CHAMBER HILL

INSIGHTS

Senate Aging Committee Hearing on the FDA Regulatory Process for Rare Diseases

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On February 26, 2026, the Senate Special Committee on Aging held a hearing to examine the Food and Drug Administration's (FDA) review process for rare diseases. The witnesses all shared their frustration that the FDA has tools to allow for flexibility in drug approvals, but that these flexibilities are inconsistently applied across the various subagencies. In their view, this has led to confusion and a lack of transparency in the drug approval process. The committee discussed the current FDA landscape, manufacturing concerns, the impact of delays on patients, and possible policy actions. Possible policy actions discussed included improved clinical trial implementation, use of surrogate endpoints, funding for the Rare Disease Innovation Hub, and greater use of advisory committee meetings.

OPENING STATEMENT

- Chairman Rick Scott (R-FL)

WITNESS TESTIMONY

- Annie Kennedy, Chief Mission Officer, EveryLife Foundation for Rare Diseases – Testimony
- Dr. Jeremy Schmahmann, MD, Director, Massachusetts General Hospital Ataxia Center – Testimony
- Bradley Campbell, President and CEO, Amicus Therapeutics – Testimony
- Dr. Cara O'Neill, MD, FAAP, Chief Science Officer, Co-Founder, Cure Sanfilippo Foundation - Testimony

MEMBER DISCUSSION

Current FDA Landscape

Senators on both sides of the aisle expressed interest in how the FDA's current FDA landscape impacts the review process for rare disease treatments. Sen. Ron Johnson (R-WI) was interested in the witness's experiences interacting with the FDA. Dr. Schmahmann shared that he felt a lack of compassion from FDA personnel during meetings and that they did not engage in open dialogue about the science and patient experiences. Dr. O'Neill has had different experiences, sharing that her interactions with regulators have been kind, with them listening to patient experiences. However, Dr. O'Neill has not seen these experiences translate into regulatory action.

Sen. Angela Alsobrooks (D-MD) asked how changes in FDA personnel and guidance have affected trends in the rare disease drug approval process. Ms. Kennedy answered that she is increasingly concerned about application reversals and reports that the FDA has changed requests to sponsors at the last minute. Ms. Kennedy is most concerned about the lack of advisory committee meetings taking place now as compared to a few years ago, as these meetings allow for dialogue between FDA personnel, drug sponsors, and patients.

Sen. Andy Kim (D-NJ) was curious about the manufacturing process for rare-disease treatments. Mr. Campbell explained that many small and mid-sized companies rely on foreign manufacturing due to the time and costs required to build domestic manufacturing facilities. According to Mr. Campbell, it takes an average of 3-5 years to build a facility and an additional 1-2 years to pass FDA inspections before manufacturing can begin. So, while Mr. Campbell is supportive of onshoring drug manufacturing, he noted that the current regulatory environment makes it very difficult.

Impact on Patients

Chairman Rick Scott (R-FL) requested that witnesses share the effects of delayed treatment on patients as well as the ethical implications of stopping compassionate use for drugs. Dr. Schmahmann stated very clearly that delayed treatments lead to increased symptoms and decreased lifespan for patients while also reducing the quality of life for them and their families. Dr. Schmahmann was a strong supporter of compassionate use, sharing that, in his opinion, preventing compassionate use or suggesting trials that would force patients off helpful drugs is very unethical. Dr. O'Neill shared her frustration that the FDA is taking steps to reduce the use of animal models in drug testing due to ethical concerns, while not considering the ethical considerations of not allowing working treatments to be given to children with rare diseases.

Possible Policy Actions

Multiple Senators, including Sen. Dave McCormick (R-PA), Chairman Scott, and Ranking Member Gillibrand, were greatly interested in possible policy actions to better support the drug approval process. Mr. Campbell expressed support for the use of centralized Independent Review Boards (IRBs) and speeding up clinical trial implementation. Mr. Campbell also suggested providing more funding and attention to the Rare Disease Innovation Hub, which could allow for rare disease experts to be trained and deployed across the FDA. Ms. Kennedy felt that allowing greater use of surrogate endpoints and biomarkers would be extremely helpful in centering on patient experience and enabling the approval of treatments for medically complex diseases.

Ranking Member Gillibrand suggested greater use of advisory committee meetings as a path forward. Ms. Kennedy shared that advisory committee meetings are extremely impactful for allowing nuance in regulatory decisions, as they allow for outside experts to be included in decision-making. Dr. O'Neill continued that they allow for dialogue with patients and sponsors.

We trust you found this summary useful. Please reach out to us with any questions.