



HAYSTACK PROJECT APPLAUDS INTRODUCTION OF BILL TO IMPROVE ACCESS TO EVIDENCE-BASED CARE FOR RARE DISEASE PATIENTS

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WASHINGTON, D.C. – Today, Congressman Dunn (R-FL), Congresswoman Doris Matsui (D-CA) and Reps. Mike Thompson (D-CA), Mike Kelly (R-PA), and Markwayne Mullin (R-OK) introduced the **The Providing Realistic Opportunity to Equal and Comparable Treatment for Rare** (“PROTECT Rare”) Act, legislation to support patients with rare and ultra-rare diseases in getting evidence-based, medically necessary care covered by their health insurance.

The bill permits Medicare and Medicaid to use clinical guidelines and peer-reviewed literature to allow for coverage of rare disease treatments.

“We look forward to the enactment of this important legislation, and for the work the sponsors have put into this so far. We are confident that this will make a significant difference in the lives of the rare disease patients we represent and work with every day,” said Haystack Project CEO, Chevese Turner.

This bill is supported by more than [60](#) rare disease organizations.

“When I needed my off-label drug for Immune Thrombocytopenia, I had to jump through several hoops. Insurance kept denying the treatment, not even sending it to the proper third-party reviewers. They kept trying to push me to take IVIG infusions every other week, which were not working and were billing at \$175,000 per treatment! There was also no formal PA or appeals process my doctor’s office could leverage. I ended up almost in critical care because my platelets dropped low enough to put me at serious risk of potentially fatal internal bleeding. Because this process took so long, it caused diseases progression in a way that made recovery more difficult. Totally avoidable if we had proper protocols in place for rare disease patients to access off-label treatments. I applaud Dr. Dunn for the work he is leading on the Protect Rare Act. It can’t come soon enough! When I learned he was going to tackle this, it made me so proud to call Dr. Dunn my Congressman!” said Candace Lerman, from Tallahassee.



The bill will also require private payers to create an expedited review pathway for formulary exception, reconsideration, and/or appeal of any denial of coverage for a drug or biological prescribed for a patient with a rare disorder.

“Although there is only one FDA approved therapy for my rare condition, pemphigus, there are several other off-label drugs that are used as standard of care for patients like me,” said Janet Segall, a constituent of Congresswoman Matsui. “Unfortunately, the off-label treatments are not covered. Also, there isn’t even an appeal process that’s easily accessible, even though my doctors tell me these treatments are standard of care for my disease. In the meantime, all the delays make my symptoms worse, cause a lot of pain, and make things near impossible financially. The PROTECT Rare Act will give rare disease patients like me access to the treatments my doctors think is best for me and are considered medically acceptable for pemphigus. This will help me and so many other patients, so thank you for tackling this important issue for all of us. Living with a rare disease is hard enough without having to be denied access to the treatments I need.”

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