AMERICAN ACADEMY OF NEUROLOGY®

American Academy of Neurology Response to the GOP Healthy Future Task Force Treatments Subcommittee Request for Information March 8, 2022

The Democrat majority intends to advance a bill that would set the price of certain drugs in Medicare. How could that bill impact future curative therapies? If there are negative projected effects, then what can Congress do to ameliorate them?

- The AAN believes action must be taken to lower the costs of prescription medications to the government, patients, and families who are all burdened by unsustainable prices for lifesaving medications. Many neurologic disorders require ongoing therapies that are increasingly expensive, which adds uncertainty and stress for the individuals that need them. The AAN supports the direct negotiation of the price of prescription drug prices in Medicare. In doing so, the AAN believes that the financial incentives that drive new innovations must be protected. We support the current version of price negotiation advanced by the House in the form of the Build Back Better Act which is limited in size and scope with minimal threat to the development of future therapies.

Republicans laid out a plan for drug pricing with H.R. 19 Lower Costs, More Cures Act of 2021. This includes over 30 bipartisan provisions to lower drug costs for seniors. Building off of this, what other policies should we consider to lower costs while maintaining access to lifesaving cures?

- The annual cost of treating neurologic disorders in the US is more than $600 billion, and prescription drugs for neurologic conditions account for a significant share of that expense. For example, the cost of MS therapies has dramatically risen since the first MS disease-modifying therapy (DMT) was approved in 1993. This trend has mostly continued unabated, even after the first generic was approved and launched in 2015. Today, the annual median price for brand MS DMTs is nearly $100,000, rising from a relatively modest $8,000/year in 1993. New innovative medications, such as those for Spinal Muscular Atrophy (SMA), have revolutionized treatment options for an often-fatal condition, but at a tremendous price tag. Most recently last June, the approval of Aduhelm and subsequent announcement that this medication would cost $56,000/year, which was subsequently reduced to $28,000, once again brought the high cost of neurologic medications into the spotlight. High-cost drugs for prevalent conditions like Alzheimer's Disease threaten the financial stability of programs like Medicare.
- The AAN supports proposals like those in H.R. 19 to promote transparency in prescription drug pricing. Broad disclosure of pricing information, including how drugs are priced, the prices paid by insurers, and the prices paid by consumers, would provide information critical to lowering costs for patients and the entire health care system. The costs of existing prescription drugs for many neurologic conditions continue to increase and these reporting requirements would

provide valuable information to patients and physicians as well as important documentation of price changes over time. The AAN also supports requiring public justifications of price increases of greater than 10 percent in one year and 25 percent over three years.

- The AAN supports a redesign of the Medicare Part D program to create an out-of-pocket spending maximum for the first time. While the AAN supports the out-of-pocket threshold of $3,100 in H.R. 19, we also support the more aggressive threshold of $2,000. Increasingly, patients are required to absorb more and more of the cost for their drugs. As a result, medication adherence, medication rationing, and treatment compliance issues are increasingly problematic for people living with neurologic diseases. Research shows that a $50 increase in out-of-pocket costs for prescriptions was associated with a 9-18% reduction in medication adherence for dementia, Parkinson's, and neuropathy patients. Neurologists work hard to provide high quality care for their patients, but the complexities of the prescription drug pricing system add undue stress and can make it difficult for patients to access necessary treatments.
- The AAN supports enacting policies that would prevent drug companies from unfairly increasing their prices beyond the rate of inflation. As drug prices increase, access to needed therapies is restricted. Patients have long faced excessive annual increases in drug prices that outpace inflation, putting medications that have been on the market for decades out of reach. This has resulted in unsafe practices such as drug rationing or only filling one of many prescriptions to save money. This change has the potential to help blunt the impacts of rising drug costs going forward.
- Insurer utilization management policies also limit patient access to treatment and should be addressed as well. This includes protections from step therapy protocols to help ensure that patients have timely access to the most medically appropriate treatment. Evidence on the practice of insurance-mandated step therapy increasingly demonstrates that without reasonable guardrails like those proposed in the Safe Step Act, step therapy can be costly and put patients at risk. Step therapy is commonly applied, and frequently misaligned with clinical guidelines. Researchers at Tufts Medical Center recently found that step therapy was applied to 38.9% of drug coverage decisions, and more than half (55.6%) of those decisions required more steps than the clinical guidelines for diseases like multiple sclerosis, psoriasis, psoriatic arthritis, or chronic migraines. In addition, emerging evidence demonstrates that barriers to access caused by utilization management, including step therapy, increases costs for the US health system and results in poorer health outcomes. A recent study found that utilization management was associated with $93.3 billion in annual costs to various stakeholders. Further, the inclusion of prior authorization, including step therapy, in formularies for inflammatory bowel disease treatments increased the likelihood of a patient requiring hospitalization, surgery, and/or emergency room visits by 12.9% within 180 days. We urge Congress to include the following step therapy protections for ERISA health plans in future legislation:
    - Establish a clear exemption process for patients and providers to request an exception to a step therapy protocol
    - Outline five exceptions to fail first protocols:
        - Patients already tried and failed on the required drug

