Please Include $1.5 million for a Rare Disease Burden Study in the Senate's Fiscal Year 2020 Labor, Health and Human Services, Education Appropriations Bill

Bill: Labor, Health and Human Services, Education and Related Agencies
Section: Department of Health and Human Services; Office of Secretary, Office of the Assistant Secretary for Health
Request: Study of the comprehensive economic and societal burden of rare diseases in the US

Requested Funding Level: $1.5 million

Requested Report Language: The Committee is concerned that rare diseases, including undiagnosed and untreated rare diseases, are a significant fiscal burden on our nation, states, municipalities, and families. No later than 90 days after the date of enactment of this Act, the Secretary of Health and Human Services shall enter into an arrangement with the National Academies of Science, Engineering and Medicine (NASEM), under which NASEM agrees to study the total impact rare diseases have on the U.S. economy, including direct medical costs, non-medical costs, loss of income, as well as the societal consequence of undiagnosed and untreated rare disease. No later than 2 years after the date of enactment of this Act, the Academy shall provide a report on these findings to the Secretary of Health and Human Services, and the House and Senate Committees on Appropriations. The committee provides $1.5 million to support this study and report.

Background: 93% of more than 7,000 rare diseases have no FDA-approved treatment or cure. On average, it takes 8 years to properly diagnose an individual with a rare disease. Undiagnosed and untreated rare diseases likely cost the United States hundreds of billions of dollars a year. Yet no one has examined the true cost, across this diverse range of diseases that impact an estimated 30 million Americans. There is a dire need for a comprehensive analysis defining the true prevalence of rare disease in the United States and the resulting costs. Understanding the causes behind these costs will support Congress, the Administration, the private sector and other stakeholders in developing effective policies and tools to reduce this burden on the economy, taxpayer, workforce, patients and their families.

While there are an estimated 30 million total Americans suffering from rare diseases, data is limited as these small underserved patient populations span more than 7,000 unique diseases. A one-time investment in a comprehensive study will identify opportunities for cost savings through minimizing delay in diagnosis, closing treatment gaps, and maximizing early and effective intervention. This study will yield exponential returns to the economy and taxpayer - far beyond the cost of conducting it.

In the Fiscal Year 2018 Consolidated Appropriations Act, Congress recognized the need to better diagnose rare diseases. We encourage the Committee to build upon the FY18 language to examine the true cost of diagnostic delay and lack of treatment. Proposals to lower healthcare costs have largely focused on the pricing of existing therapies; however, the discussion rarely covers the costs to society of not diagnosing and treating patients. The overwhelming cost to the taxpayer, economy, caregivers and families, and the individual patient needs to be further examined through a comprehensive study.

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