chairs McMorris Rodgers, Chairman Guthrie, and Ranking Members Pallone and Eschoo:

On behalf of GaitBetter, thank you for the opportunity to submit a statement for the record for the July 18, 2023 hearing on "Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology." As GaitBetter's head of U.S. sales, I have first-hand experience with the barriers posed by Medicare for innovative technology. As such, I very much appreciate your seeking public input on this important topic and thank you in advance for your attention to the issues, challenges, and recommendations outlined below.

About GaitBetter

GaitBetter, a medical technology company with locations in Maryland and Israel, has developed a patented and clinically-proven virtual reality-based motor-cognitive training solution that has helped reduce falls in older adults by 70%[1]. The technology, created by world-leading neuroscientists, physical therapists, and experts in older adults in the Laboratory for Gait Analysis and Neurodynamics at Tel Aviv Sourasky Medical Center, currently is being used by the U.S. Department of Veterans Affairs as well as by several leading hospital systems across the country. However, due to myriad federal government policies, practices, and programs we have been unable to scale the deployment of the technology, leaving seniors without access to a clinically-proven intervention that can prevent falls and reduce the serious concomitant injuries and death that can accompany them.

[1] GaitBetter. "Case Study: Maccabi Health Services Reducing Falls in Older Adults By 70%." 2022, https://www.gaitbetter.com/case-study-maccabi/

Background

Falls are Common and Costly in the U.S.

Falls are a common problem among older Americans and a leading cause of morbidity, mortality, and use of health care services in the U.S. Falls conservatively account for more than $50 billion in annual health care expenditures, with Medicare and Medicaid paying for an estimated 75% of the costs.[2,3] According to the Centers for Disease Control and Prevention (CDC):[4]

- More than 25% of older adults fall at least once per year and 20% of these falls result in a serious injury, such as a fracture or a traumatic brain injury. [5]
- Each year, three million older adults are treated by the nation's emergency departments for fall injuries.
- Falls among adults 65 and older caused almost 37,000 deaths in 2020[6] – the leading cause of injury death for that population.

Existing interventions to prevent and reduce falls have been insufficient as fall rates, fall injuries per capita, and fall death rates per capita continue to rise.[7] Current falls prevention approaches result in low adherence rates and either focus on short-term individualized therapy or longer-term wellness activities in a group setting, with neither providing optimum results in preventing falls over a large population.

The GaitBetter Falls-Prevention Technology

GaitBetter provides motor-cognitive therapy by adding semi-immersive virtual reality (VR) to existing treadmills, which are used for gait training for falls prevention. The GaitBetter system has a small footprint and easily transforms any existing treadmill into a powerful motor-cognitive training device. In less than two minutes, patients are set up to use the system, ensuring that maximum therapy is received during a visit. Additionally, since patients are secured in a safety harness, therapists can simultaneously attend to multiple patients, increasing efficiency and maximizing the clinic workforce.

---

[2] Department of Housing and Urban Development (HUD). *Overcoming Obstacles to Policies for Preventing Falls by the Elderly Final Report.* 2017, https://www.hud.gov/sites/dfiles/HH/documents/OvercomingObstaclesFalls.pdf

[3] Centers for Disease Control and Prevention. *Facts About Falls*, https://www.cdc.gov/falls/facts.html

[4] Centers for Disease Control and Prevention. *Older Adult Falls Prevention,* https://www.cdc.gov/falls/index.html

[5] Centers for Disease Control and Prevention. *Facts About Falls,* https://www.cdc.gov/falls/facts.html

[6] Santos-Lozada, Alexis R. "Trends in deaths from falls among adults aged 65 years or older in the US, 1999-2020." JAMA 329.18 (2023): 1605-1607.

[7] HUD. *Overcoming Obstacles to Policies for Preventing Falls by the Elderly Final Report.*

The trainee walks on the treadmill and sees her two feet in a simulation projected on a TV screen in front of her. As she walks, she encounters virtual challenges to practice both cognitive and motor skills at the same time. She works on obstacle negotiation and decision making in an environment that is very similar to the real world. Patients' feet movements are analyzed in real-time to drive a VR simulation displayed on a screen (there is no VR headset). A proprietary computer vision algorithm uses a single camera to capture accurate feet tracking, which boosts motor-learning. The patented artificial intelligence algorithm personalizes the intervention for each patient. The individual receives strong/measurable feedback and can track progress over time. This gamification is so motivating that more than 85% of older adults complete the training. Patients easily transfer acquired skills to daily living.

