RARE Disease
Company Coalition

March 17, 2022

The Honorable Doris Matsui
United States House of Representatives
2311 Rayburn House Office Building
Washington, DC 20515

The Honorable Brad Wenstrup
United States House of Representatives
2419 Rayburn H.O.B.
Washington, DC 20515

The Honorable Roger Wicker
United States Senate
555 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Amy Klobuchar
United States Senate
425 Dirksen Senate Office Building
Washington, DC 20510

RE: Rare Disease Company Coalition Endorsement of H.R. 4472/S. 373, the BENEFIT Act

Dear Representatives Matsui and Wenstrup and Senators Wicker and Klobuchar,

The Rare Disease Company Coalition (RDCC) is proud to support the bipartisan **Better Empowerment Now To Enhance Framework And Improve Treatments (Benefit) Act (H.R. 4472 / S. 373)**. We applaud the work Congress has done to date to advance patient engagement in drug development and this legislation introduced by Representatives Doris Matsui (CA-6) and Brad Wenstrup (OH-2) and Senators Roger Wicker (R-MS) and Amy Klobuchar (D-MN) demonstrates a continued interest in doing so.

The Rare Disease Company Coalition represents 21 innovative life science companies committed to discovering, developing, and delivering treatments for the one in ten Americans living with a rare disease. Our goal is to inform policymakers of the unique challenges—and promises—we face in taking these rare disease drugs from research through development, approval, manufacturing, to delivery to patients. Collectively, Coalition members invested over $4.5 billion in R&D in 2020; have brought 36 treatments to market to date, the majority of which are first-to-market therapies; and are presently working on more than 250 rare disease development programs, many of which would be first-to-market therapies if approved. With less than 95% of rare diseases without an Food and Drug Administration (FDA)-approved treatment option, we are urgently working to meet the needs of patients and their caregivers with currently limited or no treatment options.

Rare disease in the United States is defined as a condition that affects fewer than 200,000 people. There are 7,000 identified rare diseases that impact an estimated 25 to 30 million Americans. These diseases are often devastating and life-threatening: 80 percent of rare diseases are genetic in origin, 50 percent impact children, and 30 percent of those children will not live to see their fifth birthday.

To respond to the unique challenges in the discovery, development, and delivery of rare disease treatments for patients, it is vital that the FDA continues to advance patient-focused drug development (PFDD). Great progress has been made incorporating the patient perspective throughout the drug development process thanks to provisions included in the Prescription Drug User Fee Act (PDUFA) of 2012, (FDASIA) and the 21st Century Cures Act in 2016. It is important that we continue to build on this

progress and encourage the FDA to include patient experience or PFDD data as a part of its risk-benefit framework.

The BENEFIT Act addresses the need to reflect the patient perspective and experience in the FDA's risk-assessment tool. This policy would play a critical role in the FDA evaluation and approval decision regarding drugs and other medical products and will allow the FDA to capture meaningful patient experience, PFDD, and related data as part of the benefit-risk assessment. We believe this legislation will advance patient engagement, which ultimately can help advance the development and delivery of treatments to those living with rare diseases. The RDCC thanks you for championing policies to improve the regulatory process and supporting the voice of the rare disease patients we serve.