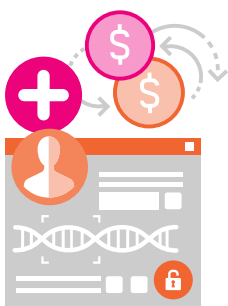


Patients with Rare and Genetic Disorders Deserve Timely Access to Gene and Cell Therapies

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 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.

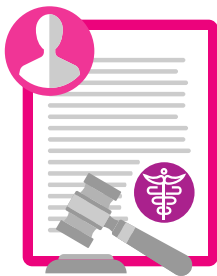


Why OBA's?

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 CMS *federal regulation* effective July 1, 2022, state Medicaid programs may opt into OBAs offered by pharmaceutical companies. This regulatory pathway is called the "multiple best price approach."

Medicaid State Plan Amendment (SPA): A state can choose to submit to CMS a SPA, 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.

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 approval from the legislature prior to adopting OBAs.



What Can I Do?

➔ Join the Rare & Ready Coalition! Connect with our network of rare disease advocates to ensure patients have access to gene and cell therapies.