AMERICAN ACADEMY OF
NEUROLOGY®

201 Chicago Avenue
Minneapolis, Minnesota 55415

Tel: (612) 928-6100
Fax: (612) 454-2744

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The Honorable Anna Eshoo
Chair
Subcommittee on Health
House Energy and Commerce Committee
Washington, DC 20515

The Honorable Brett Guthrie
Ranking Member
Subcommittee on Health
House Energy and Commerce Committee
Washington, DC 20515

Statement for the Record on "The Path Forward: Advancing Treatments and Cures for Neurodegenerative Diseases"

Dear Chair Eshoo and Ranking Member Guthrie,

The American Academy of Neurology (AAN), the world's largest association of neurologists representing 36,000 professionals, is strongly committed to improving the care and outcomes of persons with neurologic illness in a cost-effective manner. One in six people lives with a brain or nervous system condition, including Alzheimer's disease, Parkinson's disease, stroke, epilepsy, traumatic brain injury, ALS, multiple sclerosis, and headache.

The AAN thanks the Energy and Commerce Committee for hosting the upcoming hearing focused on advancing treatments and cures for neurodegenerative diseases. Included below are several policies the AAN supports, which we believe are important for advancing treatments for neurodegenerative diseases or access to those therapies once approved. We hope the Committee considers these policies in any future legislative hearing on this topic. Please let us know if we can be helpful in any other ways on these or any other ideas.

Neuroscience Center of Excellence

Brain disorders such as Alzheimer's disease, Parkinson's disease, autism, epilepsy, depression, addiction, and traumatic brain injury, are projected to be some of the most disabling and costly chronic diseases in the 21st century. One in three Americans will have a brain or nervous system disorder sometime in their life and the cost of treating neurologic disorders is more than $600 billion each year. Additionally, the Centers for Disease Control and Prevention (CDC) forecasts that the number of Americans with some form of dementia will double by 2060 to

13.9 million people (3.3 percent of the estimated 2060 U.S. population).[1] Some estimate the 2021 cost at $355 billion (approximately $239 billion of that being Medicare/Medicaid) and the 2050 cost at $1.1 trillion.[2] Such disease not only increases costs for private sector payers and government programs, but also places a heavy financial and social toll on millions of individuals, families, and caregivers.

These statistics are grave, but they pale in comparison to the real-life impact these diseases have on millions of Americans each day. Regrettably, we do not currently know enough about the brain to begin to meet this dire health and economic challenge. Supporting critical neurologic research is important for developing treatments, synthesizing data, identifying underlying causes, and overall, better understanding neurologic diseases that impact so many.

To help address the development challenges in the space, the AAN supports the creation of a NCOE at the Food and Drug Administration (FDA) with the mission of accelerating development, review, and approval of new medical products and achieving patient-centered regulatory decision-making through collaboration, engagement, and transparency. Successes of the Oncology Center of Excellence (OCE) inform the need for a NCOE and, importantly, the recently-released 21st Century Cures 2.0 discussion draft supports its creation.

Given the success of the Oncology Center of Excellence, Congress should continue the model created by 21st Century Cures and establish a NCOE at FDA to tackle the significant unmet need faced by those living with neurologic and psychiatric diseases. Despite the large societal need, medical products for neurologic and psychiatric diseases and disorders are approved by the FDA at a much lower rate than products for other disease areas. Additionally, in recent years, FDA reviewers denied more requests for (and granted fewer) breakthrough therapy designations among neuroscience New Drug Applications (NDAs) than they did for NDAs in other disease areas.

Establishing the NCOE as outlined in the Cures 2.0 draft would accelerate development by:

- Placing a stronger emphasis on drug and device development tools for treatment and cures for psychiatric and neurologic diseases;
- Increasing utilization of patient-focused drug and device development for people living with psychiatric and neurologic diseases; and,
- Improving engagement between FDA and stakeholders and strengthening internal coordination within FDA.

The NCOE should leverage regulatory scientists and reviewers with expertise in drugs, biologics, devices, and diagnostics to expedite development of drugs and devices for neurologic and psychiatric diseases. Further, this NCOE would help address the needs of persons living with serious neurologic complications resulting from contracting SARS-CoV-2, which may affect individuals' ability to function or work after the pandemic ends.

Medical Research

Ensuring strong funding of medical research is critical to developing new treatments for neurodegenerative diseases. The AAN supports the authorization of an Advanced Research Projects Administration – Health (ARPA-H) that has been proposed by President Biden to pursue

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[1] https://www.brainfacts.org/diseases-and-disorders/mental-health/2014/global-burden-of-neurological-and-mental-disorders

[2] https://www.alz.org/media/documents/alzheimers-facts-and-figures.pdf

transformational breakthroughs in medicine. As highlighted by the administration, ARPA-H would have the potential to fund high-risk, high-reward research, such as new approaches to accelerate discovery of brain imaging and blood biomarkers. There are currently no cures for any neurologic disease, meaning APRA-H could help contribute to the rapidly growing need to better understand the brain and nervous system.

Whether or not this new entity is housed within the National Institutes of Health (NIH), appropriate oversight and transparency will be of the utmost importance. While the AAN is in favor of innovative research with less bureaucratic barriers, scientific rigor and patient centered outcomes must be maintained. As recent experience has shown with the development of the COVID-19 vaccine, appropriate promotion and communication of government-funded science is essential.

