



May 20, 2022

The Honorable Patty Murray  
Chair  
Senate Committee on Health, Education,  
Education, Labor, and Pensions  
United States Senate  
Washington, D.C. 20510

The Honorable Richard Burr  
Ranking Member  
Senate Committee on Health,  
Education, Labor, and Pensions  
United States Senate  
Washington, D.C. 20510

Dear Chair Murray and Ranking Member:

Haystack Project appreciates the opportunity to provide comments in response to the discussion draft entitled “Food and Drug Administration Safety and Landmark Advancements Act of 2022 (FDASLA)” which reauthorizes the user fee programs that support the safe and efficient review of pharmaceuticals that can be used to treat rare and ultra-rare diseases. Haystack Project is the nation’s leading advocacy organization dedicated to supporting patient access for rare and ultra-rare disease patient communities. Haystack Project looks forward to collaborating with the Committee on policies to ensure that the experts in rare diseases and the patients that suffer from them are considered during the Food and Drug Administration’s (FDA) review of potential treatments associated with rare and ultra-rare diseases.

Haystack Project applauds the FDA for its recent efforts to address the unique needs of the rare disease community. Haystack Project believes S. 4071, the “Helping Experts Accelerate Rare Treatments (HEART) Act,” introduced by Senators Casey (D-PA) and Scott (R-SC), will build on these successes by positioning more rare disease experts, including patients and their clinicians, to have an active role in the FDA’s review process, and share important perspectives and expertise with those already working hard for our patient community.

The HEART Act includes the following provisions:

- Directs FDA to prepare and submit to Congress an annual, publicly available report that includes data on 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 consulting with external experts on the review;
- Commissions a National Academies review and assessment of the European Union (EU) process for approval of rare disease drugs and how they might apply in the US;
- Provides FDA the authority to consult with experts and patients during the review process;
- Provides FDA the authority to include experts and patients in the public meetings and the advisory committee meetings; and
- Inclusion of experts on small population studies in the list of experts with whom staff can consult during drug reviews.

Haystack Project encourages the Committee to consider including the HEART Act in any subsequent versions of FDASLA and looks forward to continuing to work with our bill sponsors and the Committee to enact meaningful policy changes that will help patients suffering from rare diseases gain access to more treatment options.

Sincerely,