



High-Cost Specialty Drugs: Review of Draft Chapter and Recommendations

Medicaid and CHIP Payment and Access Commission

Chris Park

Overview

- Draft chapter for the June report to Congress
 - High-cost specialty drugs
 - Developing new models
 - Accelerated approval drugs and recommendations
 - Cell and gene therapies
- Draft recommendations on accelerated approval drugs
 - Rationale
 - Implications

High-Cost Specialty Drugs

- From 2010 to 2015, net spending on specialty drugs in Medicaid almost doubled from \$4.8 billion to \$9.9 billion
- Medicaid fee-for-service (FFS) net cost per claim for traditional drugs fell by 0.4 percent from 2018 to 2019 while the net cost per claim for specialty drugs increased 8.6 percent
- In 2019, specialty drugs accounted for 48.5 percent of FFS pharmacy spending but only 1.3 percent of drug utilization
- States are seeking new strategies because the current utilization management tools permitted under Medicaid law are ineffective in containing costs for high-cost specialty drugs

Developing New Models

- Convened technical advisory panel (TAP) to assist in developing new payment and coverage models
 - Identify the types of drugs in the pipeline
 - Identify what challenges each type of drug presents
 - Develop potential policy options to address the specific challenges of each particular drug type
- TAP included drug policy and pricing experts from academia and private sector; state Medicaid and federal officials; beneficiary advocates; providers; health plans; and drug manufacturers

Challenges and Potential Solutions by Drug Type

Accelerated approval drugs

Challenges

Solutions

Clinical benefit not verified

Targeted closed formulary

Differential rebate

Value-based payment

Outcomes-based contract

Increased FMAP

Cell and gene therapies

Challenges

Solutions

High upfront cost

Budget volatility

Uncertain long-term benefit

New national benefit

Risk pool

Value-based payment

Outcomes-based contract

Increased FMAP

Pay over time

Drugs for sensitive populations

Challenges

Solutions

Limited negotiating power

Targeted closed formulary

Value-based payment

New Benefit for Cell and Gene Therapies

- Carve-out coverage of cell and gene therapies from the Medicaid Drug Rebate Program (MDRP) into a new benefit
- Allows for new coverage, payment, or rebate requirements without disrupting the existing structure of the MDRP
- Design options
 - Participation
 - Inclusion criteria
 - Price
 - Supply chain
 - Duration
 - Funding
- Stakeholder implications

Draft Recommendations

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
- The FDA has acknowledged that using surrogate endpoints creates a risk that patients will be exposed to a drug that ultimately will not be shown to provide an actual clinical benefit
- Manufacturer must conduct postmarketing clinical trial to confirm clinical benefit
 - Confirmatory trials often delayed; many take over five years to complete
- States are concerned about being required to cover accelerated approval drugs and paying high prices when clinical benefit has not been verified

Draft Recommendation 1

- Congress should amend Section 1927(c)(1) of the Social Security Act 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 postmarketing 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)(1)(B)(i).

Draft Recommendation 2

- Congress should amend Section 1927(c)(2) of the Social Security Act 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 postmarketing confirmatory trial and been granted traditional FDA approval after a specified 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).

Rationale

- Medicaid pricing policy that does not affect FDA authority or processes
- Lowers net price until manufacturer verifies clinical benefit
- Maintains coverage requirement
- Provide financial incentive for manufacturer to complete confirmatory trial in a timely manner
- Increased inflationary rebate would help mitigate any increases in list price that occurs before confirmatory trial is completed

Implications – Manufacturers

- Manufacturers argue that additional Medicaid rebates may discourage research and development or delay availability
 - Medicaid rebate is just one of many factors when making a decision on a product's development and launch
 - Increase in Medicaid minimum rebate under the Patient Protection and Affordable Care Act did not appear to decrease drug development in the aggregate
- Manufacturers control price and could possibly increase the launch price or attempt to raise costs on other payers
- Accelerated approval pathway still has benefits as it would provide earlier access to the market and allow the drug to generate revenue and establish market share while the confirmatory trial is underway

Implications – Beneficiaries

- Beneficiaries would maintain Medicaid coverage once drug enters market in contrast to state requests to exclude coverage (e.g., Tennessee Section 1115 demonstration)
- Beneficiary advocates have expressed concerns that access to innovative therapies could be decreased if manufacturers reduce research and development in, or delay the availability of, new therapies
- Could increase access if states are willing to reduce coverage and prior authorization restrictions when cost is lower

Implications – Spending

- Federal and state spending would decrease as a result of higher rebates
- CBO score assumed a 10 percentage point increase in the minimum rebate and a 20 percent increase in the inflationary rebate if the manufacturer has not completed confirmatory trial after 5 years
 - Savings between \$0–50 million in federal spending in first year
 - Savings between \$0–1 billion in federal spending over five years
- Gross Medicaid spending (i.e., before rebates) on accelerated approval drugs in FY 2019 was approximately \$1.0 billion (includes both federal and state spending)



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