RARE ACCESS ACTION PROJECT

The Honorable Richard Neal
Chair, Ways and Means Committee

The Honorable Kevin Brady
Ranking Member, Ways and Means Committee

June 7, 2019

Dear Chairman Neal and Ranking Member Brady,

Thank you for the opportunity to comment on the draft legislation posted on the Committee webpage regarding capping out of pocket costs for patients who reach the catastrophic threshhold in Medicare Part D. The focus of our comments will be on the impact of capping out of pocket costs for rare disease patients, and the positive impact such a change to the program could make in the lives of rare Medicare beneficiaries.

According to the Food and Drug Administration, "an orphan disease is defined as a condition that affects fewer than 200,000 people nationwide. Some diseases have patient populations of fewer than a hundred. Collectively, however, they affect as many as 25 million Americans, according to the National Institutes of Health (NIH), and that makes the diseases—and finding treatments for them—a serious public health concern."

Many advances have been made in rare disease treatment, with cures now within reach through new technologies. These advances were spurred by bipartisan commitment to finding treatments for rare diseases, of which there are over 7,000 and over 50 percent of rare diseases affect children. During the first 25 years of the Orphan Drug Act (passed in 1983), 326 new drugs were approved by the FDA and brought to market for all rare disease patients combined.

However, with major advances on the horizon, including the promise of gene therapies, the health care system in the United States has been slow in preparing and even slower in making those advances available to patients. We must not only continue to advance therapies through discovery and development, but we must also make certain that patients ultimately have access to them post-approval.

As you know, when Medicare Part D was designed, no one envisioned the revolution that would occur in the treatment of rare diseases over the subsequent fifteen years since its passage and implementation. You will recall that copayments were instituted to ensure that patients were invested in their care and drug selection, and a five percent copayment was not considered a barrier. Fast forward to 2019 and there are now medicines that rare disease patients utilize in the outpatient setting. And the reality is that often there is still no or only one treatment option thus making a copayment for rare patients an unnecessary burden. Many adult rare disease patients will endure years of misdiagnosis during their

patient journey, only to find once they are properly diagnosed that the rare disease medication is out of reach because of the copayment in Medicare Part D has placed it out of reach.

We applaud the committees efforts to create a bipartisan solution to what has become a hurdle to rare disease treatment. RAAP welcomes working with members of the committee and staff to find a solution that ensures that rare Medicare participants have access to critical medicines.

About RAAP

The Rare Access Action Project (RAAP) began in 2017 as an ad hoc coalition of patient and life sciences stakeholders advocating for solutions to issues that limit patient access to health care. Many rare disease patients, upon a diagnosis, believe that because they have coverage (commercial, Medicaid or Medicare) that they will be able to utilize the medicine or technology that was developed for their disease. However, this is not always the case. Our first proposal included capping rare patient out of pocket costs in Medicare Part D by extending the coverage gap discount program into the catastrophic portion of coverage.

RAAP is committed to finding solutions to reimbursement and access issues that impact patient continuity of care.

Thank you for this opportunity to comment on the current proposal that has been released by the committee. We appreciate your bipartisan efforts and would welcome the opportunity to work with the committee to provide a solution that benefits rare disease patients as well as all beneficiaries who rely on Medicare Part D.

Regards,

Michael Eging
Executive Director
Rare Access Action Project (RAAP)
Email: rareaccessproject@gmail.com
Phone: 202.631.5752