RARE READY
A GENETIC CONDITION COALITION

June 24, 2022

Attn: Melanie Reid
State of Georgia
Sent via email to: CMO.RFP@dch.ga.gov

To Whom It May Concern:

As a statewide grassroots advocacy coalition of 25 non-profit rare disease organizations called Rare & Ready: A Genetic Condition Coalition, we believe action must be taken to improve timely access to FDA-approved medicines in Medicaid for those with rare and genetic conditions. Our efforts are not disease- or treatment-specific; rather, we seek to address concerns of the broader rare community.

We are pleased to submit comments in response to eRFI (Event) Number: 41900-DCH0000127. You are seeking information from stakeholders with direct experience in the managed health and long-term care industries, and we felt it necessary to share our priorities as patients, parents, and caregivers. We have first-hand experience regarding best practices in service delivery for Medicaid managed care.

Medicaid managed care organizations (MCOs) should provide the same coverage as Medicaid fee-for-service (FFS), and we advocate for three key principles:

1) Timely, meaningful access to FDA-approved therapies
2) Coverage for telehealth services with out-of-state providers
3) Access to gene therapies through outcome-based arrangements

Timely, Meaningful Access to FDA-Approved Therapies
Patients with rare and genetic disorders need and deserve timely access to FDA-approved treatments. We support four priorities to mitigate the programmatic access hurdles placed by Medicaid FFS and Medicaid MCOs.

1. Patients with rare conditions served by Medicaid should have timely access to new FDA-approved therapies. Members of the rare community often have limited or no therapeutic options or have progressive diseases that are urgent to treat, and need timely access to innovative, clinically promising, approved drugs regardless of what pathway they are approved under by the FDA.
2. Medicaid FFS and Medicaid MCOs should have an expeditious and transparent process for both clinical reviews and developing coverage criteria for new drugs for rare conditions.
3. Patient access to therapies for rare conditions in Medicaid FFS and in Medicaid MCOs must not be limited by coverage criteria that are more restrictive than the indications and usage section of the FDA-approved label.
4. Step therapy exceptions for rare disease therapies should be provided.

Coverage for Telehealth Services with Out of State Providers
In the midst of widespread lockdowns during the COVID-19 pandemic, virtual medical visits helped patients get the care they needed. Telehealth access particularly benefitted those with rare conditions, who may have increased health risks every time they leave their home and who often found the rare diseases specialists they need to be unavailable or at capacity. With a limited number of medical experts available

who truly understand rare conditions, telehealth across state lines is critical to accurate diagnosis and good disease management. For rare disease patients, good treatment is oftentimes far from home, and may even be in another state.

Access to Gene Therapies through Outcome-Based Arrangements
For many diseases, gene therapies have the potential to provide transformative care for many diseases—including rare and genetic conditions—and access must be assured. By basing reimbursement on whether a gene therapy worked for a specific patient over a set time period, outcomes-based arrangements (OBAs) offer promise as a cost management tool. OBAs are particularly applicable to gene therapies for rare diseases, where the pipeline is robust and the need is great. The U.S. federal government has taken steps through rulemaking to enable these arrangements, but barriers remain. Some states are leading the way through Medicaid State Plan Amendments (SPAs) that allow for the reimbursement of pharmaceuticals utilizing OBAs through supplemental rebate agreements.

The decisions you make affect real people, many of them living with a life-altering rare disease and coping with the barriers that MCOs create. We spend countless, precious hours on the phone trying to get therapies or services approved. Some of us are parents who have children with fatal diseases. We quite literally do not have hours to waste.

As you are reviewing the comments, we respectfully ask you to please keep us in mind. We ask you to listen to our stories and act to ensure patient access to life-changing therapies.

Thank you,

Angelman Syndrome Foundation, Inc.
Batten Disease Support & Research Association (BDSRA)
Bleeding Disorders Alliance Illinois
Caring Board
Children's Medical Research Foundation
Columbus Children's Foundation
Fighting H.A.R.D. Foundation
Hunter’s Hope Foundation, Inc.
Hypertrophic Cardiomyopathy Association
Juju and Friends CLN2 Warrior Foundation
Kids Conquering SCD
LGS Foundation
Little Hercules Foundation
Magic Foundation
Mid Atlantic Connection for PKU and Allied Disorders (MACPAD)
MPS Superhero Foundation
National MPS Society
National Niemann-Pick Disease Foundation (NNPDF)
Noah's Hope
Rare Epilepsy Network
Sickle Cell Reproductive Health Education Directive
STAC3.org