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ONE HUNDRED EIGHTEENTH CONGRESS

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

April 18, 2024

Dr. Aaron Kesselheim, M.D., J.D., M.P.H.
Professor of Medicine, Harvard Medical School
Director, Program On Regulation, Therapeutics And Law (PORTAL)
Division of Pharmacoepidemiology and Pharmacoeconomics
Brigham and Women's Hospital
1620 Tremont Street, Suite 3030
Boston, MA 02120

Dear Dr. Kesselheim:

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 Anna Eshoo

1. As of early 2021, the FDA issued 123 noncompliance letters to companies for not completing pediatric drug studies that are legally required under the Pediatric Research Equity Act (PREA). Two-thirds of non-compliant studies remain unresolved today. While the FDA has authority to fine companies that don't complete required studies for adults, the FDA doesn't have that same authority to ensure pediatric studies are completed. I introduced the Innovation in Pediatric Drugs Act to give FDA that authority and require drugs for rare diseases to be studied in children.
    a. If non-cancer orphan drugs are made subject to PREA, in your view, would PREA label changes for these drugs be considered to be an additional "approved indication" for the purposes of section 1191(e)(3)(A) of the Social Security Act?