April 5, 2023

Michelle Baass
Director of California Department of Health Care Services
Department of Health Care Services
1501 Capitol Avenue, MS 0000
Sacramento, CA 95814

RE: MediCal Reinstatement and Transition Policy

Dear Director Baass,

Leading advocacy groups work towards extending treatment options to patients living with rare diseases. Ensuring equitable and effective rollouts with MediCal reinstatement and transition policy is a priority for the rare disease community. Many face significant healthcare disparities including access to quality medical coverage that is further exacerbated by limited and costly therapeutic options available for life-sustaining interventions. Furthermore, significantly elevated premiums and restrictions continue to isolate meaningful access to quality and affordable care.

The Department of Health Care Services announced a strategy in 2022 on Medi-Cal reinstatement policy. This rollout has only recently become known to many of those in our communities and their healthcare providers leaving delayed timing to efficiently understand the policy changes on how this negatively impacts our community. Restricting access to care by imposing utilization management policies, such as prior authorizations, step therapy and quantity limits extends unfavorable healthcare outcomes and jeopardizes quality of life. Fostering these implementation strategies creates undue burdens to individuals who rely on these medications. These burdens can directly reduce medication compliance, result in increased hospitalizations and emergency department encounters, that contribute to a delay in treatment necessary to control of disease manifestations that have profound irreversible long-term consequences.

As leaders and caregivers in this community, we strongly believe these policy changes will have a negative impact by further restricting the already limited FDA approved medications by placing unnecessary burdens to secure medications required to optimize disease management and improve quality of life. We encourage you to ensure transparent access changes to benefit plans and formularies, as well as transparent and expedited appeals processes and grievances for denials, along with special enrollment procedures when patients lose access to medications due to formulary changes during a plan year.

Rare diseases have extensive, and often severe comorbidities. Additionally, many rare diseases are progressive and cause physical, developmental, and cognitive disabilities. Early prevention and intervention are paramount. Families affected by rare diseases live daily with conditions including cardiopulmonary dysfunction; neurological deficits; debilitating seizures; chronic kidney disease; immunosuppression; failure to thrive; loss of muscle tone and coordination; muscle atrophy; chronic pain; vision and/or hearing loss; gastrointestinal issues; intellectual and learning disabilities; autism spectrum disorder; and organ failure. It is estimated 25-30 million individuals in the United States have a rare disease, of which many are life-threatening with limited or no treatment options. Because those with rare diseases are often complex and have complicated co-morbidities, the priority of care management shifts between acute and chronic management options. This rapidly expands costs between different drug classification categories which with the proposed policy change increase delay in care. As we understand the

dilemmas payers are required to navigate by keeping healthcare costs down and therapeutic options available, for those with rare diseases we must keep the focus on optimizing medication access to reduce the healthcare costs by decreased hospitalizations and emergency department encounters.

We welcome the opportunity to discuss our request further. For questions, please contact Ashley Pounders, MSN, FNP-C, Director of Medical Affairs at the TSC Alliance, apounders@tsalliance.org or 1-240-472-4302.

On behalf of patients and families affected by rare diseases, we appreciate your time and continued advocacy efforts regarding the negative impact this implementation has on those in our communities.

Respectfully submitted in alphabetical order,
ARDS Alliance, Inc.
Child Neurology Foundation
CSNK2A1 Foundation
Cystic Fibrosis Research Institute
Developmental and Epileptic Encephalopathies (DEE-P CONNECTIONS)
Dravet Syndrome Foundation
Dup15q Alliance
International SCN8A Alliance
Lennox-Gastaut Syndrome (LGS) Foundation
Phelan-McDermid Syndrome Foundation
Project 8p Foundation
Ring14 USA
STXBP1 Foundation
SYNGAP1 Foundation
The LAM Foundation
The PAP Foundation
The PCD Foundation
The SNAP25 Foundation
TSC Alliance