

Patients with Rare and Genetic Disorders Deserve Timely Access to Gene and Cell Therapies

Many state Medicaid programs do not have policies that will help patients get **access** to transformative treatments like gene and cell therapies. **Outcomes-based arrangements** may be part of the solution.

For many rare conditions, medical progress is moving from treating symptoms to actually addressing the underlying cause of the disorder through gene and cell therapies. To ensure patient access, we need reimbursement models – like outcomes-based arrangements (OBAs) – that are equally as innovative as the therapies themselves.

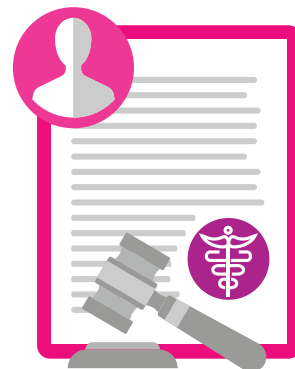


Why OBA's?

- **Risk-sharing** – OBAs tie reimbursement to pre-determined clinical outcomes. If the therapy does not work or is not as durable as intended for an individual patient, the pharmaceutical company would be contractually obligated to provide a rebate or partial refund to the payer.
- **Cost savings to the healthcare system** – Gene and cell therapies are intended to be durable treatments so their cost will be offset by expenses that would otherwise be incurred by the patient for standard treatment.
- **Access for patients** – By mitigating some of the risk and costs borne by the payer, OBAs should facilitate patients' access to these life-altering therapies.

How Can State Medicaid Programs Use OBAs for Gene and Cell Therapies?

- **Centers for Medicare & Medicaid Services (CMS) Regulation:** Under a new CMS *federal regulation* effective July 1, 2022, state Medicaid programs may opt into OBAs offered by pharmaceutical companies so they can get money back if a patient does not respond to treatment and get the best price offered to private insurers. This regulatory pathway is called the "multiple best price approach."
- **Medicaid State Plan Amendment (SPA):** In the alternative, a state can choose to submit a State Plan Amendment (SPA) to CMS. A SPA is a request to the federal government for changes in how a state administers its Medicaid program. Through a SPA, a state can seek explicit approval from the agency to enter into an OBA via an approved template. (A SPA is not necessary with the CMS multiple best price approach.)
- **Legislation:** Some states that decide to pursue a SPA will require legislation either because (1) they need a new law, or (2) the Medicaid agency wants the OK from the legislature prior to adopting OBAs. A great model for legislation passed in Texas in 2019.



What Can I Do?

- **Talk to State Medicaid Plan Officials.** Tell them about the importance of gene and cell therapies and how OBAs will help the state Medicaid program ensure patient access. Encourage the state Medicaid program to enter into OBAs offered by pharmaceutical companies either under the CMS regulation or a SPA.
- **Advocate with Legislators.** Ask them to encourage Medicaid to implement the CMS multiple best price regulation or move forward with a SPA. If Medicaid doesn't take action, request that they sponsor legislation similar to Texas SB 1780 (2019).

If you agree that policy changes are needed to help patients get access to gene and cell therapies, **join the Rare & Ready Coalition today!**

Join the conversation and tell your rare disease story on X @rare_ready

