



Medicaid: The Growing Imperative to Reform Patient Access and Participation

When the Medicaid program was instituted in 1965, the revolution in targeted rare disease medicines did not exist. However, in the 1970s and the early 1980s, the imperative for these medications galvanized policymakers to create approval pathways and incentives that would unleash investment within the life sciences community. Thus the Orphan Drug Act of 1983 was born.

In 2016, the 21st Century Cures Act drove patient centricity into drug development by requiring patient input, from the earliest stages through drug approval. This focus offers the companies and the Food and Drug Administration (FDA) insights into disease, patient journey pre and post diagnosis, and critical insights into clinically relevant end points necessary for approval. This patient centricity is needed to bring Medicaid rare disease decision making into the 21st Century.

Medicaid drug coverage requirements

The federal Medicaid rebate statute requires that, as a condition of Medicaid coverage, drug manufacturers pay rebates on Medicaid fee-for-service (FFS) and managed care utilization of their "covered outpatient drugs". In exchange for these rebates, Medicaid programs must cover the manufacturer's outpatient drugs according to the drug's "medically accepted indication," or in other words the FDA approved indication.¹ While many states contract with managed care organizations (MCOs) to provide coverage for all or part of their Medicaid populations, CMS requires that these MCOs provide coverage according to the standards set in the Medicaid rebate statute.²

Over the years, the Centers for Medicare & Medicaid Services (CMS) has reminded Medicaid programs of drug coverage obligations through technical guidance due to ongoing patient access challenges. For example, on June 27, 2018, CMS reminded programs that drugs approved under FDA's accelerated approval pathway, which expedite access to novel therapies that fill an unmet medical need³, meet the definition of a covered outpatient drug and must be covered.⁴

¹ SSA § 1927(k)(6)

² 42 CFR .§ 438.3(s)(1)

³ "[F]our FDA programs are intended to facilitate and expedite development and review of new drugs to address unmet medical need in the treatment of a serious or life-threatening condition: fast track designation, breakthrough therapy designation, accelerated approval, and priority review designation." FDA, Guidance for Industry, Expedited Programs for Serious Conditions -Drugs and Biologicals (May 2014) (Expedited Pathways Guidance), at 1.

⁴ CMS Medicaid Drug Rebate Program Notice, Release No. 185. State Medicaid Coverage of Drugs Approved by the FDA under Accelerated Approval Pathway. June 27, 2018. Available



State Medicaid Drug Review Process

While Medicaid is vital to rare disease patients, many states do not include specialists and patients in their decision-making. For example as current practice in many states, a contract vendor schedules a Pharmacy and Therapeutics Committee (P&T Committee) or a Drug Utilization Review (DUR). If at the meeting the disease specialist in the state could not attend, the vendor often proceeds to provide an overview of the disease, recommendations for program guidance, including use of information that may or may not be supported by the disease specialist, the label or the patient experience with the disease.

In addition, some Medicaid programs employ a review process that can take between 180 to 365 days (or longer) to conclude, before there is any real access to a rare disease medicine. This is unacceptable when many of those rare diseases are life threatening, and patients, many of whom are pediatric, do not have the luxury of time to wait for Medicaid to make a product available after a year or two of delay. Rather, FDA-approved drugs with a rebate agreement must be covered from the outset; there is no proscribed legal period to delay coverage based on the theory that a state must review it or make a coverage determination. So, while a state may review a drug through its P&T process, that drug must be made available prior to and post review according to its medically accepted indication. CMS included guidance regarding these statutory obligations in the preamble to its 2016 Medicaid managed care organization (MCO) final rule, making clear the imperative for immediate coverage of new drugs of a manufacturer with a rebate agreement.⁵

Medicaid programs, through P&T processes, may impose prior authorization requirements on drugs, provided they respond to requests within 24 hours and dispense a 72-hour supply of the drug in an emergency.⁶ However, prior authorization cannot be used to deny coverage for a drug's medically accepted indication, including its FDA-approved indication. Further, federal law does not permit a program to deny access to medically accepted indications of covered drugs based on state medical necessity laws, regulations, coverage determinations or the use of other utilization tools.

at: <https://www.medicaid.gov/medicaid-chip-program-information/by-topics/prescription-drugs/downloads/rx-releases/state-releases/state-rel-185.pdf>

⁵ 81 Fed. Reg. 27498, 27551 (May 6, 2016).

⁶ SSA § 1927(t)(1)(A)(5). States may also create Preferred Drug Lists (PDLs), lists of drugs that are not subject to prior authorization, and may seek supplemental rebates for PDL inclusion. CMS recently explained, "In general, [Medicaid beneficiaries] may not be denied access to covered outpatient drugs of manufacturers participating in the drug rebate program when such drugs are prescribed for a medically accepted indication. However, to determine whether the drug is prescribed for a medically accepted indication for the individual, the state or managed care plan may subject any covered outpatient drug to prior authorization as long as the prior authorization program meets the minimum requirements at section 1927(4)(5) of the Act." 81 Fed. Reg. 27498, 27553 (May 6, 2016).



SOLUTIONS

Our recommendations focus on bringing important advances to patients in need, and enhancing the spirit of the Medicaid rebate agreement program and the patient centricity of the 21st Century Cures Act.

1. State programs must provide access based on the Medicaid rebate agreement.
 - a. Rare disease treatments must be covered from the outset, once a product comes to market, according to its FDA-approved use.
 - b. States choosing to review an approved rare disease therapy and to develop prior authorization criteria must do so within 90 days of approval while providing coverage during that time according to the FDA-approved use.
 - c. Review of an approved therapy must not utilize state resources to reassess the safety and effectiveness as it would be redundant to and could conflict with the responsibilities of the FDA under the FDCA.
 - d. Coverage policies must be made public within 90 days and conform to the medically accepted indication and FDA-approved label.
2. State Medicaid decision-making bodies must include a disease specialist, as well as a patient/family participant as members and make accommodations for patient testimony to be provided via telephone and/or webcast when a representative is unable to attend a P&T meeting in person.

ABOUT RAAP

The Rare Access Action Project (RAAP) began in 2017 as an ad hoc coalition of life sciences and patient stakeholders with interests in advocating for solutions to issues that limit patient access to health care. Many rare disease patients, upon a diagnosis, believe that because they have coverage (commercial, Medicaid or Medicare) that they will be able to utilize the medicine or technology that was developed for their disease. Unfortunately, this is not always the case. The reality can often be much different. After rounds of prior authorizations and appeals, patients are left with uncertainty.

RAAP explores creative solutions to address issues in access and coverage to help ensure rare disease patients have access to the care and treatments that they need. You can find us at www.rareaccessactionproject.org or contact us at rareaccessproject@gmail.com.