Committee on Energy and Commerce
Opening Statement as Prepared for Delivery
of
Subcommittee on Health Ranking Member Anna G. Eshoo

Hearing on "Legislative Proposals to Support Patients with Rare Diseases"

February 29, 2024

Thank you, Mr. Chairman and good morning colleagues and all of our guests in our hearing room. Today, on Rare Disease Day, we're considering several bills to help the one in ten Americans living with a rare disease.

In the last 40 years, there's been a revolution in the development of drugs to treat rare disease – also known as orphan drugs. FDA has approved more than 5,000 orphan drugs since passage of the Orphan Drug Act of 1983.

Last year, more than half of all new drugs approved by the FDA were orphan drugs, bringing hope to millions of Americans.

There's room for improvement. Only five percent of the more than 7,000 known rare diseases have an FDA-approved treatment, and clinical trials don't reflect the diversity of our nation. Among the most neglected are children. Few FDA-approved treatments for rare diseases have been tested to be used in children.

Children aren't little adults, something Congress recognized 20 years ago when it passed my Best Pharmaceuticals for Children Act and my Pediatric Research Equity Act to reward and require pediatric studies.

There's more Congress can do to ensure children aren't left behind. 36 percent of drugs approved for rare diseases relevant to children since 1999 lack some or all pediatric data. The Innovation in Pediatric Drugs Act which I introduced with Rep. McCaul will close the loopholes so that we have the clinical data we need to safely treat children with new cures. The American Academy of Pediatrics, the Leukemia & Lymphoma Society, Children's Cancer Cause, the Children's Hospital Association, Stanford Medicine Children's Health, the National Organization for Rare Disorders, and the Alliance for Childhood Cancer endorse this bill, and I thank all those organizations.

Children should also benefit from advancements in cancer treatments which is why Rep. McCaul and I introduced the Give Kids a Chance Act. Currently, the FDA cannot direct clinical trials to test combinations of drugs in children despite combination therapies proving effective in adults. Our legislation gives FDA that authority. The legislation has 188 bipartisan cosponsors and has been endorsed by 50 organizations and research consortiums. Every Member of this Committee voted for this legislation as part of the FDA user fee legislation last Congress.

Finally, I joined Reps. McCaul, Burgess, Barragán, Bilirakis and Trahan in introducing the Creating Hope Reauthorization Act to incentivize research by providing pediatric drug developers with vouchers to speed FDA reviews of new drug products. The Pediatric Priority Review Voucher Program can make the difference between whether a drug comes to market or not. More than 114 organizations endorsed this bill and the last time this legislation was up for reauthorization, it passed the House unanimously by a voice vote, highly instructive to all of us.

Our Subcommittee is also considering three bills to undermine Medicare's historic new power to negotiate lower prices for drugs. Thanks to the new law, Medicare beneficiaries are saving money through free vaccines, insulin capped at $35 per month, and some beneficiaries' out-of-pocket prescription drug costs capped at about $3,500 per year. Next year, all beneficiaries will have their Part D costs capped at $2,000 per year.

There are bills before us today that unfortunately will weaken that process. They're also unneeded. As we all know, Medicare drug price negotiation is only focused on the top-selling, high-cost Medicare drugs without any market competition.

The bills before us today attempt to create unneeded loopholes to chip away at Medicare's power to negotiate and will raise costs for beneficiaries and taxpayers. I think that they are ill-advised and something I cannot support.

So, I look forward to today's hearing Mr. Chairman, thank you for calling it and I yield back.