GaitBetter Training Components		
Motor	**Motor-Cognitive**	**Cognitive**
• Gait speed • Step length/Clearance • Endurance • Dynamic balance • Symmetry • Variability	• Obstacle negotiation • Motor planning • Problem solving • Balance strategies • Coordination	• Multitasking • Memory • Response time • Attention • Environment sensory input processing

To see a demonstration of the technology, please visit: https://www.gaitbetter.com/

The Science Behind GaitBetter

The basis of the GaitBetter technology started more than 15 years ago with a longitudinal study of more than 250 dementia-free older adults who had never had a fall.[8] The mental and physical characteristics of this group were closely measured and then time to first fall and number of falls were tracked for five years. ***This study found that the risk of falls only correlated to low levels of cognitive performance, specifically executive function***.

Based on this ground-breaking research, a team from the Neurology and Physical Therapy Departments at Tel Aviv University developed the predecessor of GaitBetter. Clinical effectiveness of the intervention was evaluated in a randomized-controlled trial of more than 300 older adults and the results were published in *The Lancet*.[9] ***The results showed that individuals who used the intervention experienced a 50% reduction in falls after six months,***

[8] Mirelman, Anat, et al. *Executive function and falls in older adults: new findings from a five-year prospective study link fall risk to cognition.* PloS one 7.6 (2012).
[9] Mirelman, Anat, et al*. Addition of a non-immersive virtual reality component to treadmill training to reduce fall risk in older adults (V-TIME): a randomised controlled trial.* The Lancet 388.10050 (2016): 1170-1182.

which is two times more effective than existing interventions (exercise classes, multifactorial interventions, physical therapy).

GaitBetter's Effectiveness

By leveraging the latest research on the neuroscience of aging, GaitBetter has demonstrated a fall reduction rate that is up to three times more effective than existing interventions.[10] The reason for this efficacy is brain plasticity. The research team behind GaitBetter performed functional MRI scans and found unique changes in brain activation in the frontal regions, suggesting that GaitBetter training makes our brains more efficient and better able to handle the multitasking required for successful walking.

GaitBetter Utilization and Demand

As soon as the 2016 *Lancet* paper was published, requests came from clinics around the world asking where and how they could access such a successful technology. Based on this demand, we founded GaitBetter in 2018 and licensed the technology from Tel Aviv University. Our first installation was in May 2019 in Israel. Once we reached a certain level of growth in the company, we expanded into the U.S. with our first installation at Spaulding Rehabilitation Hospital in Boston, Massachusetts in March 2021. As of May 2023, there are 100 systems installed across the U.S. and Israel and we have treated more than 6,000 patients.

Our success in Israel has been explosive. Due to a value-based health care system that prioritizes population health and an environment that incentivizes and rewards innovation that improves outcomes, GaitBetter is now accessible to more than 90% of the Israeli population, available throughout the country. GaitBetter has been implemented in hospitals, outpatient clinics, adult day care centers, and senior living communities. Israeli citizens and the government are benefitting from this proven-effective way to prevent falls among older individuals.

GaitBetter's Potential Savings to Medicare

Gaitbetter is working with the Actuarial Research Corporation (ARC), a veteran-owned small business that is led by former senior professionals from the Medicare Office of the Actuary, to accurately capture the value of utilizing GaitBetter to improve existing gait training practices and reduce the number of falls among Medicare beneficiaries. Based on ARC's initial analysis, we conservatively estimate that GaitBetter would reduce Medicare falls-related costs by $1.1 billion on an annual basis.

[10] Sherrington, C, et al. *Evidence on physical activity and falls prevention for people aged 65+ years: systemic review to inform the WHO guidelines on physical activity and sedentary behaviour.* Int J Behav Nutr Phys Act. 2020.

GaitBetter Faces Significant Challenges in the U.S. Market Due to U.S. Federal Policy

Unfortunately, due in large part to the hurdles for acquiring proper codes and subsequently securing reimbursement for our technology, it is unclear whether GaitBetter can provide this potentially life-saving technology to Medicare beneficiaries. While we have received significant interest in the technology, when potential customers learn that Medicare does not provide payment for its use, they often indicate they are unable to proceed with a purchase. For those customers that have purchased the technology, they struggle to make the purchase revenue neutral due to the lack of Medicare payment. This creates a conundrum: proven and effective falls prevention technology exists but is unavailable for our most vulnerable population.

Medicare Challenges

We applaud the Centers for Medicare and Medicaid Services (CMS) for recognizing the problem of and seeking to reduce the occurrence of falls among the Medicare population; however, the current Medicare payment system fails to provide coverage and reimbursement in a manner that proactively supports beneficiary access to clinically-proven technology to reduce falls.

