

Advancing Breakthrough Therapies for Patients Act

Senators Bennet, Hatch & Burr

“...Our job is to ensure the safety and efficacy of FDA-regulated products and to take real steps to foster the scientific innovation that will lead to tomorrow’s new breakthrough products...Innovation is not just about new ideas, but is about making sure that those ideas truly translate into the products and opportunities that people need and count on. Moreover, it’s about changing systems replacing outmoded or insufficient patterns with new, better, more effective ones.”

- FDA Commissioner, Dr. Margaret Hamburg, April 5, 2011

Background

Advances in our understanding of diseases and conditions, including complex diseases such as cancer and HIV/AIDS, along with the sequencing of the human genome have led to major breakthroughs for patients and hold tremendous potential for advancing targeted and more effective therapies. Such new, targeted therapies are being developed for which some patients are highly likely to respond. When preliminary clinical evidence indicates that a drug may demonstrate substantial improvement over existing therapies, the traditional approach to drug development may not be the most appropriate for speeding these promising treatments to the patients, particularly if existing treatment options offer limited efficacy. There is a need to modernize FDA’s pathways to reflect the advances made in better understanding diseases and targeting promising therapies to patients. In order to advance such breakthrough therapies for patients, innovators need the regulatory certainty of a predictable and clear pathway to expedite the collection of necessary safety and effectiveness data while giving patients earlier access to life-saving therapies as efficiently as possible.

Breakthrough Therapy Designation

The Advancing Breakthrough Therapies for Patients Act will encourage and spur innovation on behalf of patients by providing greater regulatory certainty and predictability. This bipartisan legislation amends Section 506 of the Food, Drug, and Cosmetic Act to require FDA, at the request of a drug sponsor, to facilitate the development and expedite the review of the drug if it is intended for a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies. If the FDA determines that a drug meets the criteria to be designated as a “breakthrough therapy” then the agency shall take such actions as are appropriate to expedite the development and review of the drug, including holding meetings with the drug sponsor throughout the drug development process; providing timely advice to the sponsor regarding the plan to develop the drug; enlisting a collaborative, cross-disciplinary approach by senior managers and experienced review staff; and minimizing the number of patients enrolled in trials and shortening the duration of trials, when scientifically appropriate. A designated breakthrough therapy may still seek fast-track product designation, accelerated approval, and priority review. The bill also requires FDA to undertake public awareness efforts to physicians, patient organizations, manufacturers, and others regarding breakthrough therapies.

Regulatory Certainty

In order to promote regulatory certainty with respect to breakthrough therapies, the bill requires FDA to issue final guidance on authorities relating to breakthrough therapies, accelerated approvals, and fast track products, including the actions the Secretary will take to expedite the development and review of breakthrough therapies, including issuing good review management practices for breakthrough therapies.

Transparency and Accountability for Patients and Taxpayers

In order to ensure appropriate transparency and accountability for patients and taxpayers, the bill requests the Secretary to conduct an independent review and report on breakthrough therapies.