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ONE HUNDRED EIGHTEENTH CONGRESS

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April 18, 2024

Dr. Alice Chen, Ph.D.
Associate Professor and Vice Dean for Research
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Verna and Peter Dauterive Hall 414M
635 Downey Way
Los Angeles, CA 90089-3333

Dear Dr. Chen:

Thank you for appearing before the Subcommittee on Health on Thursday, February 29, 2024, to testify at the hearing entitled "Legislative Proposals to Support Patients with Rare Diseases."

Pursuant to the Rules of the Committee on Energy and Commerce, the hearing record remains open for ten business days to permit Members to submit additional questions for the record, which are attached. The format of your responses to these questions should be as follows: (1) the name of the Member whose question you are addressing, (2) the complete text of the question you are addressing in bold, and (3) your answer to that question in plain text.

To facilitate the printing of the hearing record, please respond to these questions with a transmittal letter by the close of business on Thursday, May 2, 2024. Your responses should be mailed to Emma Schultheis, Legislative Clerk, Committee on Energy and Commerce, 2125 Rayburn House Office Building, Washington, DC 20515 and e-mailed in Word format to Emma.Schultheis@mail.house.gov.

Thank you again for your time and effort preparing and delivering testimony before the Subcommittee.

Sincerely,

Brett Guthrie
Chair
Subcommittee on Health

cc: Anna Eshoo, Ranking Member, Subcommittee on Health

Attachment

Attachment — Additional Questions for the Record

The Honorable Robert Latta

1. The Maintaining Investments in New Innovation Act is intended to provide a clarification on the definition of an advanced drug product. 21st Century CURES recognized the tremendous potential of genetically targeted medicines, many of which represent the only hope for patients with rare and ultra rare genetic conditions. By targeting specific genetics these innovative, accurate and narrowly targeted medicines are able to deliver benefits of improved quality and extension of life. Would you please share how rare these conditions are and why they should be considered an advanced drug product and treated as such under the law?

2. Can you comment on how existing policies enacted by the Democrats – namely IRA's "Medicaid best price rule" and the American Rescue Plan allow Medicaid rebates to exceed the cost of actually manufacturing the drug – hinders the development of next generation treatments and cures?

3. Recent analyses done by independent policy researchers, such as Kaiser Family Foundation, Avalere Health, and Milliman highlight significant disruption in the Medicare Part D market for 2024 as a result of policies in the Inflation Reduction Act (IRA) being implemented. In 2024, the estimated average enrollment-weighted monthly patient premium for Medicare Part D stand-alone drug plans (PDPs) is projected to increase 21% from 2023, and Medicare beneficiaries have access to the lowest number of standalone PDP options and low-income subsidy benchmark plan options in 2024 than any other year since the Part D program started. Further, there are anecdotal reports that suggest that as Part D plans grapple with the plan liability shifts of the IRA, they are increasingly moving towards use of more coinsurance and narrowing formularies for patients. Dr. Chen, how will these Part D benefit trends that we are seeing in 2024, as a result of the IRA, impact access to the therapies that rare disease patients in my district and across the country need to enhance their health outcomes and live more full lives?

The Honorable Gus Bilirakis

1. There is a tremendous and undeniable unmet need for children living with rare diseases. Of the 30 million Americans living with a rare disease, half are children - and a third of those children won't live to see their fifth birthday. According to a recently-published FDA study reflecting on the first ten years of the program, "Continued support of product development for children with rare diseases is needed to find treatments for all children with unmet needs." Since the implementation of the Rare Pediatric Disease Priority Review Voucher (RPD PRV) program in 2012, nearly 50 therapies for rare pediatric diseases have reached patients that may otherwise have no available FDA-approved treatment. Over half of RPD PRVs were granted in the last 5 years alone, but an often-cited GAO study only accounts for PRVs granted prior to 2019. Based on the latest available data, can you provide updated information on the anticipated short and long-

term impacts that the continuation of the RPD PRV program will have on investment in research and development in the rare pediatric disease space?

2. Your testimony mentions the need for incentives to be in place to invest in the development of rare disease treatments. I'm proud to be a co-lead on H.R. 7384, the Creating Hope Reauthorization Act, which extends the RPD PRV program at FDA for another four years. Can you explain why streamlining regulatory process at FDA to shorten review time can provide key incentives needed to invest in new research & development opportunities?

3. One critique we've heard is the FDA faces an influx of PRV applications as the deadline approaches. Do you believe we should make this voucher program permanent?

4. What are the challenges associated with rare pediatric clinical trials, and how do PRVs help alleviate those challenges?

5. Given the length of time it takes to develop a new drug, particularly within rare disease development, can you provide an economist's perspective on what the unintended consequences would be of losing a key incentive such as the PRV program?

6. If the PRV program is not reauthorized, what other ways can Congress incentivize investment in rare diseases with high unmet medical need?

7. How has rare disease therapy development become more difficult in recent years, and why do incentives matter more for rare disease companies dealing with small patient populations?

8. What other steps can be taken to help streamline FDA's regulatory processes to ensure there are consistent pathways for companies looking to develop rare disease treatments?

The Honorable Earl "Buddy" Carter

1. Antimicrobial Resistance continues to be a growing health crisis in our nation and around the world. It is paramount that we continue to marshal the resources of the federal government while supporting the innovation occurring in the private sector to slow the spread of resistance, ensure the development of novel antibiotics to treat these deadly superbugs, and educate the public on this ongoing threat. I commend Mr. Griffith for his bill requiring a GAO report regarding federal efforts on antimicrobial resistance. Addressing AMR is a top priority of mine, which is why I am a cosponsor of the PASTEUR Act, which is so needed to fix the failing market for antimicrobials. Dr. Chen, in your testimony you mention the importance of antibiotics in addition to other medical innovations that play a pivotal role in improving health outcomes for patients. Can you please discuss the importance of tackling antimicrobial resistance in order to maintain the improved outcomes we have become so accustomed to?