CATHY McMORRIS RODGERS, WASHINGTON
CHAIR

FRANK PALLONE, JR., NEW JERSEY
RANKING MEMBER

ONE HUNDRED EIGHTEENTH CONGRESS

Congress of the United States
House of Representatives
COMMITTEE ON ENERGY AND COMMERCE
2125 RAYBURN HOUSE OFFICE BUILDING
WASHINGTON, DC 20515-6115
Majority (202) 225-3641
Minority (202) 225-2927

April 18, 2024

Ms. Khrystal Davis, J.D.
Founding President
Texas Rare Alliance
3575 Far West Boulevard, #27892
Austin, TX 78731

Dear Ms. Davis:

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. As you know firsthand, glioblastoma is one of the most complex, deadly, and treatment-resistant cancers, accounting for 48 percent of all primary brain tumors, and has had virtually unchanged survival and mortality statistics for decades. The National Cancer Institute's (NCI) Glioblastoma Therapeutics Network, a multi-hospital consortium exists to accelerate research on glioblastoma through pre-clinical drug vetting followed by starting early phase clinical trials. As a rare disease advocate, how important is it that the National Cancer Institute sufficiently fund early phase clinical trials that are essential to establishing safety and early signs of efficacy for promising treatments for glioblastoma?

The Honorable Gus Bilirakis

1. As a Co-Chair of the Congressional Rare Disease Caucus, I know the challenges that rare disease patients and their caregivers face daily. I appreciate your advocacy on behalf of the rare disease community and reading about Hunter's SMA story. I worry that without incentives for rare disease companies to invest in research and development, treatments such as the one that Hunter received would not have ultimately come onto the market. Can you explain why incentives are critical to spurring innovation of new treatments?

2. What would the negative impact on these patients be if there is no longer an incentive for companies to invest resources in rare disease therapies?

3. Can you expand on how investments in rare disease research and development has positive effects elsewhere in the health care sector?

4. How does the IRA disincentivize this type of research from continuing, and what do you believe the effects would be on the patient community as a result?

5. How does repurposing existing rare disease treatments help provide meaningful options for patients, and can policies such as the PROTECT Rare Act help streamline these options?

6. Can you discuss the need for FDA to incorporate real-world evidence and patient experience data into account, which is a requirement of the BENEFIT Act, in their benefit-risk assessment?

The Honorable Mariannette Miller-Meeks, M.D.

1. Small-molecule medicines have the benefit of being able to reach therapeutic targets inside of cells, cross the blood-brain barrier, reduce barriers to treatment adherence and help reduce health disparities, elements that are critical for treating rare diseases.

However, the IRA discourages R&D into this form of medicine by limiting time on the market to just nine years before they could be subject to price controls. Rather than put hurdles in the path of such development, shouldn't we be passing legislation like the MINI Act to encourage this type of innovation?