August 2, 2024

The Honorable Diana DeGette
U.S. House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Larry Bucshon, M.D.
U.S. House of Representatives
2313 Rayburn House Office Building
Washington, DC 20515

Dear Representatives DeGette and Bucshon:

Thank you for inviting comments on the 21st Century Cures Act 2.0 (Cures 2.0). The undersigned epilepsy organizations are writing in strong support of your efforts to expand upon the successes of the 21st Century Cures Act and realize the goals of Cures 2.0. Through this process, we urge you to continue to support epilepsy research and the development of and access to new epilepsy drugs, devices, and other interventions.

Approximately 1 in 26 Americans will develop epilepsy at some point in their lifetimes. Epilepsy is a disease or disorder of the brain which causes reoccurring seizures. It is a spectrum disease comprised of many diagnoses including an ever-growing number of rare epilepsies. There are many different types of seizures and varying levels of seizure control. There is no one-size-fits-all treatment for epilepsy, and about 30% of people with epilepsy – over one million people – lack seizure control despite available treatments. Uncontrolled seizures greatly impede quality of life and can lead to disability, injury, and even early death. Each year, 1 out of 1,000 people with epilepsy die due to Sudden Unexpected Death in Epilepsy (SUDEP). This number dramatically increases to 1 in 150 for people whose seizures are not controlled.

As detailed below, the epilepsy community is proud to support several 21st Century Cures Act programs that have already been enacted and should be continued or expanded upon, as well as additional initiatives that should be part of Cures 2.0:

Brain Research Through Advancing Innovative Neurotechnologies (BRAIN) Initiative

The 21st Century Cures Act Innovation Fund allocated substantial mandatory resources to groundbreaking initiatives like the BRAIN Initiative. This ambitious program supports high-risk, high-reward research aimed at deepening understanding of the human brain. By developing cutting-edge tools, technologies, and Artificial Intelligence (AI) approaches, the BRAIN Initiative equips researchers with the means to further their own studies of the brain and unlock new treatments and cures.

The National Institutes of Health (NH) BRAIN Initiative has, and continues to have, a direct impact on how epilepsy is studied, understood, and treated. The use of neural recording and neurostimulation techniques and devices has not only improved the ability to precisely detect seizures, including where in the brain they originate, but also to respond in real time to predict and disrupt seizures. Examples of current work supported by the BRAIN Initiative include the use of an implantable brain device to treat Lennox-Gastaut Syndrome, a form of childhood onset epilepsy; and the use of a novel network-based neuromodulation approach to disrupt seizures without interfering with memory.

Unfortunately, however, these promising advances in brain research are threatened by budget cuts. The challenging budget climate, including the confluence of diminishing Innovation Account funds and spending caps set forth by the Fiscal Responsibility Act, will have dire consequences for the BRAIN Initiative unless Congress acts. In Cures 2.0, we strongly urge you to build on the successes to date and provide additional long-term investment for the BRAIN Initiative.

The Advanced Research Projects Agency for Health (ARPA-H) and National Institutes of Health (NIH)

ARPA-H was authorized by the 21st Century Cures Act. This important agency aims to accelerate better health outcomes for everyone by supporting the development of novel treatments. As ARPA-H continues to identify focus areas, we hope that epilepsy and developing better treatments for the more than 1 million people with epilepsy who are unable to achieve seizure control becomes a priority. The epilepsy community supports robust funding for ARPA-H that supplements rather than supplants foundational investment in the NIH.

We are concerned about Congressional proposals, including in the House FY 2025 Labor-Health and Human Services Appropriations Bill, to merge ARPA-H into the new National Institute on Innovation and Advanced Research at the NIH. Similarly, the epilepsy community is greatly concerned about Congressional proposals to reorganize the NIH from twenty-seven institutes to fifteen. This proposed reorganization would merge the National Institute of Neurological Disorders and Stroke, along with other Institutes on eye and dental research, into a new National Institute on Neuroscience and Brain Research. We strongly believe that such consequential changes to the NIH and ARPA-H should be carefully considered through a deliberative process that includes feedback from all stakeholders, including the epilepsy community, rather than being rushed through the appropriations process. We urge you to support the continued success of these entities through foundational investment in the NIH, supplemental funding for ARPA-H and thoughtful, transparent and inclusive consideration of any potential restructure of the institutes.

National Neurological Conditions Surveillance System (NNCSS)

First authorized through the 21st Century Cures Act, the NNCSS is an integrated system that uses state-of-the-art data sources, tools, and analytic methods to track the epidemiology of neurological conditions to increase understanding and catalyze research into their detection, diagnosis, and treatment. The Centers for Disease Control and Prevention (CDC) has been working on demonstration projects for Multiple Sclerosis and Parkinson's Disease, with plans to expand the NNCSS to additional neurological conditions. We are grateful for the CDC's ongoing epilepsy-related surveillance and epidemiologic studies on epilepsy and support the CDC's ability to enhance surveillance and epidemiologic studies of the epilepsies including through the NNCSS. In Cures 2.0, we urge you to reauthorize the NNCSS and expand it to the epilepsies.

