



November 18, 2022

The Honorable Charles E. Schumer
Majority Leader
United States Senate
Washington, D.C. 20150

The Honorable Mitch McConnell
Minority Leader
United States Senate
Washington, D.C. 20515

The Honorable Nancy Pelosi
Speaker of the House
United States House of Representatives
Washington, D.C. 20515

The Honorable Kevin McCarthy
Minority Leader
United States House of Representatives
Washington, D.C. 20515

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

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

The Honorable Frank Pallone
Chair
Committee on Energy and Commerce
United States House of Representatives
Washington, D.C. 20515

The Honorable Cathy McMorris Rodgers
Ranking Member
Committee on Energy and Commerce
United States House of Representatives
Washington, D.C. 20515

Dear Congressional Leadership,

Haystack Project is a non-profit umbrella organization representing more than 120 ultra-rare patient groups. Most of the rare disease they represent affect fewer than 20,000 patients in the U.S., and in fact often represent 2,000 or fewer. On their behalf, we have collected their experiences with the Food and Drug Administration (FDA), and compiled a tangible, easily feasible, and impactful set of requests that formed the basis of the HEART Act ("Helping Experts Accelerate Rare Treatments").

We were honored to have bipartisan committee members leading the bills in the House and Senate and to experience such positive engagement from committee staff across both sides of the aisle too. Although we were disappointed to see Heart Act provisions fall out of the final user fee package, we are hopeful we can get them incorporated into the year-end package. We urge you on behalf of rare and especially ultra-rare patients in the U.S. to include the various HEART Act provisions in the House and Senate passed user fee bills. Both House and Senate

versions include critical reports and much-needed guidance to the FDA regarding the review of rare disease treatments. However, there are critical differences between the two bills.

The House bill has stronger language on the required FDA report [§703(a)] and comparison to EU mechanisms for reviewing drugs for rare diseases [§703(b)]. It also requires a study on the sufficiency and use of FDA mechanisms for incorporating the patient and clinician perspective in FDA processes related to applications concerning drugs for rare diseases or conditions [§703(e)].

The Senate bill has stronger language around the use of expert consultants during the drug review process, including any advisory committee meetings [§508(d)]. This language is critically important to rare disease patients because it provides additional permission for FDA to consult with rare disease stakeholders, including patients and caregivers, when evaluating a potential therapy for that rare disease. It also recognizes that there may be conditions for which a community of color or other historically underrepresented community is disproportionately affected; the bill encourages the FDA to consult with appropriate groups representing that sub-population.

Haystack Project supports thoughtful and tangible refinements to FDA's review process for the rare and ultra-rare treatments that our patients work so hard and wait so patiently for. We, and the patients we represent, look forward to continuing to work with Congress and the FDA to ensure that the rare patient voice is considered during the drug review process in more substantial and concrete ways when reviewing potential treatments for rare and ultra-rare conditions.

We urge Congress to include the House and Senate provisions outlined above from the HEART Act in the end of year legislative package. For more information, please contact Saira Sultan at saira.sultan@haystackproject.org.

Thank you for your consideration,



Deanna Darlington, CEO
Haystack Project





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