



## Incentivizing the Discovery and Development of Therapies for Rare Diseases

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**Position:** The Epilepsy Foundation is committed to fostering biomedical innovation because more than a third of people living with epilepsy do not have seizure control and many more live with significant side-effects from existing medications. **We support public policies that accelerate the discovery, development, and delivery of lifesaving medications, especially for rare conditions, including rare epilepsies.** With small population sizes, rare epilepsies do not gain the same amount of attention for research and development of new treatment options. Incentivizing breakthrough research for these small populations will bring rare epilepsies one step closer to finding treatments and a cure.

**About Epilepsy:** The Epilepsy Foundation is the leading national voluntary health organization that speaks on behalf of more than 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.

There is no “one size fits all” treatment for epilepsy, and about a third of people with epilepsy live with uncontrollable or intractable seizures. Many individuals with treatment-resistant epilepsy, mostly children, may experience hundreds of seizures a day and live with the continual risk of injury and death. There currently are no FDA-approved therapies for many rare epilepsies. People living with intractable epilepsy often have other disabilities and co-morbidities, and they require complex treatment plans in the hopes of minimal seizure reductions.

**Background:** The *Orphan Drug Act of 1983* (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 approved by the Food and Drug Administration (FDA). The ODA incentivizes the development of therapies for rare diseases through several incentives. Orphan drugs receive seven years of market exclusivity, longer than common disease therapies, in order to allow orphan drug developers to recoup their costs. In addition, orphan drug developers qualify for tax credits and government grant programs, and are excluded from some regulatory requirements and fees.

**If you have any questions or concerns, please contact Beatriz Duque Long, Interim Vice President Public Policy at [bduquelong@efa.org](mailto:bduquelong@efa.org) or 301-918-3764.**