While our technology is proven effective in published randomized clinical trials, under current Medicare reimbursement policy there is no additional payment available for clinics that wish to use it. There are several existing CPT codes that cover gait training; however, they are reimbursed at a standard rate that does not factor in the purchase or utilization of a technology like GaitBetter. As such, therapists and therapy clinics receive the same level of reimbursement whether or not they use an innovation like the GaitBetter semi-immersive VR technology. Specifically:

- Medicare has no higher paying CPT codes to address combined motor-cognitive therapy; existing codes address either physical therapy **or** cognitive therapy but not both.

- Medicare has no CPT or HCPCS codes for physical therapy that include or reflect the cost of equipment such as GaitBetter, which provides additional clinical value to the patient.

- While Medicare does have a code that allows physical therapists to bill for treating more than one patient at a time, it is not adequately reimbursed for the services provided, which limits its use.

- GaitBetter is not considered Durable Medical Equipment (DME): Because GaitBetter is installed with a standard treadmill in an outpatient therapy clinic, it is not considered DME, so reimbursement for the provider or patient is not available through that pathway.

Due to these limitations with existing CPT codes, the installation and monthly subscription fee currently are considered unreimbursed cost-centers for outpatient therapy clinics, which have limited capital equipment budgets and low margins.

Given that GaitBetter technology is motor-cognitive, semi-immersive virtual reality–facilitated gait training, there was no code to capture the functionality of our product. So, as a proactive measure, in June 2022 we applied to the American Medical Association (AMA) for a new Category III CPT Code, which is a temporary code for utilization of "emerging technologies, services, and procedures." This would serve as an add-on code to gait training, which is currently coded using CPT 97116, to cover the cost of adding semi-immersive VR to this service (CPT 97116). The AMA CPT Editorial Panel approved our application in September 2022 and released the new add-on code (CPT 0791T) in December 2022. While we are grateful for CPT 0791T, it will not be reimbursed until additional hurdles with CMS are overcome.

We have since met or emailed all 12 Medicare Administrative Contractors (MACs), which handle payments for physician office and standalone outpatient clinical settings, to explain the GaitBetter technology, educate them about the new CPT 0791T add-on code, and explain the potential number of beneficiaries that could benefit from this intervention within their region. All the MACs indicated that they would not provide any guidance on coverage and reimbursement until CPT 0791T was effective on July 1, 2023. While we are hopeful that the MACs will assign a payment to the code so therapists and therapy clinics then will have an incentive to purchase and utilize the technology, our experience has revealed that payment drives practice and without additional payment for the technology, it will have a very low adoption rate, despite its clear clinical benefit.

In addition to seeking payment for the technology through the MACs, we are hopeful that Medicare will provide payment through the Hospital Outpatient Prospective Payment System, which reimburses already for gait training therapy provided in hospital outpatient settings. Again, however, there is no existing code that naturally fits a technology like GaitBetter and there is no clarity on which pathway exists for such technology for coverage and payment. Internally, we have identified the new technology Ambulatory Payment Classification (APC) or the transitional pass-through payment (TPT) to cover the costs of the GaitBetter software and equipment. It is unclear whether other pathways to coverage and reimbursement exist. We plan to meet with CMS this week for guidance on the process and hope to have a code for payment become available in January 2024. While we are hopeful for a fruitful conversation with CMS, moving forward, a clear and established pathway for GaitBetter and similarly innovative technologies would be immensely beneficial in ensuring patients receive the care they need and streamlining Medicare payment.

How Congress Can Help

We appreciate your interest in evaluating Medicare coverage pathways for innovative technologies. We remain concerned and frustrated that within the U.S. system we have been unable to consistently offer the benefits of the GaitBetter system to individuals at-risk for falling. To that end, we respectfully urge you and your colleagues to:

- Communicate with MACs and urge them to provide timely payment for new Category III codes that support the utilization of new technology, such as GaitBetter; and

- Encourage CMS to concretely identify pathways for reimbursement of technologies such as GaitBetter.

Summary

Again, on behalf of GaitBetter and the millions of Medicare beneficiaries at risk of falling, thank you for this opportunity to provide input regarding Medicare coverage pathways for innovative technologies. GaitBetter stands ready to be a resource to you and your colleagues and we welcome an opportunity to discuss with you further the policy changes we believe are necessary to improve our nation's incentives for innovation. In particular, there are many lessons learned from our experience in Israel that we would be happy to share. Please feel free to contact me at any time.

Submitted by:
Craig Hillman
SVP, US Operations
GaitBetter
131 Rollins Avenue, Suite 4
Rockville, MD 20852
908-883-3218

Written Testimony for House Committee on Energy and Commerce

Health Subcommittee Hearing

"Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology"

Submitted by

Geneoscopy, Inc.