        - Delayed treatment to the initially prescribed drug would lead to severe or irreversible consequences. The required drug is contraindicated or has caused/is likely to cause an adverse reaction
        - Required treatment will prevent a patient from working or fulfilling activities of daily living
        - Patient is stable on their current medication
        - Require a group health plan to respond to an exemption request within 72 hours in all circumstances, and 24 hours if the patient's life is at risk.
- These protections are clearly articulated in pending bipartisan and bicameral legislation, known as the Safe Step Act (H.R. 2163/S. 464), first introduced in 2017. Currently, over 30 states have passed patient-friendly step therapy protections. These state laws apply to state regulated insurance plans such as the individual marketplace. We are asking Congress to enact these protections for federally regulated ERISA health plans, ensuring all patients, regardless of who regulates their health plan, have access to the right treatment at the right time.

What barriers to innovation in the drug, device, or diagnostic space should Congress address?

- The AAN believes that establishing a Neurology Center of Excellence (NCOE) within the FDA would help alleviate barriers to the creation of innovation for neurological products, which have historically been one of the most challenging sectors for drug development. Overall, the AAN applauds the intent of two pieces of legislation which aim to accomplish this, H.R. 5435 Bringing Regulatory Advances into Neuroscience (BRAIN), and S. 3427, the Neuroscience Center of Excellence Act. We believe the creation of this center will enable several important goals, including 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 with psychiatric and neurologic diseases; improving engagement between the FDA and stakeholders; and strengthening internal coordination within the FDA. A NCOE will spur innovation and investment in the study, creation, and regulation of brain-focused therapies by creating an environment designed to achieve patient-centered regulatory decision making, with the goal of bringing the right treatments and cures to patients who desperately need them
- The AAN also supports a five-year reauthorization of the CDC's National Neurological Conditions Surveillance System (NNCSS) program, which was initially authorized for five years in 21st Century Cures Section 399S–1 (FY 2018-2022), but it wasn't funded until FY 2019. NNCSS is an integrated system that uses state-of-the-art data sources, tools, and analytic methods to track the epidemiology of neurological conditions. The aim of the system is to derive actionable and timely information to increase understanding of neurological conditions and catalyze research into causes, diagnosis, and treatment. The initial program timeline was disrupted significantly by COVID-19, which has diverted many of the CDC staff/resources integral to the success of this project to more pressing matters in the short term. Reauthorizing the program will allow it to finish its current work on MS and Parkinson's disease and then move on to additional neurological conditions, as originally planned. Legislation is currently being developed.

- Insurance-mandated step therapy as a general practice can impede innovation as it inhibits market opportunities for new drug development and new companies are at a disadvantage to compete within this opaque system. Products with specific indications are included in plan formularies. This could be harmful to patient outcomes as patients may not be able to access the medications beyond the plan formulary or may experience prohibitively higher costs in doing so. As ERISA plan guardrails are more specific and narrower than the current Medicare guidelines, Congress should consider more specifically clarifying Part B's insurance-mandated step therapy guardrails.

What can be learned from the pandemic to speed up development of novel vaccines and treatments?