In addition to using DARPA as a model for ARPA-H, the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative could also be a useful example in promoting collaboration and transformational discoveries. The BRAIN Initiative is a unique public-private partnership that involves the NIH, DARPA, the National Science Foundation (NSF), the FDA, the Intelligence Advanced Research Projects Activity (IARPA), and private organizations. One program funded by IARPA, the Machine Intelligence form Cortical Networks (MICrONS) uses a nimble, contract-driven approach to map the function and connectivity of cortical circuits to advance use of machine learning algorithms. IARPA collaborates with other BRAIN Initiative partners to share brain imaging data that can help inform additional neuroscience research. Many of these BRAIN Initiative projects focus on technologies and data analytics, that could serve as a model for ARPA-H efforts. Given the existing success of the BRAIN Initiative and other innovative entities including NCATS and BARDA, ARPA-H should also have a clear scope of work to ensure synergy without duplication.

Also as noted by many, the creation of ARPA-H should not interfere with consistent funding for the NIH. Basic science efforts to better understand the molecular and cellular mechanisms underlying neurologic disease is critical to discovering better treatments and potential cures.

The AAN also supports the bipartisan, bicameral RISE Act, which we believe would provide crucial funding to support NIH-funded research—to continue federally funded research that has been stalled, delayed, or even stopped as a result of the pandemic. The long-term consequences of the COVID-19 pandemic on the country's biomedical research enterprise are becoming clear: funds are being diverted to support COVID-related research to the detriment of research on conditions within the missions of NIH's 27 institutes and centers; the new COVID-related expenses incurred to run research are reducing the buying power of existing grants; and we are in danger of losing a generation of investigators who are particularly vulnerable to career disruptions. The bill also authorizes funding for research grants from multiple agencies that fund scientific researchers to institutions, covering the costs of research disruptions related to the COVID-19 pandemic.

Gene Therapies

With many gene therapies on the horizon that have the potential to treat neurodegenerative diseases, the AAN supports requiring the HHS Secretary to submit a report to Congress regarding the current state of cell and gene therapy regulation and foreseeable regulatory challenges for the FDA. This report has been proposed in the 21st Century Cures 2.0 discussion draft by Reps. Diana DeGette and Fred Upton.

The first commercial gene therapy targeted to treat a neurologic disease, onasemnogene abeparvovec-xioi (Zolgensma), has revolutionized treatment options for children with spinal muscular atrophy, a previously untreatable and fatal neurologic disorder of childhood. However, insurance coverage of this life changing therapeutic remains a challenge due to its $2.1 million price tag. The report will also provide an opportunity to better evaluate current initiatives, including the National Institute of Neurological Disorders and Stroke's Ultra-rare Gene-based Therapy (URGenT) network. This new program will support the development of gene-based therapies for ultra-rare neurologic diseases.

Barriers to Care

With neurodegenerative diseases at the forefront of the upcoming hearing, it is important that the AAN voice its support for reducing barriers to timely access to therapies that do exist, such as prior authorization and step therapy. The AAN has strongly long prioritized improving patients' timely access to care, which helps improve patient outcomes while having the additional benefit of allowing physicians to spend more time treating patients and to reduce burnout.

The AAN supports the Improving Seniors' Timely Access to Care Act (H.R. 3173), one of the most broadly supported bipartisan pieces of health care legislation in the 117th Congress. This legislation would help protect patients from unnecessary delays in care by streamlining and standardizing prior authorization under the Medicare Advantage program, providing much-needed oversight and transparency of health insurance for America's seniors. The bill already has over 150 cosponsors and is supported by more than 280 organizations representing patients, providers, IT groups, and companies across the country.

The AAN also supports the Safe Step Act (H.R. 2163), a bill to institute sensible guidelines for using step therapy or "fail first" requirements in federally governed ERISA health insurance plans. Provisions include:

- Ensure that employer-sponsored prescription drug plans under ERISA have a clear process for beneficiaries and healthcare providers to request exceptions to medically inappropriate step protocols;
- Specify circumstances in which such exceptions should be granted; and
- Require responses to exception requests by payers within 24-72 hours.

The Safe Step Act complements laws enacted by more than half of all states to provide similar protections against inappropriate use of step therapy protocols within the individual marketplace and state-sponsored plans.

Diversity in Clinical Trials

The AAN is committed to intentional action to be a fully inclusive, deliberately diverse, and anti-racist organization that respects and values our membership, our staff, and the communities we serve. To that end, we work to actively promote equity and social justice in neurology and neurosciences. It is for this reason that the AAN supports H.R. 3085, Equity in Neuroscience and Alzheimer's Clinical Trials (ENACT) Act.

Older Black and Latinx Americans are much more likely than White Americans to be affected by Alzheimer's and other dementias, yet many clinical research studies focused on these diseases do not include sufficient data from these populations to be representative of the US population. The underrepresentation of these populations, along with Native American and Asian Americans, hampers our understanding about these health disparities and limits our knowledge of how potential therapeutics may affects populations that need them the most. This legislation aims to improve health care equity by taking deliberate actions to foster the inclusion of diversity in clinical trials. The ENACT Act would increase participation of underrepresented populations in dementia clinical trials by expanding education and outreach to these populations, encouraging the diversity of clinical trial staff, and reducing participation burden.

This is critical to addressing the significant well-documented widespread barriers to patient access to care and diverging health care outcomes associated with gender, race, and ethnicity, including in dementia care, as outlined in the Alzheimer's Association's *Special Report: Race, Ethnicity and Alzheimer's in America*.

Conclusion

We appreciate the work Congress has done and will continue to do on behalf of Americans suffering from neurodegenerative diseases and other neurologic disorders. If you have any questions or require additional information, please do not hesitate to contact Derek Brandt, Director of Congressional Affairs at dbrandt@aan.com or Fred Essis, Congressional Affairs Manager at fessis@aan.com. We look forward to working with you on these important issues.

Sincerely,

Orly Avitzur MD

Orly Avitzur, MD, MBA, FAAN
President, American Academy of Neurology