June 14, 2023

The Honorable Brett Guthrie
Chairman
Energy and Commerce Committee
Subcommittee on Health
Washington, D.C. 20515

The Honorable Anna Eshoo
Ranking Member
Energy and Commerce Committee
Subcommittee on Health
Washington, D.C. 20515

Dear Chairman Guthrie and Ranking Member Eshoo:

The Healthcare Leadership Council (HLC) appreciates the opportunity to provide comments in advance of your hearing, "Examining Proposals that Provide Access to Care for Patients and Support Research for Rare Diseases."

HLC is a coalition of chief executives from all disciplines within American healthcare. It is the exclusive forum for the nation's healthcare leaders to jointly develop policies, plans, and programs to achieve their vision of a 21st century healthcare system that makes affordable high-quality care accessible to all Americans. Members of HLC – hospitals, academic health centers, health plans, pharmaceutical companies, medical device manufacturers, laboratories, biotech firms, health product distributors, post-acute care providers, homecare providers, group purchasing organizations, and information technology companies – advocate for measures to increase the quality and efficiency of healthcare through a patient-centered approach. We are uniquely positioned to address innovation comprehensively from all perspectives in the healthcare industry.

HLC enthusiastically supports your goal to expand treatment innovation and access for the estimated 30,000 Americans living with a rare disease. Since Congress enacted the Orphan Drug Act forty years ago with the goal of stimulating the development of drugs for rare diseases, the US Food and Drug Administration (FDA) has approved more than 650 orphan drugs. With over 7,000 rare diseases identified, much more needs to be done.[1] If this progress is to be accelerated, Congress must unequivocally reprioritize innovation. Unfortunately, Congress has instead reversed course. Price setting provisions in the Inflation Reduction Act (IRA) will hinder innovation, including desperately needed treatments for rare diseases.

Specifically, the IRA's drug pricing provisions, once fully implemented, are likely to result in fewer small-molecule products developed and less continued research on already-approved drugs.

Small-molecule targeted therapies that patients can take orally are a promising recent innovation for treating a variety of rare diseases, particularly rare cancers.[2] Although the effective patent life for these small-molecule medicines has been found to be 13 to 14 years, when the IRA is fully implemented, small-molecule drugs will be eligible for possible government price setting only seven years after their FDA approval, with actual price ceilings applied at the

[1] Rare Diseases at FDA, U.S. Food and Drug Administration, (June 9, 2023), https://www.fda.gov/patients/rare-diseases-fda.

[2] Small Molecules in Targeted Cancer Therapy: Advances, Challenges, and Future Perspectives, Signal Transduction and Targeted Therapy, (May 31, 2021), https://doi.org/10.1038/s41392-021-00572-w.

nine-year mark.[3] The average cost to develop a new drug, including dollars spent on products that never gain approval, is between $1 billion and $2 billion.[4] Without an adequate time window to recoup these investments, drug manufacturers and capital investors are likely to redirect these investments away from small-molecule therapies.

While the IRA does include some exceptions in price setting for drugs developed to treat rare diseases, they are inadequate to protect innovation. According to CMS guidance, an orphan drug that is exempt from price caps would become eligible once the drug receives a second orphan drug designation to treat another rare disease. This disincentivizes manufacturers from producing drugs for rare diseases and harms patients.

HLC urges Congress to work with the administration to prioritize innovation and fix the IRA before it has these adverse effects. Implementation of the IRA's drug pricing provisions must be transparent and meaningfully engage stakeholders, centering the patient-experience. Collaboration with the private sector is also critical as we work towards the shared goal to reduce the cost of prescription drugs without significantly increasing health plan costs or sacrificing access or innovation.

HLC looks forward to working with Congress to increase innovation and access to care. Please reach out to Debbie Witchey with any questions.

Sincerely,

Mary R. Grealy
President

[3] Continuing Trends in U.S. Brand-Name and Generic Drug Competition, Journal of Medical Economics, (August 2, 2021), https://doi.org/10.1080/13696998.2021.1952795.
[4] Research and Development in the Pharmaceutical Industry, Congressional Budget Office, (April 8, 2021), https://www.cbo.gov/publication/57126.