Neuroscience Working Table

Working Together to Achieve a Neuroscience Center of Excellence

July 29, 2021

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

The Honorable Brett Guthrie
Ranking Member
Subcommittee on Health
House Energy and Commerce Committee
United States House of Representatives
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 Neuroscience Working Table ("Working Table"), which includes organizations and coalitions representing more than 50 million Americans with psychiatric and neurologic diseases, writes to commend you for holding this important hearing on the challenges and opportunities in neurological research as you consider ways federal policy can help respond to the needs of individuals with neurological diseases and conditions. In the following statement, we detail the significant societal impact of these diseases and disorders, describe the challenges of developing medical products in this space, and explain why establishing a Neuroscience Center of Excellence (NCOE) within the Food and Drug Administration (FDA) would help effectuate positive transformational changes to address many of these challenges.

The Significant Societal Impact of Diseases and Disorders

The adverse impact of psychiatric and neurological diseases and disorders on individuals and families across our country and the world is very significant. The data show that the adverse impact continues to increase and will continue to do so unless Congress takes action.

- As of 2020, nearly 100 million Americans live with psychiatric and neurological diseases, costing $760 billion annually.[1]
- 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). Some estimate the 2021 cost at $355 billion (approximately $239 billion of that being Medicare/Medicaid) and the 2050 cost

[1] Gooch CL, Pracht E, Borenstein AR. The burden of neurological disease in the United States: A summary report and call to action. *Ann Neurol*. 2017;81(4):479-484. doi: 10.1002/ana.24897

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 that has broader negative ramifications for our nation's economy.

- Across all ages, mental disorders are among the leading causes of ill health and disability worldwide. The World Economic Forum estimates that by 2030, the global cost of mental illness will rise to $16 trillion[3], more than double the total cost of cancer, diabetes, and cardiovascular diseases combined – and that is a pre-pandemic estimate.

Development Challenges Continue in Neuroscience

Treatments for brain and CNS disorders face more significant hurdles in the development and approval phases compared to other therapeutic areas. Challenges include:

- The complex pathologies of the diseases;
- Heterogeneity of symptoms;
- Difficulties in identifying and developing biomarkers and clinical endpoints; and
- Greater length of time for clinical trials and regulatory processes, as drugs in the neuroscience area take 20 percent longer to develop and approve than those for other therapeutic areas.[4]

Despite the large societal need, medical products for neurological and psychiatric diseases and disorders are approved by the FDA at a much lower rate than products for other disease areas.

- According to a 2018 study by the Tufts Center for the Study of Drug Development, central nervous system drugs take 20 percent longer to develop and approve than non-central nervous system drugs.[5]
- The Government Accountability Office reports that, 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.[6]

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[2] Alzheimer's Association. 2021 Alzheimer's Disease Facts and Figures
[3] https://www.psychiatrictimes.com/view/mental-illness-will-cost-world-16-usd-trillion-2030
[4] https://www.globenewswire.com/news-release/2018/09/11/1569156/0/en/CNS-Drugs-Take-20-Longer-to-Develop-and-38-Longer-to-Approve-vs-Non-CNS-Drugs-According-to-the-Tufts-Center-for-the-Study-of-Drug-Development.html
[5] https://www.globenewswire.com/news-release/2018/09/11/1569156/0/en/CNS-Drugs-Take-20-Longer-to-Develop-and-38-Longer-to-Approve-vs-Non-CNS-Drugs-According-to-the-Tufts-Center-for-the-Study-of-Drug-Development.html
[6] https://www.gao.gov/assets/gao-20-244.pdf

To Help Address the Development Challenges, Establish a Neuroscience Center of Excellence at FDA

To help address the development challenges in the space, the Working Table supports the creation of an NCOE at the FDA with the mission of accelerating the development, review, and approval of new medical products and achieving patient-centered regulatory decision-making through collaboration, engagement, and transparency. The successes of the Oncology Center of Excellence (OCE) inform the need for an NCOE and, importantly, the recently released Cures 2.0 discussion draft supports its creation.

Successes of an FDA Center of Excellence

Under the 21st Century Cures Act, FDA created the OCE. While recognizing the differences between oncology and neuroscience, an NCOE could be built upon the successful model of the OCE, which has effectively coordinated FDA activities on the review of oncology products. The Center's internal collaboration and external engagement have been very positive, and the OCE has played an important role. Last year, during the height of the COVID-19 pandemic, the FDA (with OCE staff):

- Implemented an open and transparent stakeholder engagement process, including engaging those living with cancer;
- Issued multiple guidance documents related to conducting clinical trials during the COVID-19 pandemic; and
- Held more than 10 listening sessions with patient advocacy groups with the Office of Oncologic Diseases.
- Between January 1 and November 1, 2020, oncology review teams were involved in the approval of 15 new molecular entities and more than 80 efficacy supplements for the treatment of patients with cancer,[7] as well as involved in the review of seven premarket approval (PMA) devices.[8]

The Need for a Neuroscience Center of Excellence

Given these successes, Congress should continue the model created by the 21st Century Cures Act and establish an NCOE at FDA to tackle the significant unmet need faced by those living with psychiatric and neurologic diseases. Despite the large societal need, medical products for neurological 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.

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[7] https://jamanetwork.com/journals/jamaoncology/fullarticle/2774311

[8] https://www.fda.gov/about-fda/oncology-center-excellence/oce-annual-report

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 diagnosis, 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 the development of drugs and devices for psychiatric and neurologic diseases. Further, the NCOE would help address the needs of persons living with serious neurological complications resulting from contracting SARS-CoV-2, affecting individuals' ability to function or work after the pandemic ends.

We agree with Former FDA Commissioner Scott Gottlieb, who identified neuroscience as the next logical consideration for a Center of Excellence. When commenting about the possibility of future centers of excellence during testimony before Congress, Gottlieb said the OCE "is an organizational model that we seek to adopt in other settings" and "some of the areas under consideration are immunology and neuroscience."[9]

Conclusion

We appreciate your consideration of our input and look forward to working with you and other Energy and Commerce Committee members on the important issue of neurological diagnostics, treatments and cures as Cures 2.0 moves through the legislative process. Should you have questions, please contact Clay Alspach at clay.alspach@leavittpartners.com, Josh Trent at josh.trent@leavittpartners.com, or Mark Roberts at mark.roberts@leavittpartners.com.

Sincerely,
The Neuroscience Working Table

[9] Testimony of Scott Gottlieb, M.D., FDA Commissioner before the Health, Education, Labor & Pensions Committee. 2017 https://www.help.senate.gov/imo/media/doc/Gottlieb5.pdf