Support Access to Treatment for Rare Disease Patients - Cosponsor the Access to Rare Indications Act

Dear Colleagues,

In honor of Rare Disease Week on Capitol Hill (Feb 28-March 2), we invite you to cosponsor H.R. 6160, ***the Access to Rare Indications Act***. This legislation will allow Medicare and Medicaid to use sources including compendia, peer-reviewed literature, and the expertise of rare disease specialists to validate that a prescribed therapy meets the medically accepted criteria used to determine insurance coverage for a rare disease patient. The *Access to Rare Indications Act* will work to ensure that the 1 in 10 Americans who live with rare diseases have the intended benefit of health coverage and can access evidence-based standards of care for their conditions.

This bill recognizes that over 90% of the 7,000 known rare diseases have no FDA-approved treatment, due to a lack of research in the rare disease space, and challenges in finding enough patients to conduct clinical trials. This leaves many rare and ultra-rare disease patients facing serious and life-threatening illness to rely on off-label use of drugs approved by the FDA for more common conditions. To address this problem, the *Access to Rare Indications Act* aligns coverage in Medicare and Medicaid for off-label rare disease treatment with Medicare's existing system used to determine coverage for off-label cancer treatment. Additionally, this bill requires 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.

Please consider supporting this important legislation that will ensure rare and ultra-rare patients are no longer denied access to the prescribed care they need. If you have any questions or would like to become a cosponsor, please contact Christina McCauley with Congresswoman Matsui at Christina.McCauley@mail.house.gov or Kaitlynn Skoog with Congressman Mullin at Kaitlynn.Skoog@mail.house.gov.

Sincerely,

DORIS MATSUI | *Member of Congress*

MARKWAYNE MULLIN | *Member of Congress*

MIKE THOMPSON | *Member of Congress*

MIKE KELLY | *Member of Congress*

Endorsements (64): Haystack Project, Cutaneous Lymphoma Foundation, Global Genes, National MPS Society, Cure HHT, MitoAction, Run X1 Research Program, SCID Angels for Life Foundation, SADS Foundation, PXE International, Genetic Alliance, Siegel Rare Neuroimmune Association (SNRA), NBIA Disorders Association, Share and Care Cockayne Syndrome Network, Alpha-1 Foundation, Amyloidosis Support Groups, Soft Bones, Inc., GI Cancers Alliance, Dup15q Alliance, National Niemann Pick Disease Foundation, Sarcoma Foundation of America, TSC Alliance, Vasculitis Foundation, MLD Foundation, United MSD Foundation, Caseys Cure, National Ataxia Foundation, Histiocytosis Association, Acromegaly Community, International SCN8A Alliance, Dee-P Connections, Choroideremia Research Foundation, Taylor's tale, Usher Syndrome Coalition, Usher 1F Collaborative, Phelan-McDermid Syndrome Foundation, CDG Care, Cure GM1 Foundation, CSNK2A1 Foundation, International Pemphigus & Pemphigoid Foundation, International Rett Syndrome Foundation, National Eosinophilia-Myalgia Syndrome Network, VHL Alliance, Appendix Cancer Pseudomyxoma, The Myelodysplastic Syndromes Foundation, Inc., Southern Christian Leadership Global Policy Initiative, Rothmund Thomson Syndrome Foundation, Costello Syndrome

Family network, Barth Syndrome Foundation, Cares Foundation, Hope for Marian, International Cancer Advocacy Network, Cure VCP Disease, Inc., Exon 20 Group, Organic Acidemia Association, Health Tree Foundation, Galactosemia Foundation, International Fibrodysplasia Ossificans Progressiva Association, International Foundation for CDKL5 Research, Alström Syndrome International, NTM Info and Research Inc., No Stomach for Cancer, T.E.A.M. 4 Travis, Hairy Cell Leukemia Foundation, and the Global Foundation for Peroxisomal Disorders