Opening Remarks
Chair Cathy McMorris Rodgers
Health Subcommittee Legislative Hearing:
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As Prepared for Delivery

INTRODUCTION:

Good morning.

I'm glad we're gathered on Rare Disease Day to examine legislation to help move forward efforts to promote innovation for people with rare diseases...

...and make sure all patients can benefit from all the exciting innovation that is happening.

We're going to take action for patients, like Hunter Davis, a 12-year-old from Texas with Spinal Muscular Atrophy Type 1, whose mother, Khrystal, is here today.

There are over 10,000 known rare diseases affecting an estimated 30 million Americans, like Hunter.

However, only about 500 of these diseases have FDA-approved treatments.

But now more than ever, there is increasing hope with new genetically targeted technologies, cell and gene therapies, and many more innovations being researched and developed...

...some of which we will hear about today.

FDA BILLS

We've made progress on fostering innovation to find rare disease treatments, but there's still a lot of work to be done.

Prior to the passage of the Orphan Drug Act just over forty years ago, only 38 orphan drugs were FDA approved.

Compare that to 2023 alone, when 40 novel, orphan-designated drugs and biologics were approved by the FDA, many of them potentially curative.

Our job is to make sure the FDA is ready, and the market continues to foster innovation that leads to treatments and cures for patients.

I am concerned that if we don't continue to encourage investment in rare disease efforts, we will be harming the potential for the development of life-changing treatments.

Many of the bills before us seek to provide the regulatory clarity necessary to ensure that novel therapies... and in some cases, cures... continue to become accessible to patients as rapidly as possible.

As we work to carry out that mission, we must carefully examine all of the legislation before us today...

...to ensure it doesn't have the opposite effect and stifle innovation.

MEDICAID AND AKS BILLS

Additionally, we need to make sure that once drugs are approved that patients can actually access them.

One issue that we'll discuss today is how treatment options for certain diseases are often concentrated at or limited to centers of excellence.

In many instances, access may come down to whether patients can afford to travel across the country...

...or stay in a different city for weeks at a time to receive the lifesaving care that they need.

Congressman Guthrie's draft Patient Access Act would help make these costs more manageable so that patients, and in the case of a child, a parent, can travel to get the care that they need by allowing for drug manufacturers to directly support patients' incidental costs for travel.

Meanwhile, Congresswoman Miller-Meeks's Accelerating Kids Access to Care Act would cut red tape that restricts a pediatrician's ability to get paid by Medicaid to treat kids who are enrolled in a different state's Medicaid program.

IRA BILLS

There are also three bills we'll discuss today that amend the Inflation Reduction Act's drug pricing scheme.

While I recognize members of this committee have differing opinions on the best way to ensure Americans have access to innovative, lifesaving cures, I hope we can set some of those broader disagreements aside and focus on what's best for rare disease patients.

Because the process that led to the IRA's drug price setting scheme was so rushed, I don't think everyone fully understood how some changes could have devastating impacts on the rare disease community.

Last Congress, after rejecting H.R. 3 with bipartisan opposition, this Committee did not get the opportunity to explore the potential consequences of the IRA's new scheme...

...which was seemingly conceived entirely through Senate backroom deals and made public just hours before it was brought up for a vote.

I am hopeful that today we do what this committee is known for and work in a bipartisan way to address some of the consequences that have come to light since its passage.

These three bipartisan bills before us today are a first step to doing so.

CLOSING

This committee has a long history of working together to support innovation, including things like the 21st Century Cures Act and multiple FDA user fee reauthorizations. We must continue to build on this work, and that is exactly what we are doing today.

I look forward to learning what more we can do from our witnesses and finding where the committee can move forward with bipartisan legislation to help families all across this country.

I yield back.