The Helping Experts Accelerate Rare Treatments (HEART) Act of 2021

H.R. 1184, 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. 1184, the HEART Act of 2021

- ❖ Requires FDA Rare Disease Program staff to participate in rare disease drug reviews
- ❖ Directs rare disease experts to serve on drug development Advisory Committees panels
- ❖ Calls on the FDA to develop an annual report on the number and progress of rare disease drug applications within each agency division
- ❖ Requires that patients be consulted on Risk Evaluation and Mitigation Strategies (REMS)
- ❖ Directs the General Accounting Office to review the European Union's best practices for approving rare disease drugs

Congress can help ensure that top experts and patient perspectives are front and center in rare disease research. The time to act is now!

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