HAYSTACK PROJECT
The Voices of Rare & Ultra Rare

The Honorable Paul Tonko
2369 Rayburn House Office Building
United States House of Representatives
Washington, DC 20515

The Honorable David B. McKinley
2239 Rayburn House Office Building
United States House of Representatives
Washington, DC 20515

RE: Helping Experts Accelerate Rare Treatments (HEART) Act
Dear Representatives Tonko and McKinley:

We are writing to express our appreciation for your leadership in advancing the Helping Experts Accelerate Rare Treatments (HEART) Act. We are eager to support your 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 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.

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Respectfully,