The Helping Experts Accelerate Rare Treatments (HEART) Act of 2022

H.R. 6888, 117th Congress
Sponsors: Rep. Paul Tonko (D-NY), Rep. David McKinley (R-WV)

"Let's open doors of hope and healthcare to millions of Americans with rare diseases."
❖ **Paul Tonko**

Rare diseases are a widespread challenge

- There are **7,000 known rare diseases** and more are discovered every year
- An estimated **25-30 million Americans are living with a rare disease**
  - More than 50% of people with rare diseases are children
- 30% of children with rare diseases will not reach their fifth birthday
- In 2019, rare diseases cost Americans $966 billion due to direct medical costs, non-medical costs, and reductions in worker productivity

Treatments are limited & can be costly

- 95% of rare diseases do not have FDA-approved treatments
- Due to the small number of patients, studying rare diseases and conducting high-quality clinical trials is difficult
  - Each rare disease affects 200,000 or fewer people
- It can take patients years or even decades to receive a proper diagnosis for a rare disease
  - Many patients are misdiagnosed, receive improper treatments, and experience high rates of anxiety and depression due to the lack of a proper care infrastructure

Congress Can Make a Difference!

H.R. 6888, The Helping Experts Accelerate Rare Treatments (HEART) Act

- ❖ Requires a study on sufficiency and use of FDA mechanisms to incorporate patient/clinician perspective in FDA processes related to applications for drugs for rare diseases & conditions
- ❖ Calls on the FDA to be required to develop an annual report on progress of rare disease drug applications
- ❖ Requires FDA host a public meeting to address approaches to increasing and improving engagement with rare disease or condition patients, groups representing such patients, rare disease or condition experts, and experts on small population studies, in order to improve the understanding with respect to rare diseases or conditions in terms of patient burdens, treatment options and side effects
- ❖ Directs a review of the European Union's best practices for approving rare disease drugs

Connect with Congressman Paul Tonko on Facebook, Twitter or Instagram: @RepPaulTonko