Opening Statement for the Honorable Brett Guthrie

Check Up: Examining FDA Regulation of Drugs, Biologics, and Devices

May 22, 2024

Today, three important center directors at the U.S. Food and Drug Administration are here with us to share updates about the work they oversee within their respective divisions.

With the agency now collecting the highest number of user fees on record, it is critical we hear from the center directors about the ongoing challenges the agency and industry face in getting safe and effective products to patients faster.

In addition to reauthorizing the user fee agreements, Congress granted the FDA a suite of additional authorities that were signed into law in December 2022. These include updates to ensure clinical trials are reflective of the broader patient population, greater transparency for sponsors to collaborate with payers to decrease the time from a novel product's approval to coverage, reforms to strengthen the accelerated approval pathway, and streamlined processes for manufacturers to update their software without unnecessary regulatory hurdles.

Health Subcommittee Helps Lead the Way to New Product Approvals

I am proud of the bipartisan work this subcommittee has accomplished throughout the last user fee reauthorization. Since then, the FDA has approved and cleared hundreds of new products. Our work collectively enabled the review and approval of 55 novel drugs in 2023 and the approval of almost 1,000 generic drug applications. During that same time, there were 23 biologics device applications and 20 biologics license applications approved and over 100 novel devices cleared by the FDA.

Cell and gene therapy is of particular importance and interest to me and others in Congress. We're at the precipice of a renaissance in health care, in which personalized medicine can cure otherwise incurable diseases. For example, among those medicines approved in 2023 were two cutting-edge, potentially curative, gene therapies for sickle-cell disease. Just last summer, CBER also approved a therapy to address the underlying cause of disease in Duchenne Muscular Dystrophy in boys aged 4 to 5. Both examples illustrate the incredible value American innovation plays in our health care system and the value the FDA brings to help facilitate this remarkable work.

That being said, I have some concerns I hope to have addressed today that threaten to undermine the strides we've made:

Biden Administration's Missteps Cause Questions to the Accelerated Approval Pathway

Missteps by the Biden administration have already caused uncertainty amongst innovators in these small biotechnology companies that could impede patient's ability to access innovative products. The repeated attacks on the Accelerated Approval pathway – from CMS second-guessing FDA's equities to academics and payers alike, insisting that these approvals are unproven or lesser than traditional review pathways – degrades public trust in an important tool the agency has to help safely and effectively get cures to patients more quickly. This is also a pathway that Congress, a bipartisan Congress, has repeatedly strengthened and supported.

The recently released, 500-page Laboratory Developed Test, or LDT, rule has been touted as a mechanism to drive more innovation and protect patient safety. I fear this complete overhaul of LDT regulation will have the opposite effect – instead driving up the costs of care and delaying patient access to life-saving care. Tests developed to treat patients at the bedside, to detect early stage cancer, or detect Alzheimer's sooner would be subject to onerous requirements under this new regime proposed by CDRH. The FDA must work with Congress on a long-term solution that balances patient safety and facilitates future innovation.

Bipartisan Concerns with Clinical Holds

Last year, Ranking Member Eshoo and I wrote to Dr. Marks about clinical holds. According to the Wall Street Journal, there were an average of 664 clinical holds, which temporarily stop clinical research, between 2017 and 2021, up from 557 average annual holds in prior years. In response to our inquiry, we learned that 79% of the responses to innovators' questions about their applications and the holds associated with these applications were written-only responses.

To be clear, I understand the complex nature of these applications, but providing written responses to complex questions with no chance for a true dialogue is unacceptable. It is imperative for the agency to come to the table and work through these issues with innovators or else patients will be left without answers and without life-saving care.

In closing, I want to thank the witnesses for being here with us today. I know your work is not easy, I know that your job is to ensure that we have safe and effective medications. But, we must know that patients are counting sometimes months, if you're a parent with a child with Duchenne Muscular Dystrophy, the race to keep them out of a wheelchair before they get access to medicines is real. It's important accelerated pathways is real and important, and it's been bipartisan and we want to work together. That's our plan to work together to bring these innovative technologies to market.

I yield back.