National Plan for the Epilepsies

Our organizations strongly urge you to include a new provision in Cures 2.0 that would authorize a National Plan for the Epilepsies modeled on national plans for other neurological disorders like Alzheimer's and Parkinson's Disease. Such a plan would empower the federal government to pursue effective measures to prevent, better treat and cure the epilepsies.

Food and Drug Administration (FDA) Guidance

We are also supportive of the FDA guidance on the critical issues outlined in the request for information. In Cures 2.0, we urge you to build on the progress that has already been made and ask the FDA to apply these guidances to the development of epilepsy treatments. For example, there is not currently a cell or gene therapy for epilepsy, but these treatments are in development, and if approved could help people with genetic and common forms of epilepsy.

Expedited drug approval processes and the integration of real-world evidence into regulatory decision making are also important for people with epilepsy whose seizures are not well controlled by currently approved medications. Additionally, the coverage of breakthrough medical devices is needed for seizure detection devices that may help alert family members of seizures, as well as devices that assist in controlling seizures.

Additional Cures 2.0 Act Provisions Supported by the Epilepsy Community
We support several of the other initiatives included in the 2022 Cures 2.0 bill, and ask you to include them in the next iteration of Cures 2.0, including:

- **Research on Long COVID to better understand and treat this condition:** There has been some research[1,2] showing that COVID can increase the risk for epilepsy and seizures, especially among children, but more data and research is needed.
- **Patients and caregivers:** It is critical that the FDA consider the input of people with epilepsy in medical product development and regulation, including through the use of standardized patient experience data and real-world evidence. Additionally, formally recognizing the role of caregivers on the care team and providing caregivers with appropriate training and ability for input when appropriate is vital.
- **Health literacy:** The epilepsy community greatly appreciates Cures 2.0's provisions on improving health literacy around insurance coverage. If people with epilepsy do not fully understand their insurance options and benefits, then they are more likely to face substantial costs for medical services and treatments.
- **Diversity in clinical trials:** Our organizations support the use of clinical trials to find and test better treatments for epilepsy. We strongly believe that, in order for these trials to be successful, they must represent the diversity of people with epilepsy. The FDA's recent guidance on this topic will help make progress but to fully achieve this goal, we support Cures 2.0's taking additional steps including making clinicaltrials.gov more user-friendly. We also recommend adding provisions to the bill that require the FDA to provide guidance on topics such as including people of color in decentralized trials, taking steps to reduce the burden of participation in trials, and engaging community partners in these efforts.
- **Access to genetic testing:** Genetic testing is critical for the many genetic forms of epilepsy. We are supportive of Cures 2.0's efforts to expand access to this testing.
- **Coverage of devices:** We support efforts to create a pathway to accelerate the coverage of new and innovative devices and would support alignment in Cures 2.0 with the Ensuring Patient Access to Critical Breakthrough Products Act's (H.R. 1691) approach if that bill is not passed this year. People with epilepsy will only benefit from breakthrough devices if a pathway exists to promote faster coverage and access to devices that can support their health and independence.

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[1] Taquet M, Devinsky O, Cross JH, Harrison PJ, Sen A. Incidence of Epilepsy and Seizures Over the First 6 Months After a COVID-19 Diagnosis: A Retrospective Cohort Study. Neurology. 2023 Feb 21;100(8):e790-e799. doi: 10.1212/WNL.0000000000201595. Epub 2022 Nov 16. PMID: 36384658; PMCID: PMC9984208.

[2] Taquet M, Sillett R, Zhu L, Mendel J, Camplisson I, Dercon Q, Harrison PJ. Neurological and psychiatric risk trajectories after SARS-CoV-2 infection: an analysis of 2-year retrospective cohort studies including 1 284 437 patients. Lancet Psychiatry. 2022 Oct;9(10):815-827. doi: 10.1016/S2215-0366(22)00260-7. Epub 2022 Aug 17. PMID: 35987197; PMCID: PMC9385200.

Conclusion

Thank you for your leadership on Cures 2.0. The undersigned organizations are proud to support this important initiative and urge you to continue to build on the 21st Century Cures Act in supporting epilepsy research and the development and coverage of epilepsy therapies. Should you have any questions, please do not hesitate to contact Roxanne Yaghoubi at ryaghoubi@efa.org or any of our community members.

Sincerely,
BPAN WARRIORS
CACNA1A Foundation
Child Neurology Foundation
COMBINEDBrain
CTNNB1 Connect and Cure
Cure KCNH1 Foundation
Danny Did Foundation
Developmental and Epileptic Encephalopathies Project (DEE-P Connections)
Dup15q Alliance
Epilepsies Action Network (EAN)
Epilepsy Alliance America
Epilepsy Foundation
Glut1 Deficiency Foundation
HNRNP Family Foundation
Hope for Hypothalamic Hamartomas
International SCN8A Alliance
KCNQ2 Cure Alliance
Malan Syndrome Foundation
Pediatric Epilepsy Research Consortium
Phelan-McDermid Syndrome Foundation
PURA Syndrome Foundation
Rare Epilepsy Network (REN)
Ring14 USA
SHINE Syndrome Foundation
STXBP1 Foundation
Tbc1d24 Foundation
The Cute Syndrome Foundation
The MED13L Foundation
The SPATA Foundation
The Sturge-Weber Foundation
THG1L Support Group