- Given the magnitude of COVID cases across the US, the impact of neurologic symptoms is likely enormous, and without proper information sharing, patients could suffer devasting consequences and misdiagnoses. Additionally, understanding the core causes of post-acute sequelae of COVID-19 (PASC) that impacts an estimated 3.2 million Americans, will make it easier for providers to identify patients who are more at risk of developing chronic symptoms, and potentially prevent them from happening. Legislation like H.R. 2754, The COVID-19 Longhaulers Act, builds upon this information sharing principle and works to create a patient registry that includes individuals experiencing PASC. The registry will be an important tool in the collection/surveying of research, identification of symptoms and the establishment of treatment and medical procedures.
- Another lesson that can be learned is ensuring there is adequate investment in the health care professionals who are on the ground treating patients and rolling out new vaccines and therapies. According to data collected by the Association of American Medical Colleges (AAMC), the United States is facing a shortage of between 54,100 and 139,000 physicians by 2034 that will likely be exacerbated by rising rates of physician burnout and early retirement due to the COVID-19 pandemic. Now, more than ever, it is critical that we ensure our nation's health care workforce can meet the needs of the American people. Ensuring there is a strong health care workforce in place to combat and treat patients helps strengthen the response to any future variant or pandemic. The AAN strongly supports strengthening the health care workforce by utilizing the skills of immigrant physicians who completed their training in the United States to assist in the growing shortage. As such, the AAN supports The Conrad State 30 and Physician Access Reauthorization Act (S. 1810/ H.R. 3541), which would reauthorize the Conrad 30 program for an additional three years, as well as make several key improvements, including creating a process to gradually increase the number of waivers while requiring additional employment protections. Furthermore, The AAN supports the efforts of the Healthcare Workforce Resilience Act (S. 1024/ H.R. 2255), a bill that would reallocate 15,000 visas for foreign-born physicians and 25,000 visas for foreign-born nurses to practice in the United States. The Healthcare Workforce Resilience Act would provide much-needed stability to foreign-born physicians already practicing in the United States who are stymied by the green card backlog due to per country caps. Finally, Congress should enact the Resident Physician Shortage

Reduction Act of 2021 (S. 834/H.R. 2256), which would increase the number of Medicare-supported medical resident training positions by 14,000 over seven years.

Can we continue decentralizing clinical trials and allow more patients to get access to innovative treatment in their communities and homes through remote monitoring? What regulations and laws must be addressed to facilitate continued progress?

- The AAN has fervently advocated for improving the representation of diverse populations in clinical trials. 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.
- The AAN supports H.R. 3085, Equity in Neuroscience and Alzheimer's Clinical Trials (ENACT) Act 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.
- The AAN believes that leveraging digital technologies in remote clinical trials helps eliminate the need for travel time, lost wages and childcare/eldercare, which will significantly increase the pool of potential participants. It is also critical with respect to advancing health equity by accounting for such logistical and other participant-related factors that could limit participation. State regulators have a role in breaking down additional barriers in using digital technology. Most concerning is the inability of providers to practice across state lines. Licensing limitations effectively prohibit clinicians working on clinical trials from recruiting patients from outside the state where the clinician is licensed, thereby diminishing the impact of the federal changes aimed at decentralizing clinical trials. While this is ultimately a state issue, providing non-binding guidance from the FDA to states on how to bolster clinical trial modernization through licensure flexibilities would help catalyze change at the state level. We recommend advancing language requiring the FDA to set up an intergovernmental working group with state and federal regulators to develop such guidance. This group will likely identify other areas beyond licensing that may need to be addressed, such as mailing of non-approved medications.
- Finally, bringing clinical trials to those with health care inequities is critical, and the current federal rules mandating conduct of human research within 45 CFR 46 must remain, but any loosening of the rules that interfere with proper trial design and implementation must be avoided.

Is there anything else the Treatments Subcommittee should consider?

<u>Reducing Regulatory Burden</u>

- Prior authorization (PA) is a health plan cost-control process that requires physicians and other health care professionals to qualify for payment by obtaining approval before performing a

service. According to a recent survey from the American Medical Association (AMA), 93% of patients report care delays when prior authorization is involved. Furthermore, 82% of physicians report that prior authorization can lead to treatment abandonment. Most alarmingly, over one-third (34%) of surveyed physicians reported that PA has led to a serious adverse event (e.g., hospitalization, disability, or even death) for a patient in their care. Patients—especially the vulnerable Medicare Advantage (MA) population—deserve PA reforms that will protect them from these harms associated with PA requirements. The Improving Seniors' Timely Access to Care Act (H.R. 3173) addresses prior authorization and is one of the most broadly supported bipartisan pieces of health care legislation in the 117th Congress. Introduced by Reps. Suzan DelBene (D-CA), Mike Kelly (R-PA), Ami Bera, MD (D-CA) and Larry Bucshon (R-IN), 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. H.R. 3173 has 264 cosponsors and is supported by nearly 450 organizations representing patients, providers, IT groups, and companies across the country.

