EPILEPSY FOUNDATION

June 4, 2018

Roger Brown
Director of the House of Delegates
American Medical Association

Dear Director Brown:

On behalf of the Epilepsy Foundation, we strongly urge you to carefully consider the impact of the proposed changes to the *Orphan Drug Act* (ODA) contained in Resolution 217 (A-18). We agree that there are improvements that can be made to the existing Act to uphold the underlying principles, and would request involvement in crafting specific, well-thought out recommendations. Any changes should not negatively impact individuals with rare diseases, including those with rare epilepsies. The vast majority of rare epilepsies lack an approved medication.

The Epilepsy Foundation is the leading national voluntary health organization that speaks on behalf of the at least 3.4 million Americans with epilepsy and seizures. We foster the wellbeing of children and adults affected by seizures through research programs, educational activities, advocacy, and direct services. Epilepsy is a medical condition that produces seizures affecting a variety of mental and physical functions. Approximately 1 in 26 Americans will develop epilepsy at some point in their lifetime.

For the majority of those living with epilepsy, prescription medication is the most clinically-effective and cost-effective treatment for controlling and/or reducing seizures. People with epilepsy must have meaningful and timely access to physician-directed care because epilepsy medications are not interchangeable and treatment of epilepsy is highly individualized. There is no "one size fits all" treatment option for epilepsy, and the response to medication may be different for each person. About a third of people with epilepsy experience uncontrollable or treatment-resistant epilepsy. Many individuals with treatment-resistant epilepsy, mostly children, can experience hundreds of seizures a day and live with the continual risk of injury and death. There are currently no FDA-approved therapies for many of the rare epilepsies. Each year, 1 in 1,000 people die of Sudden Unexpected Death in Epilepsy – with the highest risk factor being uncontrolled, under treated seizures. With small population sizes, in the past rare epilepsies did not gain the same amount of attention for research and development of new treatment options. Incentivizing breakthrough research for these small populations, as the ODA does, brings rare epilepsies one step closer to finding treatments and cures.

The ODA has proven to be critical in incentivizing therapeutic development for rare diseases. Before ODA was enacted, there were only 34 orphan therapies in existence. Today, there are more than 600, and 2017 was a record year in the number of orphan therapies for rare diseases. This Act gives hope to those with rare epilepsies and their families, and has fostered research and innovation leading to a number of approved medications. For example, Lennox-Gastaut syndrome, which accounts for 4% of children with epilepsy, now has two treatment options that

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were designated as orphan drugs under the ODA. Emergency medication, such as Diastat for individuals who have breakthrough seizures, was also approved under the ODA. In 2009, the FDA approved everolimus for the treatment of certain symptoms of Tuberous Sclerosis Complex—another rare epilepsy. The ODA has fostered continued innovation in this space; on April 19, 2018 the Peripheral and Central Nervous System Drugs Advisory Committee of the FDA unanimously recommended (13-0) approval Epidiolex for the treatment of Dravet syndrome and Lennox-Gastaut syndrome. Without the important incentives included in the ODA, it is unlikely these potential treatment options would exist at this time. Changes to the Act could negatively impact future innovation, which provides hope for new lines of treatment for individuals and their families.

We are concerned about the vague statement about proposed changes to the incentives provided that encourage research and innovation in the rare disease space. Just 6% of orphan drugs in development reach approval, which is significantly less than the success rates for medication overall.[1] Companies must perform complex and costly clinical trials to gain an orphan indication as with any new indication. Only 1 in 5 orphan drugs has a non-orphan indication,[2] and those that do face the same market pressures and competition of the broader indication to help contain costs. Without the existing incentives, we are concerned that critical research and innovation would be stifled at a time when we are seeing unprecedented progress in our fight against rare epilepsies. The ODA allows researchers to follow the science, which helps define diseases more accurately and precisely than ever before; this is the kind of research that should be incentivized.

The Epilepsy Foundation requests involvement in crafting specific, well-thought out recommendations and urges you to carefully consider the impact that potential changes to the *Orphan Drug Act* may have on biomedical innovation for rare diseases, including epilepsy. Please feel free to contact Abbey Roudebush, Government Relations Manager, at aroudebush@efa.org or 301-918-3784 with any questions or follow-up.

Sincerely,

Philip M. Gattone, M.Ed.
President & CEO
Epilepsy Foundation

[1] CH Wong and AW Lo, "Estimation of Clinical Trial Success Rates and Related Parameters," *Biostatistics* (2018), 1-14.
[2] QuintilesIMS Institute, "Orphan Drugs in the United States: Providing Context for Use and Cost," October 2017.

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