

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

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

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

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

If you agree with these principles, **join the Rare & Ready Coalition today!**

Join the conversation and tell your rare disease story on  [@rare_ready](https://twitter.com/rare_ready)