<u>Expanding Access with Telehealth</u>

- The COVID-19 pandemic has forced neurology practices around the country to dramatically reshape their delivery of care for the vulnerable populations they treat. Telehealth has become an essential method of delivering care for most neurologists, which has only been possible due to the policy flexibilities enacted by Congress, along with the broad interpretation of these provisions by the Centers for Medicare and Medicaid Services (CMS). Neurology is one of the top specialties benefitting from telehealth flexibilities – with evaluation and management (E/M) visits being highly adaptable to the virtual setting.
- Many of the telehealth flexibilities that have helped dramatically improve patient access to care are temporary and limited to the duration of the COVID-19 PHE – and impact both public health programs and private health coverage. While the Biden Administration may elect to extend the COVID-19 PHE, the fact that the PHE determination must be renewed every 90 days and could end later this year has introduced significant uncertainty into all parts of the U.S. health care system. As it stands today, providers must weigh the costs of investing in the technological and clinical infrastructure required to maintain telehealth programs at scale against the possibility that Congress may ultimately decide not to support permanently expanded telehealth coverage.
- To that end, we ask for your leadership in facilitating a pathway to comprehensive permanent telehealth reform that would provide certainty to beneficiaries and our nation's health care providers while providing sufficient time for Congress and the Administration to analyze the impact of telehealth on patient care. Specifically, we ask that Congress:
    - Authorize the continuation of all current telehealth waivers through December 31, 2024. Currently the HHS Secretary's waiver authority for many key telehealth expires immediately upon expiration of the PHE.

- Require HHS complete all feasible evaluations related to telehealth by fall 2023 and combine findings into a single overarching dashboard with recommendations to inform permanent telehealth legislation by Congress.
- Take up permanent, evidence-based telehealth legislation for implementation in 2024.

<u>Support Fundamental Medical Research:</u>

- Strong funding for innovative medical research and other programs to better understand diseases is essential for developing new groundbreaking treatments. The AAN supports numerous research programs that are essential, including:
- The National Institutes of Health (NIH) is the world's leading funder of basic biomedical research, providing vital discoveries that can help lead to treatments and cures for neurologic diseases. Decades of basic, translational, and clinical research are necessary to develop lifesaving therapies. The NIH is also vital in helping us understand the long-term effects of COVID-19. For example, the NIH's National Institute of Neurological Disorders and Stroke launched NeuroCOVID, a new database to collect information about COVID-19 related neurologic symptoms, complications, and outcomes as well as the virus' effects on existing neurologic conditions. We thank you for your leadership in ensuring NIH has had record levels of funding in recent years and urge you to continue that momentum going forward.
- The Brain Research Through Advancing Innovative Neurotechnologies® (BRAIN) Initiative is an NIH led multidisciplinary collaboration involving public and private partners is working to map circuits of the brain, measure electrical and chemical activity, and understand how their interplay creates unique cognitive and behavioral capabilities. Since its founding in 2013, the BRAIN Initiative has made substantial inroads toward creating and leveraging new tools and resources to decipher the inner workings of the brain, with the ultimate goal of curing human brain disorders. It is now rolling out three large "BRAIN 2.0 transformative projects" that will (1) build a comprehensive atlas of cell types in the human brain, (2) develop and scale up the tools necessary to complete a microconnectivity map of an entire mammalian brain, and (3) provide tools for precision access to the identified cell types to allow interrogation and modulation of neural circuits. The AAN believes this fundamental research is critical to finding effective treatments and cures to complicated neurologic conditions such as Alzheimer's Disease. We urge you to continue to strongly support this critical program.
- The AAN urges you to support medical research at the Department of Veterans Affairs, given the disproportionate impact of neurologic conditions on Veterans such as traumatic brain injuries. Increased funding for VA research would allow for new efforts to address COVID-19, access to clinical trials, and more while renewing support for groundbreaking programs like the Million Veteran Program and research on neurologic disease.