Addressing High-Cost Drugs and Pipeline Analysis

Medicaid and CHIP Payment and Access Commission
Amy Zettle and Chris Park

Overview

• Background
  – Commission’s past work on specialty drugs
  – Spending on specialty drugs
• Technical advisory panel
  – Key policy questions
• Pipeline analysis
Background

• Specialty drug spending is a growing share of Medicaid budgets
  – 12 of the top 20 Medicaid drugs by spending are specialty drugs
  – Specialty drug spending is growing at a faster rate than traditional drugs
• Experts from 2019 roundtable largely agreed that specialty drugs can be harder for states to manage

Technical Advisory Panel

• October meeting: specialty drug pipeline
  – To what extent will high-cost specialty drugs in the pipeline have a disproportionate or significant effect on Medicaid?
  – What challenges will these pipeline drugs likely present?
• November meeting: model design
  – What changes to Medicaid payment and coverage policies could help address these challenges?
• December meeting: model effects
  – What are the operational barriers and effects on stakeholders?
Priority Specialty Pipeline Drugs for Medicaid

**Type 1: Expensive Pediatric**
Pediatric gene and cell therapies plus high incremental spending (Phase I-III).

**Type 2: Adult Gene and Cell Therapy**
Extremely expensive therapies for adult indications with significant Medicaid volume (Phase III).

**Type 3: Other High Budget Impact**
Other specialty drugs with large Medicaid use, high cost, and incremental spending (Phase III).

**All Other Products**
Specialty products that may drive spending but do not have a disproportionate impact on Medicaid.
The Pediatric Pipeline Includes Gene Therapies for Sickle Cell Disease and Blood Cancers

- There are >180 drugs in the pipeline with pediatric indications, across all phases of development
  - 45 are gene or cell therapies—3 in Phase III trials and 4 in Phase II with expedited approval

**Pediatric Medicaid Prevalence**
(per 100,000 children)

<table>
<thead>
<tr>
<th>Condition</th>
<th>Prevalence (per 100,000 children)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Sickle Cell Disorders</td>
<td>182.7</td>
</tr>
<tr>
<td>Leukemia</td>
<td>33.3</td>
</tr>
<tr>
<td>Muscular Dystrophy</td>
<td>24.4</td>
</tr>
<tr>
<td>Lymphoma</td>
<td>16.1</td>
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<tr>
<td>Achromatopsia</td>
<td>1.5</td>
</tr>
<tr>
<td>Cystic Fibrosis</td>
<td>34.4</td>
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</tbody>
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Adult Gene Therapies for Diabetes, Rheumatoid Arthritis, Multiple Sclerosis, and Hemophilia Could Be Top Cost Drivers

- While only a fraction of patients with these diagnoses will be eligible for gene therapies, their high list prices are likely to drive Medicaid spending.

**Medicaid Prevalence**
(per 100,000)

<table>
<thead>
<tr>
<th>Condition</th>
<th>Prevalence (per 100,000)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Type 1 Diabetes</td>
<td>563</td>
</tr>
<tr>
<td>Rheumatoid Arthritis</td>
<td>346</td>
</tr>
<tr>
<td>Multiple Sclerosis</td>
<td>218</td>
</tr>
<tr>
<td>Hemophilia</td>
<td>49</td>
</tr>
</tbody>
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There Are Many Pipeline Products to Treat Cancer

Medicaid Prevalence of Cancer, by Tumor Type (per 100,000)

Products With Expedited Approval Can Be Particularly Challenging for States

44% of Phase III specialty drugs are on an expedited approval pathway

- **Speed:** These products move through the approval process more quickly
- **Endpoints:** Accelerated-approval drugs may rely on surrogate endpoints or be approved with broader indications
- **Post-Market Surveillance:** Most accelerated-approval drugs are required to complete post-market clinical trials
Three Priority Drug Types That Are Challenging for Medicaid to Manage With Existing Tools

- Gene and Cell Therapies
  - High List Prices & Up-Front Costs
  - Budget Volatility
  - Long-term Benefit of Investment

- Accelerated-Approval Drugs
  - Limited Evidence

- Drugs for Sensitive Populations
  - Lack of Negotiating Power

Thank you.

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