The Honorable Richard Burr
217 Russell Senate Office Building
Washington, DC 20515

The Honorable Patty Murray
154 Russell Senate Office Building
Washington, DC 20515

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

Dear Chairman Murray and Ranking Member Burr:

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,


VHL
Alliance


AA·MDS
INTERNATIONAL FOUNDATION


Hairy Cell Leukemia


ACROMEGALY
community


Choroideremia
RESEARCH FOUNDATION


ippf
International
Pemphigus&
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ALPHA-1
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DISORDERS ASSOCIATION
from discovery to cure


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international
cancer
advocacy
network
the virtual lifeline for proactive cancer patients


VASCULITIS
FOUNDATION


Tuberous Sclerosis Alliance


BORN A HERO
RESEARCH FOUNDATION


cure hht
The Cornerstone of the HHT Community


PXE
international


Genetic Alliance


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Siegel
Rare Neuroimmune
Association


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HealthTree
Foundation


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