St. Louis, MO

Thank you for the opportunity to provide written comments regarding evaluating Medicare coverage pathways for innovative drugs, medical devices, and technology. Geneoscopy is a start-up biotech company based in St. Louis, MO, and our first product is a stool-based colorectal cancer (CRC) screening technology that is currently under review by the Food and Drug Administration (FDA). Like many small biotech companies, we worry about the time we will have to wait between gaining FDA approval and generating revenue which depends on gaining coverage for our test by the Centers for Medicare and Medicaid Services (CMS) and private insurance companies. We believe there are unnecessary bureaucratic hurdles that innovative companies like ours encounter in our efforts to bring life-saving technology to patients.

About Geneoscopy

Geneoscopy was founded in 2015 with a vision to improve how gastrointestinal diseases are prevented, detected, and treated. Geneoscopy was started by Dr. Erica Barnell while she was earning her MD/PhD degrees at the Washington University School of Medicine in St. Louis, MO.

Dr. Barnell developed groundbreaking technology to isolate and analyze RNA biomarkers in human stool and this led to the development of Geneoscopy's initial product, a non-invasive CRC screening test that detects CRC and high risk pre-cancerous polyps called advanced adenomas (AA).[1]

The Promise of New Technology

As innovations in the field of preventive screening advance for the country's deadliest diseases, more effective screening modalities become available. For example, Geneoscopy's non-invasive, at-home CRC screening test using mRNA technology has demonstrated the potential to improve the detection of CRC and AA above and beyond existing tests on the market. Geneoscopy's CRC-PREVENT pivotal clinical study demonstrated 94% sensitivity for CRC and 45% sensitivity for AA, representing the highest sensitivity profile reported for any non-invasive CRC screening test in a prospective clinical study.[2] When it comes to screening, more options are better as it leads to greater compliance -- sorely needed to increase screening rates. Geneoscopy's clinical trial showed that their test worked successfully for people across demographic groups all over the country and has the real potential to advance the vital goal of increasing access to critically needed screening for historically underserved populations. In Geneoscopy's trial, 30% of participants had annual household income below $50,000 and 9% were on Medicaid.[3] Given the promise of Geneoscopy's test to reduce the incidence and mortality of CRC, FDA granted Geneoscopy's test its breakthrough device designation.

Colorectal Cancer is the Problem: Screening and Early Detection are the Solution

[1] https://pubmed.ncbi.nlm.nih.gov/11916153/
[2] https://www.prnewswire.com/news-releases/geneoscopys-non-invasive-colorectal-cancer- screening-test-demonstrates-high-sensitivity-and-specificity-in-large-pivotal-clinical-trial- 301717145.html
[3] https://doi.org/10.1158/1940-6207.CAPR-20-0294

CRC is the third most diagnosed cancer and the second leading cause of cancer death in our country.[4] This year alone, the American Cancer Society estimates there will be 153,020 new cases and about 52,550 deaths nationwide.[5] Everyone is at some risk for developing CRC, however, some groups are at an elevated risk. Of particular concern, African Americans have the highest CRC incidence and mortality rates of all racial groups in the U.S. African Americans are approximately 20% more likely to develop CRC and an estimated 40% more likely to die from it than most other populations.[6]

CRC is also the most preventable cancer if people get screened for it regularly. CRC almost always develops from precancerous polyps in the colon or rectum. If these pre-cancerous polyps are detected and removed through CRC screening, CRC can be prevented before it develops. Moreover, every 1% increase in adenoma detection leads to a 3% decrease in CRC incidence and a 5% decrease in CRC mortality risk.[7] Screening can also identify early-stage cancer. When found at an early stage before it has spread, CRC is more treatable, and the five-year relative survival rate is about 90%. The percentage of individuals diagnosed with advanced-stage CRC has increased from 52% in the mid-2000s to 60% in 2019.[8] Survival rates are lower when cancer has spread outside the colon or rectum.[9]

Unfortunately, many patients avoid screening, and their cancer is diagnosed at later stages. Approximately 40% of patients fail to get screened in part because they do not want to have a colonoscopy, which is the gold standard for CRC screening in the U.S. A colonoscopy is frequently met with patient aversion due to its required bowel preparation, sedation, and

[4] https://www.cdc.gov/cancer/colorectal/statistics/
[5] https://acsjournals.onlinelibrary.wiley.com/doi/full/10.3322/caac.21772

[6] https://acsjournals.onlinelibrary.wiley.com/doi/full/10.3322/caac.21772
[7] https://jamanetwork.com/journals/jama/fullarticle/2792977
[8] https://acsjournals.onlinelibrary.wiley.com/doi/full/10.3322/caac.21772
[9] https://www.cancer.org/cancer/colon-rectal-cancer/detection-diagnosis-staging/detection.html

potential time away from work.[10] Non-invasive screening tests that can be used at home, such as Geneoscopy's test, serve as important alternatives to colonoscopy for average-risk patients.

