



TOPIC: Press Release, Access to Rare Indications Act

For Immediate Release

December 7, 2021

Media Contact:

Deanna Darlington, CEO

Deanna.Darlington@haystackproject.org

Haystack Project Applauds Introduction of the Access to Rare Indications Act

The Access to Rare Indications Act was introduced by Representatives Matsui (D-CA) Thompson (D-CA), Kelly (R-PA) and Mullin (R-OK)

Washington, DC - December 2, 2021 — Haystack Project, the nation’s leading advocacy organization dedicated to supporting patient access for rare and ultra-rare disease patient communities, is grateful for the leadership of Representatives Matsui, Thompson, Kelly and Mullin on the Access to Rare Indications Act. This bill recognizes that rare patients often do not get the medically necessary care they are insured for and will ensure that they are more easily able to access what is evidence based, standard of care for their conditions. “We look forward to the enactment of this important legislation so that our community of rare patients can get the treatments their providers think best for them,” said incoming Haystack Project CEO, Deanna Darlington.

Specifically, this legislation will help level the playing for rare disease patients by allowing Medicare and Medicaid to use sources other than the FDA-approved drug label or compendia to determine whether a treatment prescribed for a rare condition is a medically accepted use. It 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 to treat or manage a patient’s a rare disorder. Existing processes can be time consuming for what are often debilitating and progressive conditions.

Patients, as well as their caregivers and physicians have noted the significant impact this legislation will make in their lives if enacted. These are just a few of their testimonials in support of Reps. Matsui, Thompson, Kelly and Mullins’ recognition of what they live with every day.

“I live in Erie, PA and not only have a very rare condition, but an atypical form of it. Because I present with different symptoms, the diagnostic process has taken nearly 10 years, causing a significant financial and emotional burden. Worst of all, there is no cure or even effective treatment for my condition. Legislation like the Access to Rare Indications Act is critical for our community of rare patients, and we thank you for your leadership here.” Mark P.

Deanna Darlington,
CEO
Haystack Project
deanna.Darlington@HaystackProject.org

www.HaystackProject.org

In 2013, when our daughter Madilyn was diagnosed with tuberous sclerosis complex (TSC), I knew I had to get involved. TSC has manifested itself in our daughter's brain, skin, heart, and kidneys. In 2015 our daughter had brain surgery to stop the hundreds of seizures she had weekly. It was a devastating time in our lives. Thankfully I have found healing during her journey by volunteering with the TSC in Oklahoma, where I have personally witnessed several parents crying out for help as they fought to get life-saving medication for their loved ones. It is the worst feeling as a parent to know what your loved one needs, only to be told it doesn't matter because of insurance red tape and policies. Our community deserves to be able to access life-changing medications. The Access to Rare Indications Act will give caregivers and parents peace of mind knowing we won't have to spend hours on the phone fighting for our loved ones to have their basic needs met. It means we can sleep better at night, knowing we won't have to go without the medicine that gives our family members the best quality of life” Heather L.

“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. 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 Access to Rare Indications 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.” - Janet S.

“We live in Benicia, California and have a son, Oliver, with a very rare disease called Congenital Disorder of Glycosylation (CDG). His rare disease has impacted our lives on every level since the day he was born. Our hope for more research and funding as well as potential therapies remains strong. We look forward to a day when there are therapies for CDG. The Access to Rare Indications Act will ensure that all patients with rare and ultra-rare diseases have access to the therapies they desperately need. This Act is incredibly important and desperately needed for our Rare Disease Community. Thank you for introducing this very important bill. – Claudia G-V