



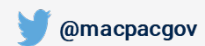
# Addressing High-Cost Drugs and Pipeline Analysis

**Medicaid and CHIP Payment and Access Commission**

Amy Zettle and Chris Park

October 30, 2020

[www.macpac.gov](http://www.macpac.gov)



## Overview

- Background
  - Commission's past work on specialty drugs
  - Spending on specialty drugs
- Technical advisory panel
  - Key policy questions
- Pipeline analysis

October 30, 2020



# 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

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# 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?

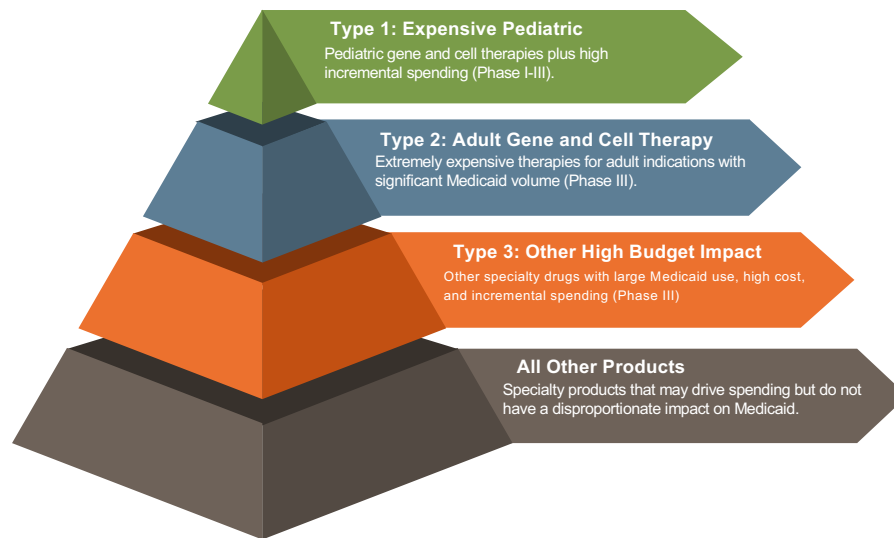
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# Priority Specialty Pipeline Drugs for Medicaid

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## Priority Specialty Pipeline Drugs for Medicaid

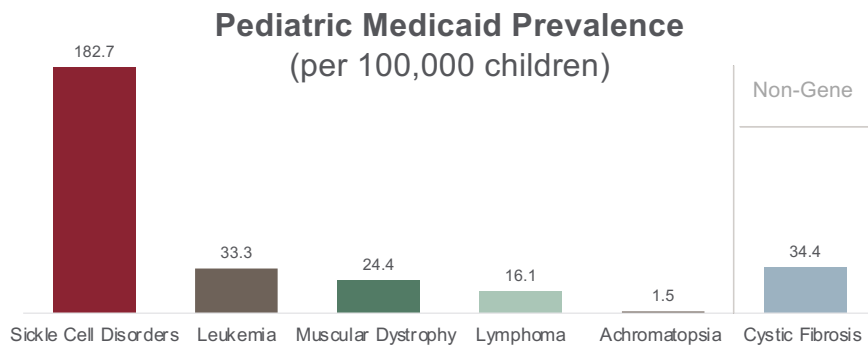


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## The Pediatric Pipeline Includes Gene Therapies for Sickle Cell Disease and Blood Cancers

- 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

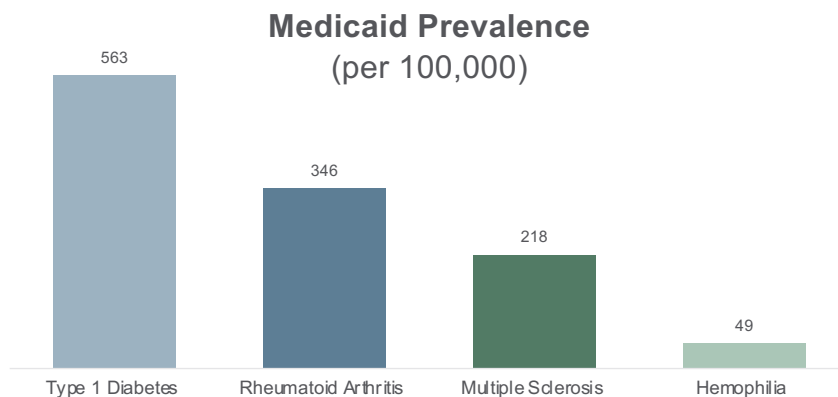


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## Adult Gene Therapies for Diabetes, Rheumatoid Arthritis, Multiple Sclerosis, and Hemophilia Could Be Top Cost Drivers

- 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.

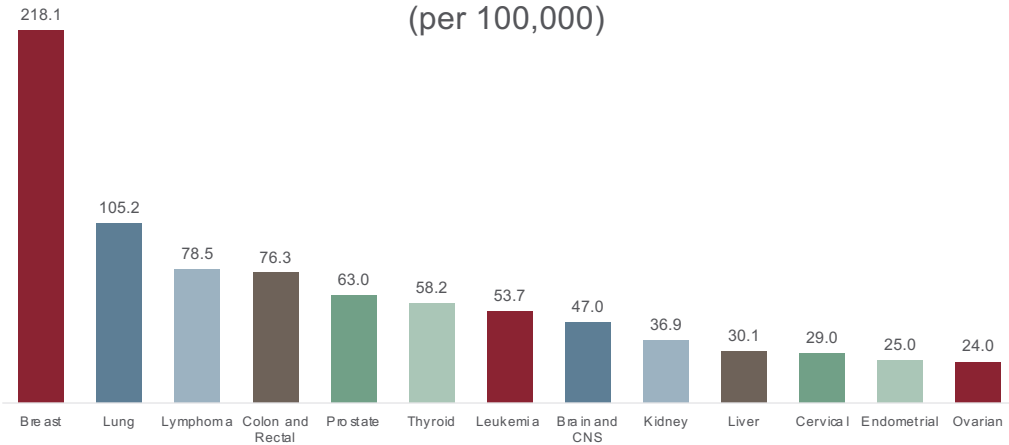


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## There Are Many Pipeline Products to Treat Cancer

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

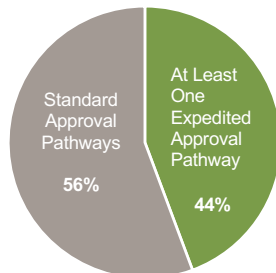


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## Products With Expedited Approval Can Be Particularly Challenging for States

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

Phase III Specialty Drugs on an FDA Expedited 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

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## 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

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# Thank you.

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