National Brain Tumor Society

21st Century Cures Act, H.R. 6
Impact on the Brain Tumor Community

The 21st Century Cures Act (H.R.6), sponsored by Representatives Fred Upton (R-MI) and Diana DeGette (D-CO), is comprehensive legislation that will help accelerate the development of new treatments and increase the patient's voice in process. It will also enable innovation in clinical trial design, help expand access to investigational medicine and invests in medical research. The legislation is a significant step forward for advancing our efforts to ultimately deliver new and effective treatments and ultimately a cure to brain tumors.

Following are the key sections of the 21st Century Cures Act that are critical to National Brain Tumor Society's mission and community:

Investment in Medical Research
National Brain Tumor Society strongly supports the bill's investment in the National Institutes of Health (NIH) including an increase in funding from 2016-2018.

In addition, we support the proposed NIH Innovation Fund including the following attributes:

- Investment in early stage investigators
- Strategic planning and focus areas on precision medicine and biomarkers
- Emphasis on rare and pediatric diseases
- Emphasis on data sharing

A brain tumor diagnosis represents, at minimum, a potentially life-threatening and life-altering disease. Depending on the type, a brain tumor can be one of the deadliest cancers; such is the case with glioblastomas and pediatric high-grade gliomas. The NIH is the largest source of brain tumor research funding in the United States. While there have been important advancements in brain tumor research, there continues to be only 4 drugs and 1 device approved for the treatment of malignant brain tumors, and progress to extend life has been too slow. Now is the time to increase investment in brain tumor research - to invest in new, promising projects and leverage previous investment by the NIH to drive clinical and pre-clinical research forward, toward the development of new treatments that more precisely target the right patient, with the right treatment at the right time and are less drug resistant, extend survival and improve patients lives.

Clarity and Standardization for Drug Development
The legislation requires that a process for qualification of drug development tools be established. Tools could include biomarkers, clinical outcomes assessment, and any other measure that aids drug development or regulatory review. The legislation also requires the creation of guidance on the development of biomarkers and surrogate endpoints. *(A biomarker is a physiologic, pathologic or anatomic characteristic that is objectively measured and evaluated and is an indicator of normal biologic processes, pathologic processes or biologic response to a therapeutic intervention.*

A surrogate endpoint is a marker such as a laboratory measurement, radiographic image, physical sign or other measure that is not a direct measure of clinical benefit but is known to predict clinical benefit and could be used to support traditional approval of a drug or biological product, or is reasonably likely to predict clinical benefit and could be used to support accelerated approval of a drug or biological product by the FDA).

National Brain Tumor Society strongly supports this section because it enables greater opportunity than ever to establish clarity in the assessment of novel investigational agents in brain tumor clinical trials. We believe a clear qualification process for drug development tools will increase confidence for sponsors to use drug development tools in their clinical trials. Moreover, a well-defined process for the qualification of biomarkers and surrogate endpoints provides a framework or criteria for the development of new and improved scientifically and medically appropriate predictive biomarkers and endpoint measures for use in clinical trials. Importantly, the legislation also provides drug sponsors with a "roadmap" and incentives to request that the FDA consider an accelerated approval development plan requiring mutual agreement on which surrogate endpoints should be used, as well as on the design of the clinical trial. This provision will accelerate the delivery of novel treatment options earlier to patients with deadly diseases and is consistent with the aims of National Brain Tumor Society's efforts to jumpstart brain tumor drug development by improving clinical trial endpoints.

Guidance on Precision Medicine
The legislation requires the creation of guidance on the topic of precision medicine. Precision medicine in a research context is research that identifies and establishes the efficacy of treatments targeted at likely responders in the right dose, at the right time. The proposed guidance on precision medicine is poised to help industry sponsors understand the evidence needed to use biomarkers to identify subsets of patients likely to respond to a treatment, and help with the development of biomarkers that can inform the measurement of the impact of a drug, as well as prescribing decisions.

Guidance on Adaptive Clinical Trial Design
The legislation calls for a new regulatory guidance document on the use of adaptive clinical trials designs. Adaptive clinical trial design has been used successfully to test drugs in humans and offers several advantages over traditional designs including a requirement for smaller numbers of human subjects, thereby reducing patient exposure to the potential toxicity of investigational drugs. Adaptive design can also help validate novel biomarkers. National Brain Tumor Society believes such guidance will help sponsors plan new and potentially less costly and more patient-centered clinical trials.

Expanded Access Policy
The legislation requires that pharmaceutical companies make publically known their policy for allowing access, outside of a clinical trials context, to investigational new drugs. We support this provision, as it will help increase transparency and hopefully improve access by doctors and their patients to not-yet-approved drugs that could potentially extend life for brain tumor patients.

National Neurological Diseases Surveillance System
The legislation requires the Centers for Disease Control (CDC) to enhance and expand infrastructure and activities to track the epidemiology of neurological diseases. The CDC is required to collect information on neurological diseases including demographic information and risk factors, diagnosis and progression markers, and the natural history of the disease. The section also authorizes the CDC to make grants to carry out these activities.

Brain tumors are also a neurologic disease. National Brain Tumor Society supports this section as it could expand research opportunities and facilitate the development of treatments for rare diseases such as brain tumors. We are pleased that the Energy and Commerce Committee in its report make it clear that brain tumors could be included in the CDC funded system.

NIH Authority to Require Data Sharing
The legislation authorizes the Director of the NIH to require recipients of financial support to share scientific data generated from the research with certain limitations (such as privacy).

National Brain Tumor Society supports this section as a means of facilitating the flow of information across the research community. The brain tumor research community is relatively small, but spread worldwide, and, thus, dependent upon researchers being able to access data. It is also in the public interest for publicly funded research to be shared for research purposes to maximize the potential for new discoveries and potential treatment strategies.

Standardization of Data in Clinical Trial Registry Data Bank on Eligibility for Clinical Trials
The legislation requires the Director of NIH to ensure that a clinical trial registry data bank is easily able to be used by the public, that the information may be easily compared, and that the criteria for clinical trial eligibility is included in a standardized format.

National Brain Tumor Society supports efforts to make information about clinical research more accessible and understandable to the public and especially to patients and their families.

Council for 21st Century Cures
The legislation would establish a new nonprofit corporation called the Council for 21st Century Cures to accelerate the discovery, development and delivery in the U.S. of innovative cures, treatments and preventative measures for patients. The Council will establish a strategic agenda for acceleration and identify gaps and opportunities.

National Brain Tumor Society supports the concept of the Council as a means for developing new strategies aimed at increasing drug development for brain tumors. We are pleased that the legislation calls for patient advocacy participation in the Council.