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May 1, 2022

The Honorable Patty Murray
Chair
Committee on Health, Education, Labor and Pensions
428 Senate Dirksen Office Building
Washington, D.C. 20510

The Honorable Richard Burr
Ranking Member
Committee on Health, Education, Labor and Pensions
835 Senate Hart Office Building
Washington, D.C. 20510

The Honorable Frank Pallone Jr.
Chairman
Committee on Energy and Commerce
2125 Rayburn House Office Building
Washington D.C. 20515

The Honorable Cathy McMorris Rodgers
Ranking Member
Committee on Energy and Commerce
2322 Rayburn House Office Building
Washington, D.C. 20515

There have been few actionable cancer bills that have the potential to have as much impact as the Promising Pathway Act has. Particularly with pediatric brain cancers - terminal diagnoses that many experts may fundamentally change how we fight many other cancers - the Promising Pathway Act (PPA) not only will double the speed of revolutionary treatments but may also uncover the cure we've been seeking for over a century.

This is no more evident than in the fight against diffuse intrinsic pontine glioma (DIPG) and diffuse midline glioma (DMG); a disease that has a survival rate of less than 3% and is resistant to current treatment methods. As parents that have lost our children to DIPG/DMG and those that are still fighting, we are painfully aware that this pediatric cancer is not only the "biggest bully" but may also provide "homerun cure" implications that can change how we cure many other forms of cancer. Still, today we do nothing.

We ask for your support of the PPA, not only because it may save lives and refocus our efforts in the fight against cancer, but may actually reduce the costs involved with treating patients.

Through the passage of the PPA:

- Patients will obtain access to provisional treatments faster than current FDA pathways, while maintaining the FDA gold standard. Right now, there are many families trying to get access to experimental therapies via trials or expanded access and they are not able to because the programs are too limited by geography and number of patients they can handle. We know of several examples where we have seen well-tolerated drugs sidelined by the FDA requests for more information, while sometimes demonstrating 20-30% response rates or unparalleled successes in translational studies at the same time as witnessing families with no treatment options. Instead they are told to take their child home because there is nothing they can do. Imagine if you had a child with such a horrific disease and knew that there was something that even had a little chance to help but you were not able to get access. How would you feel? Under the PPA, this would never happen.
- Pediatric therapies will be accessible for terminal cases at the same time as conventional adult trials. This is an important consideration because, in diseases like DIPG that typically affect only children, we often must first conduct an adult trial before the therapy can then progress to the patients it was intended for.
- In rare cancers like DIPG/DMG, the current FDA approval process is not practical because we have such a small patient population - with only 200-300 patients per year and because the average lifespan is around 9-18 months. It is also thought that the ultimate cure is going to be a combination of a few treatments, each of which are not likely to get FDA approval on their own. This makes it impossible to test the combinations needed, and with personalized medicine, clinical trials to test 4 treatment combinations would take decades and be impractical. With PPA, all patients are followed in a virtual trial registry while their doctors determine the best combinations for their specific case. This registry data can be mined to direct future combinations.
- Under the current compassionate access system, once the treatment is approved, there exists no centralized, third-party tracking system on patient responses or side effects. This is both reckless and short-sighted. Through the PPA, third-party registries are required both for communication to the patient population and researchers, but also to eventually assist in the full drug approval determination. This is also critical in the design of combinational therapy options as it will demonstrate future considerations of care. The FDA approval pathways must be continually rethought and redesigned to balance faster access to therapies with increased post-market surveillance requirements. The PPA provides the framework to protect patient safety, while also providing patients with the opportunity to access potentially life-saving treatments the current system prevents them from accessing.

As Congress continues to consider legislative solutions to support patients with rare and life-threatening diseases, and in light of the current congressional efforts to reauthorize the FDA

user fee program, we urge you support and pass the PPA. The PPA is not only a way to advance trials and protect patients, but it may be the most effective way to save lives. It is for this reason that over 50 foundations and chapters in 21 states and 3 countries support this measure. We need your help in making it a priority.

Sincerely,

Brooke Desserich
Founder & CEO
The Cure Starts Now Foundation and Chapters

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The Brooke Healey Foundation

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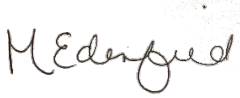

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