



Madame President, I rise to speak today in support of the Food and Drug Administration Safety and Innovation Act.

First, I would like to applaud the hard work of the Senate HELP Committee Chairman Senator Tom Harkin and ranking member Senator Mike Enzi.

This bill is truly one of the most bipartisan efforts I have had the opportunity to be a part of in the three years I have served in the U.S. Senate.

It ought to be a reminder that, yes, when we work together across the aisle, the Senate can still get things done.

I am also particularly proud to support this bill because of what it will mean for patients who are suffering with diseases, who do not have access to adequate treatments or who do not have access to any treatment at all.

This bill we are voting on includes key provisions of the TREAT Act, or the Transforming the Regulatory Environment to Accelerate Access to Treatments Act, which I introduced in February.

These important provisions will expedite the review of treatments for serious or life-threatening diseases, without compromising the FDA's already high standards for safety and effectiveness.

I introduced the TREAT Act after meeting with a family whose child suffered from Spinal Muscular Atrophy, or SMA. SMA is an incurable neuromuscular disease and is the leading genetic cause of infant deaths.

Of course, that family was not alone.

There are 30 million Americans suffering from rare diseases, and I have had the honor to meet a number of them. Their stories are both heartbreaking and inspiring.

When I visited the North Carolina Children's Hospital earlier this month, I met with Meagan and Jarrod Hendren of Lumberton, North Carolina, whose 13-month old twins, Logan and Lucas, suffer from Gaucher's (**go-sheyz**) disease. Gaucher's disease is a painful and potentially debilitating metabolic disorder for which there is currently no cure.

I also met with 8-year-old Ashley Burnette of Raleigh, who is resilient and wise beyond her years, but who is suffering from Neuroblastoma.

For patients like these suffering from rare diseases for which there are **no** approved medications, medical advances can't come fast enough.

Of the 7,000 known rare diseases, fewer than 250 currently have FDA-approved therapies. The provisions of the TREAT Act that have been included in this bill take great steps toward resolving that problem.

There is currently a pathway at the FDA to expedite the review of drugs for illnesses that are serious or life-threatening and for

which there is no adequate treatment. This is called the “accelerated approval pathway.”

Since the early 1990’s, it has been successfully used to advance treatments for patients with HIV and cancer by leaps and bounds.

However, it has **not** been applied regularly or consistently to the review of drugs to treat other diseases.

This inconsistency is **why** I introduced the TREAT Act.

My bill will broaden the application of the accelerated approval pathway beyond HIV/AIDS and cancer to a wider range of diseases, with a particular focus on rare diseases.

That’s why my proposal enjoys broad support from patient advocates, including the National Organization of Rare Diseases, Us Against Alzheimers, Parkinson’s Action Network, the Huntington’s Disease Society of America, and many more.

By providing for **consistent** application, we will help the FDA implement these provisions, assist drug sponsors navigate the approval process, and hopefully bring safe and effective treatments more rapidly to the patients that need them.

I am also very proud to have played a critical role in the legislation that led to the negotiation of the first biosimilars user fee agreement, which is included in the bill before us today.

Last Congress, we passed the Biologics Price Competition and Innovation Act to facilitate the introduction of lower cost alternatives to biologic drugs while ensuring continued research

and development into innovative biologics, which could save or improve the lives of millions of Americans.

The user fees negotiated by the industry and FDA will provide the necessary funding for the review of these critical therapies.

The biosimilars industry is in the earliest stages of development, and the Biosimilars User Fee Agreement will help facilitate the industry's growth.

In addition, the FDA Safety and Innovation Act provides the necessary regulatory updates to keep pace with the rapid innovations of the biopharmaceutical industry.

This is imperative for creating jobs in states like North Carolina and maintaining America's competitive edge in the global economy.

Companies with footprints in North Carolina are partnering with our world-class universities to improve the health of people all across the globe everyday by researching, discovering, and developing life-saving treatments for those suffering from devastating diseases.

We must pass the FDA Safety and Innovation Act for states like North Carolina and for our nation to remain global leaders and to attract the jobs of the future.

The American public also expects the FDA to be the world's gold standard when it comes to ensuring the safety and integrity of our drug supply.

By sending the FDA Safety and Innovation Act to the President's desk, we will establish a clear and effective pathway for turning **ideas into cures**, and **cures into treatments**. And, we'll have shown the foresight and flexibility required to maintain our country's position at the top of the medical treatment and device industries.

I urge my colleagues to join me in supporting the FDA Safety and Innovation Act.

Madame President, I yield the floor.