

United States Senate

WASHINGTON, DC 20510

March 3, 2017

The Honorable Gene L. Dodaro
Comptroller General
U.S. Government Accountability Office
441 G Street, N.W.
Washington, D.C. 205488

Dear Mr. Dodaro,

Since passage of the Orphan Drug Act (ODA) in 1983, the FDA has approved over 600 orphan drugs and has granted nearly 4,000 orphan drug designations. The incentives provided in this historic legislation motivate drug companies to invest in areas that would otherwise be overlooked and, as a result, has helped save countless lives.

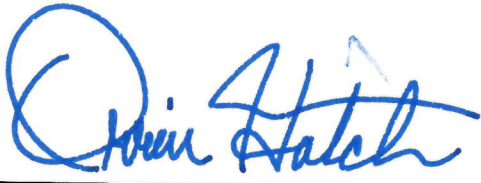
While few will argue against the importance of the development of these drugs, several recent press reports suggest that some pharmaceutical manufacturers might be taking advantage of the multiple designation allowance in the orphan drug approval process.

To inform Congress' work on this important issue, please provide a list of each drug for the past year for which there was a request for a designation of orphan status and which were approved and which were denied. Of those drugs, please list any indications for the drug, other than the orphan designation. Additionally, please include a list of all orphan-approved drugs since enactment of the ODA and whether resources at the FDA have kept up with the number of requests received by the Office of Orphan Products Development (OOPD). We would also like to know if evaluation criteria are consistent across reviewers in the OOPD. Furthermore, we feel that it is important to include the patient voice in your review, and to the extent possible, please include patient experiences in your review.

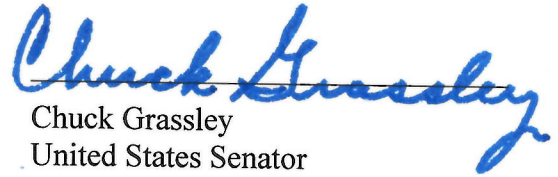
Additionally, we request the Government Accountability Office to investigate whether the ODA is still incentivizing product development for diseases with fewer than 200,000 affected individuals, as intended, and provide any regulatory or legislative changes that may be needed in order to preserve the intent of this vital law. It is essential that we maintain the integrity of this important law and continue to stimulate the pipeline for rare disease drugs.

Thank you for your careful consideration of our request. If you have any questions about this request, please contact the relevant staff in our offices: For Senator Hatch, please contact Stuart Portman at stuart_portman@hatch.senate.gov; for Senator Grassley, please contact Karen Summar at karen_summar@grassley.senate.gov; for Senator Cotton, please contact Abigail Welborn at abigail_welborn@cotton.senate.gov. We look forward to working with you.


Sincerely,

A handwritten signature in blue ink that reads "Orrin Hatch". The signature is written in a cursive style with a large, prominent "O" at the beginning.

Orrin Hatch
United States Senator

A handwritten signature in blue ink that reads "Chuck Grassley". The signature is written in a cursive style with a large, prominent "C" at the beginning.

Chuck Grassley
United States Senator

A handwritten signature in blue ink that reads "Tom Cotton". The signature is written in a cursive style with a large, prominent "T" at the beginning.

Tom Cotton
United States Senator