



High-Cost Specialty Drugs: Moving Towards Recommendations

Medicaid and CHIP Payment and Access Commission

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Overview

- Accelerated approval drugs
- Differential rebate
- Implications
- Options for recommendations
- Next steps

Accelerated Approval Drugs

- Can be approved based on a surrogate endpoint that is reasonably likely to predict a clinical benefit, but clinical benefit has not been verified
- Manufacturer must conduct post-market clinical trial to confirm clinical benefit
 - Confirmatory trials often delayed; many take over five years to complete
- Medicaid Drug Rebate Program (MDRP) requires states to cover accelerated approval drugs
 - States must pay for therapies while additional studies are still needed to verify clinical benefit

Differential Rebate

- Increase minimum rebate
 - Reduce Medicaid spending while there is limited evidence of clinical effectiveness
 - Create incentive for manufacturers to complete confirmatory trial
- Increase inflationary rebate if confirmatory trial not completed after a set number of years
 - Mitigate price increases while awaiting completion of confirmatory trial
- Once manufacturer completes confirmatory trial and receives traditional approval, the rebates would revert to the standard amount

Implications

- Manufacturers are likely to argue that additional Medicaid rebates may discourage research and development
 - Need to weigh the cost of the additional rebates with the benefit of early market access
 - Could build higher rebate into launch price
- Beneficiaries would maintain similar access once drug enters market, but may lose early access to some products if manufacturers decide to forego accelerated approval pathway
- Federal and state spending would decrease as a result of higher rebates

Potential Recommendation 1

- Congress should amend Section 1927(c)(1) to increase the minimum rebate percentage on drugs that receive approval from the U.S. Food and Drug Administration (FDA) through the accelerated approval pathway under Section 506(c) of the Federal Food, Drug, and Cosmetic Act. This increased rebate percentage would apply until the manufacturer has completed the confirmatory trial and been granted traditional FDA approval. Once the FDA grants traditional approval, the minimum rebate percentage would revert back to the amount listed under Section 1927(c)(B)(i).

Potential Recommendation 2

- Congress should amend Section 1927(c)(2) to increase the additional inflationary rebate on drugs that receive approval from the U.S. Food and Drug Administration (FDA) through the accelerated approval pathway under Section 506(c) of the Federal Food, Drug, and Cosmetic Act. This increased inflationary rebate would go into effect if the manufacturer has not yet completed the confirmatory trial and been granted traditional FDA approval after a certain number of years. Once the FDA grants traditional approval, the inflationary rebate would revert back to the amount typically calculated under Section 1927(c)(2).

Next Steps

- Decide whether to proceed with Recommendation 1 only or both Recommendations 1 and 2
- Vote at the April public meeting and a chapter for inclusion in the June report

Summary of Potential Recommendations

1. Increase minimum rebate amount on accelerated approval drugs. Rebate would revert back to standard amount once the FDA grants traditional approval.
2. Increase inflationary rebate on accelerated approval drugs if the manufacturer has not yet completed the confirmatory trial within a certain number of years. Rebate would revert back to standard amount once the FDA grants traditional approval.



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