Haystack Project Applauds Critical Progress on the HEART Act

Haystack Project, the nation’s leading advocacy organization dedicated to supporting patient access for rare and ultra-rare disease patient communities, is pleased to report that the HEART Act, H.R. 6888, met a critical milestone yesterday at the House Energy and Commerce Health Subcommittee hearing yesterday.

The Helping Experts Accelerate Rare Treatments (HEART) Act, co-led by Reps Paul Tonko (D-NY) and David McKinley (R-WV), is supported by a broad coalition of rare and ultra-rare patient advocacy organizations. Haystack Project supports thoughtful and tangible refinements to the Food and Drug Administration (FDA)’s review process for the rare and ultra-rare treatments that we work so hard and wait so patiently for.

Lead sponsor, Rep Paul Tonko said, “I drafted the HEART Act because we can do more to better incorporate the patient and rare disease medical experts in the FDA process - especially as it relates to rare and ultra-rare conditions.”

The important provisions of the HEART Act are easy-to-implement and represent an essential step forward in recognizing rare disease patients and providers as the experts that they are. The HEART Act calls for these changes:

- An annual, publicly available report from FDA indicating how many rare disease drug applications were accepted for filing and reviewed by each division at the Agency, including the prevalence of those conditions and the extent to which FDA is consulted with external experts on the review;
- A National Academies review and assessment of the EU process for approval of rare disease drugs and how they might apply in the US;
- FDA public meeting(s) to solicit rare disease community input on leveraging external expertise for rare disease product reviews;
- Inclusion of experts on small population studies in the list of experts with whom staff can consult during drug reviews; and,
- A GAO review and recommendations related to FDA’s mechanisms and tools for ensuring rare patient and physician perspectives are considered and incorporated in the benefit-risk assessment.

Haystack Project, and the patients it represents, looks forward to enactment of the HEART Act this year.