



The Honorable Frank Pallone  
2107 Rayburn House Office Building  
Washington, DC 20515

The Honorable Cathy McMorris Rodgers  
1035 Longworth House Office Building  
Washington, DC 20515

RE: Helping Experts Accelerate Rare Treatments (HEART) Act, HR 1184

Dear Chairman Pallone and Ranking Member McMorris Rodgers:

We are writing in ask for your support in passing the HEART Act, HR 1184 which contains five provisions critical to the patients we represent with rare diseases.

We support the pragmatic, thoughtful, incremental and tangible refinements to the Food and Drug Administration (FDA)'s review process in the HEART Act. Our patients wait every day for the rare and ultra-rare treatments for which we work so hard, wait so long and urgently need.

We applaud the FDA for their many recent efforts to address the unique needs of the rare disease community. The important changes in the HEART Act are easy-to-implement modifications that represent a major advance to the agency's efforts to date.

The HEART Act will position 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 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 indicating 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.

We appreciate the opportunity to support such thoughtful and important legislation and look forward to working with you to pass the HEART Act in 2021.

Respectfully,





