



June 15, 2022

The Honorable Susan Collins
413 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Tim Kaine
231 Russell Senate Office Building
Washington, DC 20510

Dear Senator Collins and Senator Kaine,

In follow up to our letter of March 16th we write today to offer our endorsement of your legislation to preserve and strengthen FDA's accelerated approval pathway. As we said in March, at each turn when Congress has legislated on accelerated approval the pathway has been reaffirmed and the Agency has been encouraged to fully harness the promise of the pathway on behalf of patients. Lives have been saved, extended and improved. Yet there is so much work to be done.

We support this legislation on behalf of the 6.5 million Americans living with Alzheimer's, where treatments are just beginning to emerge, but the unmet need remains vast. And on behalf of the 1.6 million Americans living with Type 1 diabetes, where treatments have emerged but there are still critical unmet needs for curative disease modifying therapies and regenerative therapies. For Americans living with a rare disease, the unmet need is also staggering. According to the Rare Genetics Institute, one in ten Americans (half of them children) has a rare disease and 30% will not live to see their 5th birthday. The accelerated approval pathway exists so that FDA can bring urgency and flexibility to the review process. Whether that is for a first of its kind treatment or a curative gene therapy. Patients waiting months instead years for access to treatments has extraordinary meaning to them and their families.

We believe your legislation brings clarity and focus to the purpose behind the accelerated approval pathway. Specifically, the legislation sets an expectation that every review division at FDA will be conversant in and, as appropriate, embrace use of the accelerated approval pathway where unmet need exists, and regulatory flexibility is warranted. The legislation makes clear that the Agency leadership will support and engage as needed on accelerated approval – not to create another step in the process for reviewers or divisions where the pathway is well understood and exercised, but rather where confidence and use is lacking. For years Agency leadership has heralded the accelerated approval pathway as the gateway – in many cases the only gateway – to novel treatments to meet urgent patient needs. This legislation codifies their responsibility to support and advance use of the pathway across FDA.

The legislation also furthers the bipartisan, shared goal of leveraging real world evidence in assessing and proving the value of accelerated approval products. It also calls for additional accountability measures to bring more transparency to the process for the Agency, sponsors, and patients.

As the Senate advances the FDA User Fee reauthorization, which includes your legislation, the undersigned organizations and our broader community of patients and families offer our unequivocal support. We believe your bill will preserve the accelerated approval pathway and ensure that all patients living with unmet need, regardless of their specific condition, can be assured of the FDA's appropriate utilization of the pathway. We stand ready to work with you on any modifications or technical reviews of the language as the legislative process moves forward.

Celebrating scientific advancements and innovation and speeding that to patients should not be controversial. In fact, that is how we define success. Thank you again for your tremendous leadership and willingness to fight for patients and their families.

Sincerely,

Robert Egge
Chief Public Policy Officer, Alzheimer's Association
Executive Vice President, Alzheimer's Impact Movement

Cynthia Rice
Chief Mission Strategy Officer, JDRF