

Congress of the United States
House of Representatives
Washington, DC 20515

Help Patients with Rare Diseases Obtain Drugs Faster

Cosigners: Bilirakis, Butterfield, Ros-Lehtinen, Moran, Blackburn, McCaul, Huffman, Cartwright, Barletta, Carter, Polis, Michaud, Clay, Burgess, Bentivolio, Collins, Pittenger, Carson, Carney, Fitzpatrick, Petri, Joyce and Jackson-Lee.

Dear Colleague:

Nearly one in ten Americans suffer from a rare disease—the majority of those affected are children. Even more alarming is that 95 percent of the 7,000 rare diseases, many of them life-threatening, have no approved treatment by the Food and Drug Administration (FDA).

The Food and Drug Administration Safety and Innovation Act of 2012 (FDASIA) sought to address some of the regulatory uncertainty by including the Faster Access to Specialized Therapies Act (FAST) as an amendment. This bipartisan legislation improved the Accelerated Approval process for all life-threatening diseases, in addition to calling on FDA to take into consideration in drafting their guidance the specific and unique issues that hinder access to Accelerated Approval for rare disease treatments.

The FDA's recently-released draft guidance was a good first step in fulfilling Congressional intent to expand the use of Accelerated Approval. However it fails to address the specific issues with rare disease drug development as clearly required by the law.

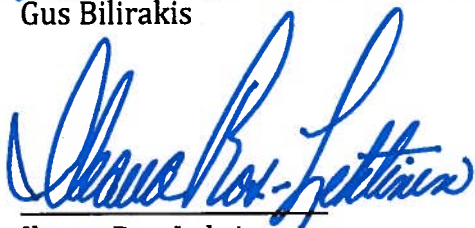
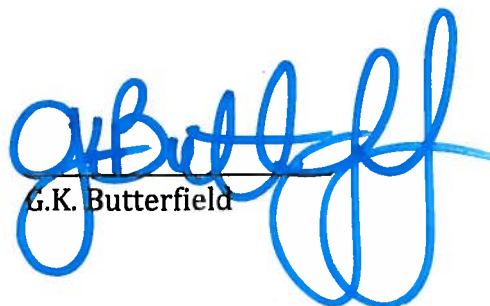
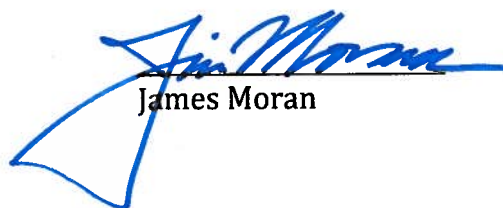
We are sending the attached letter to FDA because:

- There is an extremely urgent need for rare disease patients to have access to lifesaving treatments;
- Regulatory clarity is important to foster biotechnology, innovation and investment capital;
- More than 300 patient groups, academic institutions and medical societies supported the FAST Act and deserve timely implementation; and
- FDA's response to FDASIA requirements is inadequate and does not meet Congressional intent.

We hope that you will join us in signing this letter to the FDA to bring hope and options to the 30 million Americans with rare diseases.

Please contact Thomas Power with Rep. Gus Bilirakis (Thomas.Power@mail.house.gov) or Saul Hernandez with Rep. G.K. Butterfield (Saul.Hernandez@mail.house.gov) for more information.

Sincerely,


Gus Bilirakis
Ileana Ros-Lehtinen
G.K. Butterfield
James Moran