Access to Screening for Patients

A key hurdle to bringing life-saving screening tests to patients is CMS coverage and reimbursement. Additionally, many commercial insurance providers refuse to cover a test until after CMS has done so. Start-up companies like Geneoscopy take risks when developing new technologies and face the "valley of death" when coverage fails to come quickly after FDA approval. Unfortunately, many innovative companies such as Geneoscopy fail to survive the valley of death because of undue delays in coverage.

To keep pace with biotech innovation, CMS should provide a new predictable pathway for coverage of innovative technology. We are pleased that CMS published the Transitional Coverage for Emerging Technologies (TCET) proposed rule to provide a coverage pathway for FDA-approved breakthrough designated products. We are concerned however, that the TCET as currently drafted does not apply to laboratory diagnostic tests or products within a Medicare benefit category that is already subject to a national coverage determination (NCD). TCET should be modified to apply to laboratory diagnostic tests, even those that are subject to an existing NCD.

Conclusion

New technology and screening tools like Geneoscopy's CRC screening test hold the exciting promise of improving CRC screening rates, enabling early-stage detection of CRC and AA, and, in turn, reducing morbidity and mortality associated with CRC. Once the FDA approves a breakthrough designated test, we believe CMS should take steps to ensure it is

[10] https://www.sciencedirect.com/science/article/pii/S2211335519300750

covered immediately upon FDA approval. Patients cannot wait to get access to the latest advances in cancer screening; delays by the agency can make the difference between life and death.

We appreciate your consideration of our testimony, and we stand ready to be a resource to you and the committee. Thank you.

For questions or comments please contact:
Vince Wong, Chief Commercial Officer
(317) 225-3754

AiARTHRITIS
INTERNATIONAL FOUNDATION FOR AUTOIMMUNE & AUTOINFLAMMATORY ARTHRITIS

July 18th, 2023

Chair Cathy McMorris Rodgers
Ranking Member Frank Pallone, Jr.
Subcommittee on Health Chair Brett Guthrie
Ranking Member Anna Eshoo
House Energy and Commerce Committee
2125 Rayburn House Office Building
Washington, D.C. 20515

RE: Written Statement for the Hearing Record - Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology

Dear Chair Rodgers, Ranking Member Pollone, Chair Guthrie, Ranking Member Eshoo, and distinguished members of the House Energy and Commerce Committee,

The International Foundation for Autoimmune & Autoinflammatory Arthritis (AiArthritis) appreciates the opportunity to submit comments on the potential impacts of government policies on patient access to innovative therapies for those living with progressive, heterogeneous diseases. AiArthritis is the only nonprofit in the world that focuses on autoimmune and autoinflammatory diseases that include inflammatory arthritis as a major component (examples include Psoriatic Arthritis, Rheumatoid Arthritis, Lupus, and Crohn's Disease). We are leading efforts in Education, Advocacy, and Research through peer-led guidance, collaboration, and resources driven by patient-identified issues and patient-infused solutions.

As we are led by patients, we understand the importance of ensuring better health outcomes and lower costs for seniors. We also realize the challenges patients experience due to policies and procedures that focus on "patient population" levels versus the needs of more realistic disease "subgroups". AiArthritis diseases *are comprised of subgroups* that are based on several factors, including disease severity (mild, moderate, severe), levels of damage, numbers of comorbidities (present in over 70% of patients), and varied disease classifications (i.e. early and late-onset Rheumatoid Arthritis, Difficult to Treat*/D2T - or patients who have failed at least two mechanisms of action biologic treatments)*.[1] For this reason, population-level decisions and policies made by life science companies, insurers, and the Centers for Medicare & Medicaid Services (CMS) that look at evidence in the same general way (i.e., all people diagnosed with Rheumatoid Arthritis must be the same) ultimately exclude a significant percentage of beneficiaries. This current pathway may appear to save the healthcare system money, but realistically, it elevates costs for patients, their families, and the economy long term.

AiARTHRITIS
INTERNATIONAL FOUNDATION FOR AUTOIMMUNE & AUTOINFLAMMATORY ARTHRITIS

We applaud the House Energy and Commerce Committee and Subcommittee for recognizing the importance for America's seniors to access the best proven treatments and ensure doctors are positioned to provide the most effective care based on their patient's individual needs. We agree Medicare needs to consider the timeliness of coverage and develop a more reliable pathway to treatments based on subgroup data, post-market research, and what doctors feel is the right treatment based on this information.

