



October 12, 2015

The Honorable Paul D. Ryan, Chairman
Committee on Ways and Means
U.S. House of Representatives
1102 Longworth House Office Building
Washington, DC 20515

RE: *Preserving Access to Orphan Drugs Act (H.R.3678)*

Dear Chairman Ryan:

Thank you for your continued leadership and commitment to ensuring that the men, women and children affected by one of the nearly 7,000 known rare diseases continue to have access to appropriate and often life-saving treatments.

The National Organization for Rare Disorders (NORD) encourages you to support the *Preserving Access to Orphan Drugs Act (H.R.3678)*, introduced by Congressmen Mike Kelly and Richard Neal. This legislation will make a small technical correction related to the exclusion of orphan drugs in the annual pharmaceutical fee provision of the *Patient Protection and Affordable Care Act (PL 111-148)*.

The existing statute requires that an orphan drug, in order to be excluded from the annual fee, receive an Orphan Drug Tax Credit. While many orphan drugs have received the credit, not all drugs that only treat orphan conditions have received it. For example, if a therapy has multiple orphan indications, and did not receive the Orphan Drug Tax Credit for those licensed indications, it would be subject to the fee.

It is NORD's belief that the statute unintentionally created an imbalance for rare disease therapies. The statute specifically excludes those therapies that have received the orphan tax credit from the annual pharmaceutical fee. However, the statute requires those products that are solely indicated to treat the very same orphan condition(s), but did not receive the orphan drug tax credit, to pay the annual pharmaceutical fee. Should such products be ineligible for the orphan drug tax credit, we are concerned that this fee may actually be a disincentive for future development of therapies to treat orphan conditions.

The Orphan Drug Tax Credit has been a successful tool to promote the development of over 450 therapies to treat over 350 rare conditions. However, linking that tax credit to the eligibility for exclusion from the annual fee will have an adverse impact on innovation and eventual access to life-saving orphan drugs. Therefore, we respectfully request that the pharmaceutical fee and its exclusion for orphan drugs not be linked to the Orphan Drug Tax Credit. We believe the credit can be one measure for exclusion from the fee but alternatively, if a therapy is indicated solely for the treatment of an orphan condition, it should also be excluded from the fee.

NORD is a unique federation of voluntary health organizations dedicated to helping people with rare “orphan” diseases and assisting the organizations that serve them. We are committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and service. NORD supports a social, political, and financial culture of innovation that supports both the basic and translational research necessary to create diagnostic tests and therapies for all rare disorders.

We ask that you consider the *Preserving Access to Orphan Drugs Act* as you review and address legislation in this session of Congress. For additional information regarding this important legislation, please contact Paul Melmeyer, Associate Director of Public Policy, at pmelmeyer@rarediseases.org, or (202) 588-5700 ext. 104.

Sincerely,

A handwritten signature in cursive script, appearing to read "Peter L. Saltonstall".

Peter L. Saltonstall
President and CEO