May 14, 2018

The Honorable Bill Cassidy
520 Hart Senate Office Building
Washington, DC 20510

Dear Senator Cassidy:

As patient advocates and stakeholders representing the diverse community impacted by rare diseases, the undersigned are committed to advancing development of, and access to, treatments for these serious, and often life-threatening conditions. We are writing to ask for your support for legislative changes to the 340B discount drug program so that innovators, and the investors supporting their work, continue to view development of treatments for extremely rare diseases as a worthwhile enterprise.

The 340B discount drug program injects a complex interplay between providers, the Medicaid program, and manufacturers. The significant growth of the 340B drug discount program over the past several years has increased the level of risk and uncertainty for manufacturers. When innovators set their prices for breakthrough therapies, they have to work under a set of assumptions, including patient population size and payer mix, and they certainly have to consider both the Medicaid rebate and projected volume of 340B discounted drug sales. Unfortunately, 340B exposure can vary a great deal from expectations, diverge from quarter to quarter, and even climb steadily over time. Since treatment for very rare conditions is often concentrated in a few facilities with expertise, any consolidation, 340B abuse, or shifts in site of care from the inpatient setting can drive 340B market share well above the already-high 25% toward half or more of total sales, and in very unpredictable ways. With smaller patient populations, unexpectedly high 340B exposure can present dramatic revenue shortfalls that cannot be "corrected" with price increases without risk of triggering "penny pricing." This can have a catastrophic impact on investor perception and innovator abilities to recoup the costs of product development.

As patient advocates, we have worked hard to bring attention to the disorders impacting, and even threatening, our lives and/or the lives of our loved ones. We have forged strong relationships with innovators, often working hand-in-hand toward developing treatment options, and ultimately, cures. Our experience tells us that innovators share our goal of ensuring that their treatments advances will reach the patients who need them. The 340B program, however, imposes a forced discount on an uncertain volume of sales without a clear nexus to patient needs and/or access. We fear that it will increasingly tip the scales against innovation and ultimately deprive patients of new treatment options.

We believe that the 340B program can play an important role in ensuring that vulnerable patient populations have access to medical care. Unfortunately, manufacturers of ultra-orphan drugs do not have the luxury of volume or price elasticity to counterbalance 340B exposure. These entities need a safety valve for maintaining viability that is short of declining Medicaid participation.

We urge you to support changes to the 340B program that align with the needs of patients with rare disorders.

Lipodystrophy United
Tuberous Sclerosis Alliance
APF American Porphyria Foundation
Choroideremia Research Foundation
MPN Research Foundation Change Your Prognosis
CRPS Foundation of America
debra
MLD foundation We C.A.R.E.
Mickie's Miracles
Children's Project
Twist of Fate
Bridge the Gap SYNGAP Education & Research Foundation
PTEN Hamartoma Tumor Syndrome Foundation
ACD Association for Creating Deficiencies
Envision the Cure Cure CADASIL Embrace the Challenge