February 26th, 2026

Ranking Member Kirsten Gillibrand's Opening Statement
"From Regulator to Roadblock: How FDA Bureaucracy Stifles Innovation"

Thank you, Chairman Scott. I really appreciate you calling today's hearing. Thank you to our witnesses and the advocates who are here for Rare Disease Day on the Hill. It makes a big difference that you come together to make sure your loved ones are being heard and that the challenges and struggles that you go through as families and supporters are being understood by lawmakers.

Every one of us in this room today knows that when we have a friend, or a loved one, or a family member who has a rare disease, that the most important thing is finding a cure, getting treatment, and making sure they survive.

A disease is considered rare if it affects fewer than 200,000 people, but rare diseases actually aren't that rare. 1 in 10 Americans is living with a rare disease, and as you know, many of these patients face substantial unmet medical needs.

Because it can be extremely difficult and expensive for companies to develop these treatments and the FDA to evaluate them, Congress has provided the agency with significant regulatory flexibility to encourage both biotech innovation and rare disease therapies. These include the

authorization of accelerated approval pathways to speed up the drug review, establishing programs like the Rare Disease Endpoint Advancement Pilot to bolster novel endpoint development, and strengthening and expanding the use of patient experience data and real-world evidence in drug reviews.

These mechanisms are designed to help improve access to novel treatments for our patients, and they are supposed to provide drug sponsors with a predictable and consistent approach to addressing regulatory science challenges that are unique in rare disease therapy development and review, like designing complex clinical trials, developing appropriate endpoints, and using real world evidence. But it is not working how it should be.

FDA's approach, transparency, and flexibility varies widely between its offices, divisions, and centers. We have seen a pattern of hesitation to use authorized flexibilities, limited communication with drug sponsors, failure to incorporate patient experience in real world evidence reviews, and FDA shifting its regulatory position on trial design at the last minute, rejecting drug applications, and requiring new clinical trials the sponsor may be unable to perform. This is heartbreaking for patients, and it is why we can't afford delays and disruptions in treatment. Rare diseases can progress rapidly, cause irreversible harm, and in some cases premature death.

This is also frustrating for drug sponsors who face increased costs and delayed timelines that impact the viability of their clinical trials, particularly in the United States. Uncertainty shapes behavior across the biotech ecosystem. Without consistency and predictability, drug sponsors will continue to struggle with seeking FDA approval and will take their clinical trials elsewhere, like China.

This is bad for business, and it is bad for patients. If we want the U.S. to remain the global leader in biotech and we want American patients to have access to these novel treatments, things need to change. Congress must hold the FDA accountable. We must make sure FDA fixes its inconsistent and unpredictable application of regulatory flexibility. It is essential for supporting innovation, while upholding the highest standard for safety and efficacy in the approval of these rare disease drugs.

FDA appears to be moving in the right direction, with the Rare Disease Evidence Principles, the Rare Diseases Innovation Hub, proposing new pathways for approval and trying to hire more reviewers. But it doesn't matter what agency leadership puts in a press release. It is about execution and implementation across all levels of that agency. Consistency from top to bottom.

Congress will ensure that happens by conducting oversight, encouraging application of authorized flexibilities, and providing adequate resources to restore agency capacity.

I look forward to hearing from our witnesses and working with this committee to hold the FDA accountable because rare disease patients cannot wait.