

September 10, 2019

Mr. Kevin K. McAleenan
Acting Secretary
Department of Homeland Security
Washington, DC 20528

Dear Acting Secretary McAleenan:

As organizations representing rare disease patients in need of lifesaving therapies, we are writing to express our concern over the United States Citizenship and Immigration Services (USCIS) change in policy to eliminate medical and humanitarian criteria from the deferred action program. This change essentially terminates the legal status of patients, who are currently residing in the U.S., in order to receive life-saving medical treatments or to participate in clinical trials. Under this change in policy, patients who are in the U.S. legally may now be forced, with little notice, to return to countries that do not have the ability to provide their treatments and lack the infrastructure for crucial medical care. Requiring deportation, particularly in cases of rare disease patients whose countries of origin are not able to provide access to treatment, is tantamount to a death sentence.

While we appreciate that USCIS subsequently announced it would re-open some of the pending deferred action requests, the future of this life-saving program as the only option for patients to legally remain in the U.S. remains unclear. We ask you to urgently establish and clarify the process under which migrant individuals who participate in a clinical trial or receive life-saving medical treatment in the U.S. may request and obtain legal permission to reside in the U.S., through deferred action or another program.

Many of the patients who may be affected by this change in policy are in the U.S. because they participated in a clinical trial. It is critical that patients be allowed into this country to participate in clinical trials, particularly for rare diseases, because there are often not enough patients in any one country (including the U.S.) to conduct a robust clinical trial. As you may know, a rare disease is defined by the U.S. National Institutes of Health (NIH) and the U.S. Food and Drug Administration (FDA) as a condition affecting 200,000 individuals or less in the U.S. An estimated 30 million Americans are affected by one of 7,000 rare diseases. With only 7% of the 7,000 rare diseases having FDA approved treatments, ensuring that the U.S. remains a leader in hosting clinical trials is not only critical to innovation, but also vital to saving the lives of U.S. patients with rare diseases. Participation of migrant and U.S. born patients in clinical trials paves the way for U.S. patients to gain access to FDA approved therapies proven to be safe and effective. In many cases, migrant patients must remain in the U.S. after the trial has completed in order to continue receiving life-saving treatment and/or for further study.

Each year, approximately 1,000 deferred-action applications related to medical issues are granted for patients who need to be in the U.S. for their medical care and as such make valuable contributions to the understanding of medical science through their participation in trials and

ongoing treatment. One such patient is Maria Isabel Bueso, who participated in a clinical trial in the U.S. that helped lead to the approval of the only treatment for other patients who share her rare, fatal disorder, MPS VI. Isabel's doctors have said she will not survive without the weekly enzyme infusion therapy she still receives.

Thus and fundamentally, the deferred action program has been life-saving for Isabel and many other rare disease patients across the country. As noted by University of California San Francisco, Chancellor Sam Hawgood in a public statement released August 30, "This unilateral and unannounced policy by the Administration to shut down access to life-saving care for patients ... who are legally residing in our country, is contrary to the most fundamental notions of basic human rights."

We, the undersigned organizations dedicated to ensuring that rare disease patients have access to lifesaving therapies, ask that you ensure that migrant individuals who participate in a clinical trial or who are receiving life-saving medical treatment have a legal pathway to reside in the U.S. during the time such treatment is necessary. Action is imperative on this matter for the individuals currently receiving treatment by way of the deferred action program, vital for innovation and the development of new treatments, critical for U.S. rare disease patients, and quite simply, the humane thing to do.

Sincerely,

Aarskog Foundation
Adrenal Insufficiency United
Adult Polyglucosan Body Disease Research Foundation
Aicardi-Goutieres Syndrome Americas Association
Aidan Jack Seeger Foundation
Alabama Rare
ALD Connect, Inc.
American Society of Nephrology
Amyloidosis Research Consortium
Amyloidosis Support Groups
ANGEL AID CARES
Angelman Syndrome Foundation
APBD Research Foundation
Asbestos Disease Awareness Organization (ADAO)
Association for Creatine Deficiencies
Autoinflammatory Alliance
Barth Syndrome Foundation
Batten Disease Support and Research Association
Bonnell Foundation: Living with cystic fibrosis
Bridge the Gap - SYNGAP Education and Research Foundation

California Action Link for Rare Diseases (CAL RARE)
Canadian MPS Society
Children's Medical Research Foundation, Inc.
Coalition for Hemophilia B
Congenital Hyperinsulinism International
Congenital Muscle Disease International Registry
Courageous Parents Network
Cure GM1 Foundation
Cure Sanfilippo Foundation
CureCMT4J/Talia Duff Foundation
DADA2 Foundation
Desmoid Tumor Research Foundation
EveryLife Foundation for Rare Diseases
Foundation for Sarcoidosis Research
Friedreich's Ataxia Research Alliance (FARA)
Gene Spotlight Foundation
Genetic Alliance
Global Foundation for Peroxisomal Disorders
Global Genes
HCU Network America
HCU Network Australia
HemoAwareness Project
Hereditary Neuropathy Foundation
Hide & Seek Foundation
Hunter Syndrome Foundation
International Fibrodysplasia Ossificans Progressiva (FOP) Association
International Foundation for Autoimmune and Autoinflammatory Arthritis
International Pemphigus and Pemphigoid Foundation
International WAGR Syndrome Association
Jansens Foundation
Jeffrey Modell Foundation
Jonah's Just Begun Foundation
Kids v Cancer
LALD-Aware
Lupus and Allied Diseases Association, Inc.
Lymphedema Advocacy Group
Mastocytosis Society, Inc
Max Cure Foundation
M-CM Network
Medically Complex Children of California
MitoAction

MSD Action Foundation
MSUD Family Support Group
MTM-CNM Family Connection Inc
Mucopolysaccharidosis Type IV Foundation
Narcolepsy Network
National Gaucher Foundation
National MPS Society
National Perinatal Association
National PKU Alliance
National PKU News
National Tay-Sachs & Allied Diseases Association (NTSAD)
Neurofibromatosis Central Plains
Neuropathy Action Foundation (NAF)
Noah's Hope- Hope4Bridget
One Rare
Organic Acidemia Association
Organization for Rare Diseases India, USA
Orphan Disease Center at the University of Pennsylvania
Our Odyssey
Oxalosis & Hyperoxaluria Foundation
Parent Project Muscular Dystrophy (PPMD)
Pathways for Rare and Orphan Studies
Patients Rising Now
Phelan-McDermid Syndrome Foundation
Pompe Alliance
Pompe Warrior Foundation
Professional Patient Advocates in Life Sciences (PPALS)
Project Alive
Rare Genomics
Rare New England, INC
Rasopathies Network
Recurrent Respiratory Papillomatosis Foundation
SADS (Sudden Arrhythmia Death Syndromes) Foundation
Sickle Cell 101
Sickle Cell Thalassemia Patients Network
TargetCancer Foundation
Team Titin
Texas Rare Alliance
Thalassemia Support Foundation (TSF)
United Mitochondrial Disease Foundation
United MSD Foundation

Cc:

John M. Mitnick, General Counsel, DHS

Christina Bob, Executive Secretary, DHS

Brandan Wales, Acting Chief of Staff, DHS

Kenneth Cuccinelli, U.S. Citizenship and Immigration Services, Acting Director

Matthew T. Albence, U.S. Immigration and Customs Enforcement, Acting Director