Opening Remarks
Chair Brett Guthrie
Health Subcommittee Legislative Hearing:
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As Prepared for Delivery

Thank you to our witnesses for being here today to discuss such an important topic. The legislation before us will take an important step in helping to support rare disease patients.

The Orphan Drug Act defines a rare disease as a disease that affects less than 200,000 patients across the United States. According to the National Institutes of health (NIH), there are over 10,000 known diseases that fit this definition, impacting as many as 30 million Americans. Despite 10% of the U.S. population living with a rare disease, about 95% of these diseases lack treatments. Most patients can't even begin to think about treatments until they are diagnosed, which can be a long and costly journey, only to discover there are limited treatment options, if any.

Research and development into therapies designed to treat rare diseases is challenging, especially when it comes to measuring the safety and efficacy of drugs for a smaller patient population. These dynamics can make investments in rare disease therapies and treatments risky and unpredictable.

Recognizing these hurdles, Congress has taken numerous steps over the years to better support every step along the drug approval pipeline, from supporting basic research for rare diseases, to improving the process by which drugs seek and receive FDA approval for rare disease indications, to ensuring that payers like Medicare and Medicaid will cover these treatments when they come to market.

For example, Congress authorized the pediatric rare disease priority review voucher program at FDA almost a decade ago, to bolster existing incentives created under the Orphan Drug Act. The priority review voucher reduces the financial risk for innovators to obtain resources needed to conduct critical rare disease research, which ultimately helps patients access therapies or treatments more quickly.

That's why reauthorizing the pediatric rare disease priority review voucher program through H.R. 7384, the Creating Hope Reauthorization Act, is so important. Since its inception, almost 50 priority review vouchers have been granted that have paved the way for groundbreaking therapies that may otherwise not have made it to patients. I would like to thank my colleagues on the committee, Representatives Bilirakis, Eshoo, Burgess, Barragan and Trahan, for their leadership on this issue and encourage my colleagues to strongly support the passage of the bill.

We are also considering bipartisan solutions to ensure access to treatments for rare disease patients by promoting certainty and consistency through the regulatory and reimbursement process. These are key considerations as Congress recognizes the need for rare disease patients to have access to treatments and cures.

First, we have the Accelerating Kids Access to Care Act, led by Representative Miller-Meeks, which will streamline care for kids in Medicaid by making it easier for them to receive necessary care by a provider in another state. This is especially a concern for children who have to travel out of state to centers of excellence to get care for rare diseases and have to jump through bureaucratic hoops just to get the care that they need.

Next is my discussion draft, the Patient Access Act, which removes burdensome regulations that make it harder for patients to access life-saving therapies. In some cases, patients and their families must travel significant distances to receive therapies that also require lengthy stays, leaving the patient and their families responsible for hotels, food, and other important expenses while they miss work and other obligations. My bill would amend Antikickback Statute by permitting manufacturers to pay for these incidentals in very limited circumstances to make accessing these critical therapies easier for patients and their families.

These bills today build off this Committee's important work from this Congress to ensure that all patients can access the care that they need like our work in passing the Protecting Health Care for All Patients Act, which eliminates discriminatory barriers for patients with disabilities to access life-saving care, and my bill, the MVP Act, which provides access to curative cell-and-gene therapies for Medicaid patients.

In closing, while none of these bills serve as a silver bullet to solving all of the challenging problems faced by rare disease patients, they are important steps and will make a meaningful difference in the lives of the millions of Americans living with rare diseases. I thank my colleagues on the committee for leading on the important bills we're examining today.

Thank you, I yield back.