



May 12, 2015

Dear Chairman Upton and Ranking Member Pallone:

The Pediatric Low Grade Astrocytoma (PLGA) Foundation is pleased to offer this letter of support for the 21st Century Cures draft language released on April 29, 2015. The 21st Century Cures Initiative has the potential to dramatically alter the landscape for innovation. We thank you for your dedication, vision, and hard work towards bringing bi-partisan legislation forward that will speed the development and delivery of cures to patients. The PLGA Foundation is dedicated to giving hope to children battling slow-growing children's brain cancer by supporting targeted scientific and medical research.

PLGA is the most common form of childhood brain cancer, impacting over 20,000 children in the United States. Each year, over 1,000 new children diagnosed with some type of astrocytoma brain tumor. Current treatments for slow-growing Pediatric Low Grade Astrocytoma brain cancer are invasive, highly toxic, and relatively ineffective. For far too long, research on pediatric cancers, and PLGA cancer in particular, has been underrepresented at the NIH. With the support of private investment by the PLGA Foundation, dramatic advances have been made in the understanding of the disease. With the support of the NIH and the PLGA Foundation, tremendous progress has been made in understanding the pathways that are active in slow growing PLGA brain cancer. The progress, however, has been much slower with regard to translating this understanding into patient access to new therapies to treat PLGA. The PLGA Foundation is endorsing the 21st Century Cures effort because we believe that this legislation will help to translate the understanding of rare diseases like PLGA into new and effective treatments.

Specifically, the PLGA Foundation supports the following provisions of the 21st Century Cures package:

- **NIH Funding and Reauthorization:** The lack of a mouse model, and the absence of sufficient tissue samples for research are two of the greatest challenges facing PLGA research. Strong support for the NIH is critical to unlocking these types of basic research questions. (Title I, Subtitle A).
- **Support for High-Risk Research Efforts:** Although PLGA is the most common childhood brain cancer, the disease impacts a relatively small number of patients. The bill's support for research that pursues innovative, but high-risk, approaches to major challenges in biomedical research will spur the out-of-the-box thinking necessary for advances in rare diseases like PLGA. (Title I, Subtitle B, Section 1028)

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- **Pediatric Research Network:** The establishment of a national pediatric research network will assist in the creation of a global pediatric clinical trial network. This type of network will encourage more collaboration on rare diseases like PLGA. (Title I, Subtitle E)
- **Data Sharing:** The relatively low number of cases of PLGA children's brain cancer complicate efforts to advance research on this devastating disease. NIH-led efforts to encourage collaboration can assist efforts to achieve breakthroughs on PLGA. (Title I, Subtitle F)
- **Patient-focused drug development:** This section is of particular importance to patients with rare, pediatric brain tumors. The current treatments for PLGA brain cancer, including chemotherapy, surgery, and radiation are devastating on children's developing bodies and brains. Allowing an opportunity for patients to be included in the drug development process will ensure a better understanding of the impact of new therapies. (Title II, Subtitle A)
- **Precision Medicine:** – NIH Director Francis Collins noted in a recent hearing that precision medicine holds great promise for rare pediatric cancers like PLGA which have largely not benefited to date from the advancements made in this field. Guidance from the FDA, as recommended in this section, is sorely needed to provide quicker access to innovative therapies. (Title II, Subtitle C)
- **Compassionate Use:** Very few treatment options exist for PLGA brain cancer. For patients seeking compassionate use access to these treatments, this provision will ensure that patients receive timely information on the status of their request, as well as the reason for approval or denial. We thank the Committee for inclusion of this important provision. (Title II, Subtitle E)
- **Repurposing of drugs:** The discovery that PLGA shares the same gene and pathways as melanoma has added scientific value and worldwide scientific interest in PLGA. Encouraging the repurposing of drugs for serious and life-threatening conditions, and making these drugs available in a timely fashion, could be particularly important for PLGA patients. We look forward to reviewing this section and providing feedback once language is available. (Title II, Subtitle I)

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- **Streamlining the clinical trial process:** The limited number of patients available for PLGA clinical trials could necessitate that clinical trials for this disease be conducted at multiple sites. Providing an efficient process for review of data collected will expedite the approval process and ensure the most innovative therapies reach PLGA patients quickly. (Title II, Subtitle N, Section 2241).

Our patients cannot wait. 21st century cures are necessary to ensure access to the latest discoveries that hold the promise of cures for children with rare pediatric brain tumors. We thank the committee for its thoughtful consideration of this bi-partisan legislation, and look forward to serving as a resource as the legislative process continues.

Best,

Amy J. Weinstein
Executive Director
PLGA Foundation: A Kids' Brain Tumor Cure

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98 Random Farms Drive, Chappaqua, NY 10514 • Phone/Fax: **914-762-3494** ► akidsbraintumorcure.org