

## 21st Century Cures Act:

Enabling improved and accelerated brain tumor research and development of new treatments



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### Problem

Investment in the United States' biomedical infrastructure has not kept pace with life science innovation in the 21st Century. A comprehensive approach is necessary to remove barriers to drug discovery and development, as well as increase funding for the National Institutes of Health, the largest funder of brain tumor research.

### Solution

**National Brain Tumor Society strongly supports many of the provisions granted in the 21st Century Cures Act, which will improve brain tumor research and drug development:**

**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.

**Advancing Tools to Aid and Expedite Drug Development:** National Brain Tumor Society strongly supports the creation of guidance on the development of biomarkers and surrogate endpoints. A well-defined process for the qualification of biomarkers and surrogate endpoints provides a framework for the development of new and improved scientifically and medically appropriate predictive biomarkers and endpoint measures for use in clinical trials. This enables greater opportunity to establish clarity in the evaluation of drugs and other treatments in brain tumor clinical trials.

**Guidance on Precision Medicine:** National Brain Tumor Society strongly supports the requirement of the creation of guidance on the topic of precision medicine. Precision medicine is a research concept that identifies and establishes the efficacy of treatments targeted at likely responders in the right dose, at the right time.

**Guidance on Adaptive Clinical Trial Design:** Adaptive clinical trial designs have been used successfully to evaluate drugs in other cancers, and offer several advantages over traditional designs, to include a requirement for smaller numbers of human subjects. National Brain Tumor Society believes such guidance will help industry plan new, potentially less costly and more efficient patient-centered clinical trials.

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# Public Policy Issue Brief

**Expanded Access Policy:** The Act requires that drug developers make publically known their policy for allowing access, outside of a clinical trials context, to investigational new drugs. National Brain Tumor Society supports 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 of brain tumor patients.

**National Neurological Diseases Surveillance System:** National Brain Tumor Society supports the requirement for the Centers for Disease Control (CDC) to enhance and expand infrastructure and activities to track the epidemiology of neurological diseases to include brain tumors.

**NIH Authority to Require Data Sharing:** The Act authorizes the Director of the NIH to require recipients of financial support to share scientific data generated from the research with certain exceptions (such as for privacy). National Brain Tumor Society supports this section as a means of facilitating the flow of information across the research community.

**Standardization of Data in Clinical Trial Registry Data Bank:** The Act requires the Director of NIH to ensure that a clinical trial registry and results database is easily able for use 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 Act would establish a new nonprofit corporation called the Council for 21st Century Cures to accelerate the discovery, development and delivery of innovative cures, treatments and preventative measures for patients. 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.

## About National Brain Tumor Society

National Brain Tumor Society is the largest nonprofit organization dedicated to the brain tumor community in the United States. We are fiercely committed to finding better treatments, and ultimately a cure, for people living with a brain tumor today and those who will be diagnosed tomorrow. This means aggressively driving strategic research and advocating for public policies, which meet the critical needs of this community. It's time to build on progress and transform tomorrow, today. To learn more visit [www.braintumor.org](http://www.braintumor.org).