

February 3rd, 2014

The Honorable John A. Boehner, Speaker 1011 Longworth House Office Building Washington, DC 20515-3508

Dear Mr. Speaker,

On February 28, millions of Americans will observe Rare Disease Day, an awareness event that takes place on the last day of February each year. At this special time, and on this special day, we ask that you join us in recognizing the successes the rare disease community has achieved, as well as the challenges the rare disease patient faces in accessing safe, effective and affordable treatments.

Since approximately 1 in 10 of your constituents has a rare disease, we are enclosing resources (a sample press release and sample tweets and Facebook posts) to help you show your support for the many residents of your state who will be participating in Rare Disease Day activities.

The rare disease community celebrated many achievements in 2013. The implementation of the Patient-Focused Drug Development Initiative at the Food and Drug Administration, which was enacted as part of the Food and Drug Administration Safety and Innovation Act (FDASIA), has already strengthened the patient's voice in the drug review process. And this past November, Congress passed the National Pediatric Research Network Act that will strengthen the coordination of pediatric research by setting up a collaborative network across the country.

While the Orphan Drug Act continues to be considered one of the most successful pieces of legislation of the 20th Century, with over 400 orphan therapies approved since 1983, there is still work to be done. Although there are over 400 orphan therapies treating about 300 rare diseases, the vast majority of the 7000 known rare diseases do not have adequate treatment.

We hope that you join us this Rare Disease Day to recognize the importance of rare disease research and orphan therapy development, while recognizing the many challenges the rare disease community continues to face. If you haven't done so already, please consider joining your colleagues in the House Rare Disease Caucus. Please also consider showing your support for the rare disease community this Rare Disease Day on social media, on your website, or on any platform you see fit. Your constituents who have a rare disease will be heartened to know their Congressman standing up for them.

If you desire additional information, please contact me at <u>ddorman@rarediseases.org</u> or (202) 588-5700 ext. 102.

Sincerely,

Diane Edquist Dorman

Vice President