Providing Realistic Opportunity to Equal and Comparable Treatment for Rare (PROTECT Rare) Act

(formerly Access to Rare Indications Act)

Reps. Doris Matsui, Neal Dunn, Mike Thompson, Mike Kelly

More than thirty million Americans, or one in 10, have a rare disease, and over 90 percent of the over 10,000 known rare diseases still have no FDA-approved therapy. Given the challenges of rare disease therapy development, new therapies are few and far between. This means that many rare disease patients are left to rely on unapproved or "off-label" use of drugs approved by the FDA for more common conditions to treat the symptoms of their diseases. Rare disease patients need speedy access to potential treatment options to maximize reduction of disease burden, slow the progression of their disease, improve their long-term prospects, and even save their lives.

Rare Disease Patients Cannot Access Off-Label Treatments:
Despite the promise of potentially lifesaving off-label treatments for patients with rare diseases, many patients cannot access these treatments due to lack of insurance coverage for off-label use. By statute, Medicare can generally only cover medications used in accordance with their FDA-approved label or for indications common enough to be recognized in Medicare-approved reference publications. Commercial insurers and Medicaid create barriers to coverage, including step-therapy and other utilization management tools or even outright denial of coverage for off-label treatments. These policies can expose patients to drugs that are ineffective and even harmful while denying them the therapies that are not only the standard of care but also are often the only available treatments for their conditions.

Such delays and poorer outcomes are especially burdensome for patients in underserved communities who may lack the resources to either pay out-of-pocket for needed therapies or navigate the health care system to secure coverage. Rural, low-income, and minority communities have continued to raise the alarm that the ongoing burden of fighting appeals, either themselves or through their clinicians, is a strong deterrent to patient access of needed care.

The *PROTECT Rare Act* would:

- Allow Medicare and Medicaid to use additional sources including compendia and peer-reviewed literature to meet the criteria for "medically accepted indication," which is the standard that must be met for Part D and Medicaid coverage.
    - This builds on 1993 legislation that improved access to cancer care by allowing cancer drugs to be considered "medically accepted" if included in certain compendia or supported by clinical evidence in peer-reviewed medical literature.
- 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, including off-label prescriptions.

This is not a coverage mandate, and applies only to individuals with rare diseases, and not broadly to all uses of the drugs or biologicals.

This bipartisan legislation is endorsed by Haystack Project, EveryLife Foundation for Rare Diseases, National Organization for Rare Disorders (NORD), and Rare Disease Diversity Coalition (RDDC).

For more information or to cosponsor this bill, contact Jackie Weinrich at Jackie.weinrich@mail.house.gov or Sarah Gilbert at sarah.gilbert@mail.house.gov.