



## Patients with Rare and Genetic Disorders Deserve Timely Access to New Treatments

Many **state Medicaid programs** are developing detrimental strategies to limit access to newly approved rare disease therapies.

The Rare & Ready Genetic Condition Coalition supports **four priorities** to mitigate the programmatic hurdles to access put into place by Medicaid fee-for-service (FFS) and Medicaid managed care organizations (MCOs).

### Patients with rare conditions served by Medicaid must have timely access to new FDA-approved “qualifying rare disease therapies”

- Approved with the Orphan Drug Designation; **or**
- Approved for the treatment of rare, pediatric diseases; **or**
- Designated as a breakthrough therapy; **or**
- Designated as a regenerative medicine advanced therapy

# 1

### Medicaid FFS and Medicaid MCOs must have a specific timeframe and transparent processes for clinical review and setting coverage criteria for new drugs

- Within 60 days of FDA approval, Medicaid must conduct clinical review as well as development (and publishing) of coverage criteria for qualifying rare disease therapies
- Manual reviews must be available to ensure coverage immediately after approval and during the 60-day window

# 2

### Patient access in Medicaid FFS and Medicaid MCOs must not be limited by coverage criteria that are more restrictive than the FDA label

- Inclusion/exclusion criteria from clinical trials must not be permitted as a basis for coverage criteria
- Coverage criteria must be consistent with the medically accepted indication
- Coverage must not be more restrictive than the indications and usage section of the drug's label approved by the FDA

# 3

### Exceptions to step therapy for qualifying rare disease therapies are needed

- A clear exceptions process to override a step therapy protocol imposed for certain drugs must be in place
- Medical necessity of a drug must be determined by providers – not Medicaid
- A response to an exceptions request must be provided within 24 hours for an urgent request and 72 hours for a non-urgent request

# 4

If you agree with these principles, **join the Rare & Ready Coalition today: [rareandready.org](https://rareandready.org)**  
Join the conversation and tell your rare disease story on X [@rare\\_ready](https://twitter.com/rare_ready)