Thankfully, innovative efforts in pharmaceuticals, diagnostics, and therapy successes are paving the way for improved quality of life and increased opportunities for remission. **For those living with progressive diseases, timely and accurate diagnoses and getting on the right treatment path is critical.** ***Neither can happen unless there is coverage for and access to innovative diagnostic tools and treatments when patients need them.*** **As the following statement is read, we hope Congress realizes the importance to view policies around drug price negotiations, breakthrough devices, and drug access, particularly as this viewpoint is provided through the lens of the people most impacted by their decisions.**

Potential Roadblocks Associated with Medicare's Drug Price Negotiation Program

With the passing of the Inflation Reduction Act in 2022, CMS will have the authority to negotiate prices for certain drugs. Given there are few specifics regarding how CMS should do this, we are hopeful they will consider our recommendations to 1) put the welfare of patients in the forefront, including their input on processes 2) consider incentives for innovation 3) caution use of QALYs 4) consider Precision Medicine over trial-and-error approaches.[2]

Disease prevalence, window of opportunity, and consequences of delays. Every 1 in 10 people are living with an AiArthritis disease. According to the American College of Rheumatology (ACR), the recommended time to treat to increase odds for remission is six months from onset.[1] However, the average time to diagnosis is 1 to 3 years in conditions like Rheumatoid Arthritis, and 5 or more years in many others.[3] The average age of onset in adults is 20 to 40, and any age in children. Recently research has demonstrated that remission is obtainable, but only if a person is treated very early after onset (even "pre-onset") or if they present with mild disease (which is rare).[4] *An estimated 90% of those with AiArthritis diseases and on Medicare have missed the window of opportunity, thus likely among the 70% affected by comorbidities and part of the subgroups who have exhausted most therapeutic options*.

Comorbidities in these diseases are caused by levels of uncontrolled inflammation (not on the right treatment to contain it) that redirect into the immune system and form dual plus diagnoses. These can range from developing multiple autoimmune disease diagnoses to potentially life threatening, inflammatory conditions. Failure to access early intervention leads to progressive

disease. For example, at the time of ***onset***, all people with AiArthritis diseases are twice as likely to develop heart disease and, as they continue to age, are four times more likely to develop Alzheimer's or dementia.[5] In the Medicare population, it is highly likely these patients also fall into another "subgroup" of those who have varied levels of permanent, irreversible damage. However, they are lumped into treatment matching with others based solely on a shared diagnosis ("general patient population"), without any consideration of the complexity associated with their therapeutic needs. ***This must stop***.

Current treatments and the need for Precision Medicine. While there are several therapies on the market for many of our conditions, only 30 to 40 percent of patients with the same diagnosis will experience high therapeutic efficacy. **This is due, in part, to the heterogeneity of our diseases, which cannot be properly represented in traditional clinical trial models.** So when the drug gets to market, 60 to 70 percent will not have an ideal response. This should be of high concern given we know most people are excluded from trials if they have comorbidities, which is representative of a large percentage of Medicare beneficiaries.

As previously mentioned, people with AiArthritis diseases have approximately 6 months from the time of onset to start effective treatment in order to have the greatest opportunity to avoid developing lifelong disease activity and associated chronic inflammation. The current treatment protocol involves a trial-and-error approach, with most therapies requiring a minimum of 3 months to determine full efficacy. If a clear, positive response has not occurred, the decision to continue forward or change course becomes challenging, as several barriers can interfere with continuity of care (i.e. access delays, step approaches). Often the doctor simply pulls the next treatment on the formulary and hopes this will be 'the one'. Thankfully, with the rise of precision medicine (personalized therapy based largely on scientific molecular marker matching), it is becoming possible to identify which subgroups may or may not respond to treatments. Access to these technologies must be available and timely to provide the most value to patients, the healthcare system, and society.

Transforming Medicare Coverage: A New Medicare Coverage Pathway for Emerging Technologies and Revamped Evidence Development Framework

In a June 2023 publication, CMS stated, "We are committed to fostering innovation while ensuring that people with Medicare have faster and more consistent access to emerging technologies that will improve health outcomes. As part of this commitment, CMS announced a proposed Transitional Coverage for Emerging Technologies (TCET) pathway and proposed approaches to coverage reviews and evidence development.." [6]

CMS further stated, "The goal of the TCET pathway is to develop reliable evidence for patients and their physicians to make health care decisions and ensure that people with Medicare receive high-quality care." We are excited about the possibility that Medicare beneficiaries may gain access to breakthrough technologies sooner due to review considerations for fit-for-purpose and breakthrough devices. We are equally enthusiastic that CMS developed these proposed processes based on feedback from various stakeholders, including patient groups.

