STAT Act
SPEEDING THERAPY ACCESS TODAY

Cosponsor the Speeding Therapy Access Today Act of 2021, H.R. 1730/S. 670

The STAT Act is a bipartisan bill that was created with the input of the rare disease community aimed at improving the development of and access to therapies for the rare disease community.

"*In many cases, developing a treatment for a rare disease can be especially hard and present unique challenges. Each success is the end of a long uphill climb. It requires the concerted efforts of stakeholders, including scientists, product developers, regulators, policy makers, and of course, the energy and organization of patient advocacy groups.*"

- Former FDA Commissioner Scott Gottlieb, 11th Annual Rare Disease Day, 2018

Problem

- More than 30 million Americans are living with one or more rare disease.
- Between 93% and 95% of the more than 7,000 known rare diseases have no U.S. Food and Drug Administration-approved therapy.
- The development process for a rare disease drug takes an average of 15 years.
- Traditional regulatory processes have become more complex involving combinations of therapies, genomics, novel diagnostic tests, multi-systemic diseases, small patient populations, and precision medicine. As a result, numerous parts of the regulatory system need to cohesively work together.
- When new therapies for rare diseases are approved, patients often face unnecessary delays and barriers to access, resulting in avoidable deterioration in health.

Solution

- The STAT Act will enact targeted, impactful, and attainable policy reforms at the Food and Drug Administration (FDA) to accelerate development of therapies across the spectrum of rare diseases and disorders and facilitate patient access to such therapies.
- The STAT Act will:
  - ✓ Improve rare disease coordination, stakeholder engagement, and policy development within FDA by expanding existing authority to create a Rare Disease Center of Excellence,
  - ✓ Inform rare disease policies and actions by creating a Rare Disease and Condition Drug Advisory Committee,
  - ✓ Fund regulatory science and related activities to support development of therapies to treat very small rare disease populations, and
  - ✓ Strengthen rare disease patient access to FDA-approved therapies in both public and commercial plans through enhanced FDA and Centers for Medicare and Medicaid Services coordination, proactive engagement of payers, and specific actions intended to strengthen Medicare and Medicaid beneficiary access to novel therapies.
- FDA already has authority under the 21st Century Cures Act to establish Centers of Excellence. The COE would not supplant any authorities held by the FDA review divisions.
- The Rare Disease Center of Excellence would be cross-cutting, capacity-building, and consultative to support review of rare disease applications but would not supplant any authority of the existing Centers.
- Five years ago, the FDA established the first FDA Center of Excellence, focused on oncology, which has been extremely successful in bringing new cancer therapies to patients.

For more information please contact the Rare Disease Caucus Co-Chairs:
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