TOPIC: Press Release, Rare Disease Provisions in User Fee Act
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Haystack Project Applauds the Inclusion of the HEART Act In the Senate HELP Committee User Fee Package

Washington, DC June 14 — Haystack Project is pleased to see provisions of the Helping Experts Accelerate Rare Treatments Act (S. 4071/H.R. 6888) included in the Food and Drug Administration Safety and Landmark Advancements Act (FDASLA) of 2022 (S. 4348).

The bill will require GAO to assess the effectiveness of Food and Drug Administration's (FDA) policies and practices in (i) considering patient focused drug development data and information, patient experience data, and the views of patients; (ii) consulting and engaging stakeholders and external experts; and (iii) training Agency personnel reviewing rare disease applications to .ensure adequate expertise.

The bill will also require FDA to report on the number of rare disease applications submitted, approved, and for which the Agency consulted experts, as well as the size of the affected population. "Haystack Project is especially pleased to see a breakdown of FDA activity by rare disease population size, since the challenges from R&D to approval in conditions with 200,000 or fewer patients is very different than in 20,000, 200 or even fewer affected individuals. We hope this helps the Agency look across its review divisions to assess where additional expertise in rare and especially ultra-rare would be useful," said Deanna Darlington, CEO.

"As patients, we want to feel confident that the division reviewing our application has the needed experience in rare diseases," noted Melissa Goetz, co-president of the Familial Chylomicronemia Foundation. "We know the expertise is there, it's just not always in every review division though," added Nicole Casale, president of the Galactosemia Foundation.

Importantly, the bill also encourages FDA to consult with patients and patient groups, together with an expert selected by the group, to meet with FDA prior to application submission, and to consult with an expert in the science of small population studies if a disease-specific expert is unavailable. Furthermore, the FDA is encouraged to invite at least one rare disease expert identified by the patient group as a non-voting member of the Advisory Committee meeting, as well as a voting rare disease expert or an expert in the science of small population studies.

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The Voices of Rare & Ultra Rare

> "Patients diagnosed and living with ultra-rare diseases have no choice but to become experts in their conditions. They are far more sophisticated and well versed in benefit-risk assessments than the average patient. We applaud the HELP Committee for recognizing the need to give our patients a seat at the table," said Deanna Darlington.

The Helping *Experts* Accelerate Rare Treatments (HEART) Act was introduced by Representatives Paul Tonko (D-NY) and David McKinley (R-WV) in the House and Senators Casey (D-PA) and Scott (R-SC) in the Senate. The HEART Act is supported by a broad coalition of rare and ultra-rare patient advocacy organizations that is thankful for the leadership of these Members on this critical issue.

In addition to the leaders and co-sponsors of the HEART Act, Haystack Project is grateful for the support of strong HELP committee staff engagement as we worked to bring attention to the need for greater agency partnership with rare patients during an application's review.

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. We, and the patients we represent, looks forward to continuing to work with Congress and the FDA to ensure that the patient voice is considered during the drug review process for potential treatments for rare and ultra-rare conditions.