Opportunities and concerns regarding the TCET Pathway. It is an exciting time in rheumatology research, particularly in regards to precision medicine efforts lending credibility to subpopulation patterns that can expedite diagnosis and match patients to the right treatments for their needs (thus potentially eliminating the missed window of opportunity, the costly trial-and-error process, and progressive disease and associated comorbidities).

One example of a new device involves testing a collection of blood samples and biomarkers to determine if a patient with Rheumatoid Arthritis is unlikely to respond to tumor necrosis factor inhibitor (TNFi) therapies. ***Knowing this information is game changing given 90% of RA patients are prescribed TNFi therapy as their first biologic intervention***. However, a majority of patients fail to achieve clinically meaningful change on TNFi's (*for reasons explained above under Current treatments and the need for Precision Medicine*).[7] Access to such molecular signature tests early in the disease therapy continuum could provide the data necessary to avoid therapies likely to fail and, in turn, keep the window of opportunity open for these patients to achieve remission. This test is currently available but access is limited, in part, because coverage often requires significant upfront evidence. But, efforts are underway - including those by AiArthritis - to conduct more research with groups who use these types of tests.

Thankfully, this pathway will address a current barrier regarding lack of upfront evidence required to meet the current coverage standard. However, we are concerned that the current guidance does not fully consider true meaningful impact for 'subgroups' and their need to access options that include precision medicine solutions. We question how subgroup determinants (like disability and D2T patients) will be considered, but feel hopeful groups like AiArthritis can work to facilitate opportunities to ensure their voices are counted in any deliberations.

Health Technology Assessments (HTA's) and Assessing "Value" for Subgroups

When considering complex and progressive conditions like AiArthritis diseases, methods that value price and necessity based on population level criteria (and often incomplete information) are problematic. To truly determine a drug's maximum fair price, CMS will need to consider the clinical and societal benefits of disease "subgroups," while ensuring to incorporate what Medicare beneficiaries (with progressed disease, existing damage, and comorbidities) value.

Value Assessment Frameworks (VAFs), like those conducted by Institute for Clinical and Economic Review (ICER), base their conclusions largely on safety and effectiveness at the population level. In their 2023 VAF Proposed Changes Publication, they incorporated more consideration to heterogeneity, subgroups, and disease burden, but we are unclear how ICER plans to effectively utilize this in conjunction with methods like the QALY and evLYG.[8] ***No matter how assessments are handled, one fact remains - without the right population representation (including a patient sample most representative of those on Medicare) - the data produced will not accurately translate to real world adaptation.***

Thankfully, ICER has recognized the need to bring more patient voices 'to the table' and, as such, has established a new Patient Council. As a person living with Axial Spondyloarthritis, I am proud to have been selected as the Council Patient Co-Chair, alongside five other patients with varied degrees of HTA knowledge.[9] **I encourage Congress and CMS to invite patients, like me - who fully understand value assessments - to more conversations about incorporating them into any decision making processes.**

Additional Recommendations for Increased Patient Input

At AiArthritis, we don't represent the patient voice, we *are* the patient voice. We are very grateful that Congress has taken this time to read the thoughts, perspectives, and concerns from the patient community. We encourage you, as well as CMS, to continue inviting groups that understand lived experiences and barriers that need to be eradicated to improve access and outcomes for those most representative of those they serve.

As a patient-led organization focused on improving the lives of those impacted by AiArthritis diseases, we urge Congress to consider these recommendations. Together we can achieve the crucial goal of ensuring better outcomes and reducing costs for patients. Thank you again for this opportunity and for considering what patients feel is most important.

Sincerely,

Tiffany Westrich-Robertson
Chief Executive Office & Person living with Axial Spondylitis

