Haystack Project Applauds Introduction of Tonko-McKinley "HEART" Act to Bolster Rare Expertise in FDA Review of Rare and Ultra-rare Treatments

The HEART Act, sponsored by Congressmen Paul Tonko (D-NY) and David B. McKinley (R-WV), is supported by a broad coalition of ultra-rare patient advocacy groups.

ANNAPOLIS, MD – July 9, 2020 – Haystack Project, the nation's leading advocacy organization dedicated to supporting patient access and reimbursement for rare and ultra-rare disease patient communities, announced its support for The HEART Act 2020 (**H**elping **E**xperts **A**ccelerate **R**are **T**reatments Act), a bill to improve the Food and Drug Administration (FDA)'s review of rare and ultra-rare treatments. Introduced today by Congressmen Paul Tonko (D-NY) and David B. McKinley (R-WV), the HEART Act provides tangible and practical solutions to support rare disease patients and accelerate efforts underway at the agency.

"In our mission to expand treatment access and reimbursement for patients living with rare and especially ultra-rare diseases, the first and most important issue is that drugs that can provide benefit are reviewed and approved," said Jim Caro, CEO of Haystack Project. "We applaud the FDA for their many recent efforts to address the unique needs of the rare disease community. The easy-to-implement modifications in The HEART Act represent a major advance in that process."

The HEART Act will position more rare disease experts, including patients and their clinicians, to have an active role in the review process for new drugs. The changes outlined in the HEART Act are designed to be implemented seamlessly and quickly, without increasing drug development timelines or adding new levels of bureaucracy. The HEART Act calls for these changes:

- The FDA must consistently include its own Rare Disease Program staff in reviews for drugs to treat rare diseases.
- The FDA must consult directly with patients about any Risk Evaluation and Mitigation Strategies (REMS) for a rare disease drug when those REMS programs call for patient participation.
- Experts in rare diseases must be included in FDA Advisory Committee panels when reviewing rare disease drugs.
- Each year, the FDA must prepare a report showing how many rare disease drug applications were reviewed by each division at the Agency, including numbers on the prevalence of those conditions.
- The Government Accounting Office must review the EU process for approval of rare disease drugs and provide an assessment of how those processes might apply in the US, including their use of data from open label extension studies.

"The HAYSTACK Project shares our commitment to finding ways to improve the regulatory review process for drugs to treat rare diseases in the U.S. and we are very pleased to join with them in supporting this important bill," said Lindsey Sutton, co-founder of The FCS Foundation and an FCS patient. "We invite all members of the rare disease community including patients, clinicians, researchers and industry partners to support the HEART Act by asking their own representatives in Congress to cosponsor this important legislation."

It is estimated that there are more than 7,000 different rare diseases, many life-threatening and most without treatments. The development of new therapies to treat rare diseases can be challenging, but patients and their families work tirelessly to bring new treatments forward and should have a place in the regulatory and reimbursement decisions.

“Rare and ultra-rare diseases present a unique challenge for drug development,” said Tonko. “Our bipartisan legislation tackles this by ensuring rare disease experts are at the table as new therapies are being reviewed by the FDA. I urge my colleagues to join us in moving this legislation forward without delay.”

“Often times people suffering from rare diseases have few or no treatment options available. This bill makes practical changes to FDA’s approval process that can lead to new treatments and ensure rare disease experts have a voice at the table,” said Rep. McKinley.

About HAYSTACK PROJECT

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease patient advocacy organizations to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access. Our core mission is to evolve health care payment and delivery systems with an eye toward spurring innovation and quality in care toward effective, accessible treatment options for rare and ultra-rare patients. We strive to amplify the patient and caregiver voice in these disease states where unmet need is high and treatment delays and inadequacies can be catastrophic.

Media Contact:
Jim Caro
CEO
Haystack Project
Jim.caro@haystackproject.org

Saira Sultan
Policy Consultant
Haystack Project
Saira.sultan@haystackproject.org