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THE VOICE FOR HEALTH CARE CONSUMERS

Statement for the Record

House Energy and Commerce Subcommittee on Health

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

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Chair McMorris Rodgers, Health Subcommittee Chair Guthrie, Ranking Member Pallone, and Health Subcommittee Ranking Member Eshoo, thank you for holding this hearing and the important discussion around opportunities to improve research, treatment options, and access to care for people dealing with rare diseases. More than 30 million people in the United States live with a rare disease, many of whom experience life-threatening conditions with limited options for effective treatment.[i] The breadth of bipartisan bills considered in today's hearing is a testament to the work of this committee in prioritizing the unique health needs of people living with rare diseases who desperately need new and innovative treatments and medications to maintain and improve their health.

Specifically, we appreciate the opportunity to discuss the importance of access to prescription drugs and the ongoing affordability crisis that many families face when trying to obtain lifesaving and sustaining medications. Currently, 60% of U.S. adults take at least one prescription medication and 25% take four or more.[ii] Over the past 15 years, launch prices – the initial prices of drugs set by manufacturers – grew more than 20% each year.[iii] And even after launching, prices continue to increase at staggering rates leaving families and individuals paying more and more, year after year for their needed medications. For example, the price of Victoza (a popular diabetes and weight loss medication launched in 2010) increased a staggering 42% in just five years, rising from $7,936 per year in 2015 to $11,300 per year in 2020.[iv] This is a crisis that demands comprehensive solutions.

One of the most important steps towards addressing the high cost of drugs was passage of the *Inflation Reduction Act of 2022* (IRA), which included critical reforms such as giving Medicare the authority to negotiate for fair drug prices. The administration is currently working to implement those reforms faithfully, the full impact of which will be felt by patients and their families when negotiated prices go into effect for the first ten drugs in 2026. **Families USA has concerns that some of the legislation discussed in today's hearing would create unnecessary delays or carve-outs from IRA Medicare negotiation, in turn allowing big drug companies to continue price gouging families at the expense of their access to lifesaving and sustaining medication.**

<u>Legislation to lengthen exclusivity period before negotiation</u>

Currently, the IRA allows for drugs to become eligible for Medicare negotiation after a set length of time on the market: Small molecule drugs are eligible after being on the market for 7 years whereas biologics can only be eligible after they have been on the market for 11 years.[v] These time periods are longer for some types of drugs than the periods of exclusivity granted by federal bodies like the Food and Drug Administration, which grants exclusivity periods of 5 and 12 years (for small molecules and biologics respectively).[vi] The IRA ensures that drug companies are given an ample window of time to cover the costs of research and development (R&D) before their drugs are eligible for negotiation.

Yet, some of the proposed legislation, including H.R. 5539 *Optimizing Research Progress Hope and New (ORPHAN) Cures Act* and H.R. 5547 *Maintaining Investments in New Innovation (MINI) Act,* would lengthen the time a drug must be on the market prior to being eligible for Medicare price negotiation. Families USA has concerns that this kind of change would further limit the number of drugs eligible for negotiation to an even more narrow list, while allowing drug companies to continue abusing market exclusivity to drive up costs and unreasonably high profit margins.

Time and again, drug companies have shown that it is easier and more profitable for them to abuse their market and patent exclusivity privileges through tactics like pay-for-delay schemes and patent thickets

as a way to limit competition and raise prices rather than investing in new and innovative treatments that help people live longer, healthier lives.[vii] Congress should not allow the IRA, a law that will bring relief to millions of Americans who need affordable, accessible medication, to become another pawn in drug companies' gaming of the prescription drug market.

Legislation to exclude orphan drugs from Medicare drug negotiation

In order to ensure drug companies are incentivized to invest in R&D on innovative drugs for rare diseases, the IRA excludes rare drugs with a single designation for a rare disease or condition from being included in the Medicare drug negotiation program.[viii] These "orphan drugs," which are drugs that treat small populations of people with rare diseases or conditions, can provide lifelines for people with very limited treatment options.[ix] Drug companies also receive significant additional supports for investment in orphan drug development, including market and patent exclusivity, expedited access to markets, as well as 25% tax credits on qualified clinical trials.[x]

Families USA has concerns that attempts to further exempt orphan drugs from Medicare price negotiation under the IRA will allow drug companies to continue to move the goal posts for some of the drugs where price negotiation is most needed to ensure patient affordability. **H.R. 5539, *Optimizing Research Progress Hope and New (ORPHAN) Cures Act*, which would provide further orphan drug exemption from Medicare drug negotiation, unnecessarily creates further exemptions in the law for big drug companies.**

We need companies to invest in the development of new therapies that give new hope to people with limited or no treatment options, but those people also need to be able to afford the resulting medication in order to benefit from it. It is critical to strike a fair balance between drug innovation and affordability – orphan drugs are often some of the more expensive drugs on the market and their prices are rapidly growing. Drugs that treat rare diseases are *25 times more expensive* than non-orphan drugs and in 2017 the average annual cost for an orphan drug was $186,758.[xi]

Orphan drugs are quickly becoming a larger share of the drug market. In 2023, 43% of new drugs received orphan drug indications.[xii] From 1990 to 2022, 491 novel orphan drugs received approval, 15% of which have been approved for multiple conditions.[xiii] Adding additional types of orphan drugs to the list of exempt drugs under the proposed legislation would further chip away at the list of potential drugs eligible for negotiation. If Congress continues to add additional exclusions, particularly for growing portions of the drug market, the ability of the program to actually provide savings for millions of people who rely on Medicare winnows.

Orphan drugs are the prime example of why Medicare negotiation is so needed. Companies are charging exorbitantly high prices, even though they have benefited from significant financial incentives to research and create the drug – and the people who rely on them for chronic health conditions and all taxpayers that who contribute to the Medicare program are paying the price. The financial relief provided by Medicare negotiation would be most keenly felt by those with rare diseases. Conversely, exempting additional medications would be rewarding the predatory, profiteering behavior that necessitated creation of the Medicare negotiation program in the first place.

Conclusion

Affordability of a medication is at the heart of accessibility. If a person is forced to choose between filling their prescription and filling their fridge, or is skipping, taking over-the-counter medications instead of their prescription, or not filling their prescriptions at all because of cost – as almost 30% of adults in the U.S. taking medications do every year – then that treatment is not accessible to them.[xiv]

At a time when millions of families need relief from high costs, specifically in health care, Congress should focus on legislation that would bring costs down. Unfortunately, many of the proposed bills discussed in today's hearing not only fail to bring down costs, but further allow big drug companies to abuse their market exclusivity and perpetuate false arguments around research and development costs, all of which contributed to creating such high drug prices in the first place.

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