References

1. Nagy G, Roodenrijs NM, Welsing PM, et al. EULAR definition of difficult-to-treat rheumatoid arthritis. Annals of the Rheumatic Diseases 2021;80:31-35.
2. AiArthritis CMS Comments https://drive.google.com/file/d/1WQJKO3MVdXTbyCvpXHv3PZniaP3B0srX/view?usp=sharing
3. Lapane, K.L., Khan, S., Shridharmurthy, D. et al. Primary care physician perspectives on barriers to diagnosing axial Spondyloarthritis: a qualitative study. BMC Fam Pract 21, 204 (2020). https://doi.org/10.1186/s12875-020-01274-y
4. Demoruelle MK, Deane KD. Treatment strategies in early rheumatoid arthritis and prevention of rheumatoid arthritis. Curr Rheumatol Rep. 2012 Oct;14(5):472-80. doi: 10.1007/s11926-012-0275-1. PMID: 22773387; PMCID: PMC3616381.
5. Sangha PS, Thakur M, Akhtar Z, Ramani S, Gyamfi RS. The Link Between Rheumatoid Arthritis and Dementia: A Review. Cureus. 2020 Apr 27;12(4):e7855. doi: 10.7759/cureus.7855. PMID: 32489719; PMCID: PMC7255531.
6. Lee Fleisher, MD; Steve Farmer, MD, PhD, et al. Transforming Medicare Coverage: A New Medicare Coverage Pathway for Emerging Technologies and Revamped Evidence Development Framework. Centers for Medicare & Medicaid Services, June 22, 2023.
7. PRISM RA - https://www.sciphermedicine.com/scipher-medicine-announces-publication-of-clinical-study-validating-prismra-a-blood-test-predicting-non-response-to-tnf-inhibitor-therapies-in-patients-with-rheumatoid-arthritis/
8. ICERs Proposed Changed to the Value Assessment Framework - Public Comments https://icer.org/wp-content/uploads/2023/06/Proposed-VAF-Changes-For-Public-Comment_For-Publication_06052023.pdf
9. ICER Patient Council https://icer.org/patients/patient-council/

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

July 17, 2023

RE: LuMind IDSC Foundation 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:

LuMind IDSC is a leading national non-profit organization accelerating Down syndrome research to increase availability of therapeutic, diagnostic, and medical care options and empowering families through education, connections, and support. LuMind IDSC is closely connected to 300,000 families in our online community. 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.*" 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.

Adults with Down Syndrome depend heavily on Centers for Medicare and Medicaid Services' (CMS) to cover their health care and other needs. The life expectancy of individuals with Down syndrome has increased dramatically in the past few decades. As individuals with Down syndrome age, they have a 90% lifetime risk of developing Alzheimer's disease (AD), with first symptoms of dementia often showing in their late 40s and early 50s. Overexpression of the Alzheimer's disease associated genes for the amyloid precursor protein (APP) on the 21st chromosome, of which individuals with Down syndrome have three copies, is the likely genetic cause for Down syndrome associated Alzheimer's disease. Medicare and Medicaid are essential to individuals with Down syndrome and their caregivers, who rely on coverage to have access to the best drugs, medical care and supports.

The last two years were filled with hope and excitement for people with Alzheimer's disease as two drugs, Aduhelm™ and Leqembi™, received accelerated FDA approval. On July 7th, 2023, the traditional FDA approval of Leqembi™ marks a major milestone as the approval also comes with Medicare providing coverage for patients enrolling in a registry. Despite these great advancements, the Down syndrome community continues to face barriers to accessing these innovative drugs:

- In the 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. This discriminatory exclusion could have had a profoundly detrimental effect on the Down syndrome community. Thankfully, after a large national advocacy effort by the United Coalition for Down Syndrome, a coalition of 6 national DS organizations, CMS in its final National Coverage Decision removed the discriminatory language.

- Following FDA approval of a drug, state prior authorization criteria are issued gradually in each state. They are however not nationally coordinated by CMS. In addition, the issued criteria inadvertently exclude people with Down syndrome from access and require adaptation.
- CMS' CED Registry that was published on July 7th require more adaptations for access to people with Down syndrome such as age, assessment scales and other factors.
- No one with Down syndrome was included in the clinical trials for the three most promising drugs in this class (Aduhelm™, Leqembi™ and donanemab), compared to more than 17,000 participants with Alzheimer's in the general population. Therefore, critical safety and efficacy data in adults with Down syndrome does not exist and many clinicians advise against the prescription of the drug until safety can be assured.

Innovative new drugs and treatments against Alzheimer's disease are still not accessible to the Down syndrome community that needs it most. Several of those access questions for the Down syndrome community relate to CMS coverage processes. The Down Syndrome Community aligns with other groups recommending that the CED requirement in the National Coverage Decision (NCD) for anti-amyloid antibodies from April 2022 be removed at the earliest possibility and that CMS provides traditional Medicare coverage to these life-changing treatments for Alzheimer's patients.
In our view, the negative effects of the CED requirement include: 1) limiting access to patients that would otherwise be treated with the latest life-saving treatments; 2) inadvertently exacerbating inequities for minority populations; 3) registry compromises for clinical expediency runs the risk of inadequate data depth to support relevant analysis and decision-making.

Lastly, we hope also that the Subcommittee brings 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.

LuMind IDSC Foundation envisions a world where people with Down syndrome have healthy and independent lives and have equal access to treatments, clinical trials and research. We thank 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,

Hampus Hillerstrom

President & CEO, LuMind IDSC Foundation

LuMind IDSC Foundation, 20 Mall Road, Suite 200, Burlington, MA 01803
Phone: 781-825-1300 ; LuMindIDSC@LuMindIDSC.org