

Medicare Spending on Orphan Drugs with Single and Multiple Rare Disease Indications

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INTRODUCTION

- The Inflation Reduction Act (IRA), passed in 2022, allows the Centers for Medicare & Medicaid Services (CMS) to negotiate maximum fair prices for certain prescription drugs covered in the Medicare Drug Price Negotiation Program.¹
- Orphan drugs are excluded from negotiation, but only if they treat a single rare disease or condition, and the sole approved indication is for that specific disease.²
- Of orphan drugs that treat more than one condition, some treat exclusively rare disease indications while others also have indication(s) that treat more common diseases.

OBJECTIVES

- To identify orphan drugs that treat a single rare disease, multiple rare diseases, or non-rare diseases.
- To assess the proportion of Medicare expenditures for orphan drugs and stratify by those with exclusively rare disease indications compared to those with some non-rare disease indications.
- To understand trends over time in proportionate Medicare expenditures on Orphan Drugs.

CONCLUSIONS

- Spending on Orphan Drugs in Medicare Part B and Part D was concentrated primarily in Multi-Non-Rare Orphans and not in Multi-Rare Orphans.
- The proportion of Medicare drug spend attributable to Orphan Drugs is stable over time.
- Most of the top drugs with the highest Medicare drug spend were Non-Orphan Drugs, followed by Multi-Non-Rare Orphans.

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METHODS

STUDY DESIGN

- Design:** Retrospective, non-interventional study of pharmaceutical spend
- Data:** 100% Medicare Part B Fee-For-Service (FFS) claims data and 100% Medicare Part D claims data
- Study Period:** January 1, 2022 to December 31, 2022

OUTCOMES

- The proportion of Medicare drug spend that is associated with orphan drugs
- The proportion of Medicare total (medical + drug) spend that is associated with orphan drugs
- Time trends of proportion of Medicare spending on orphan drugs, from 2018-2022

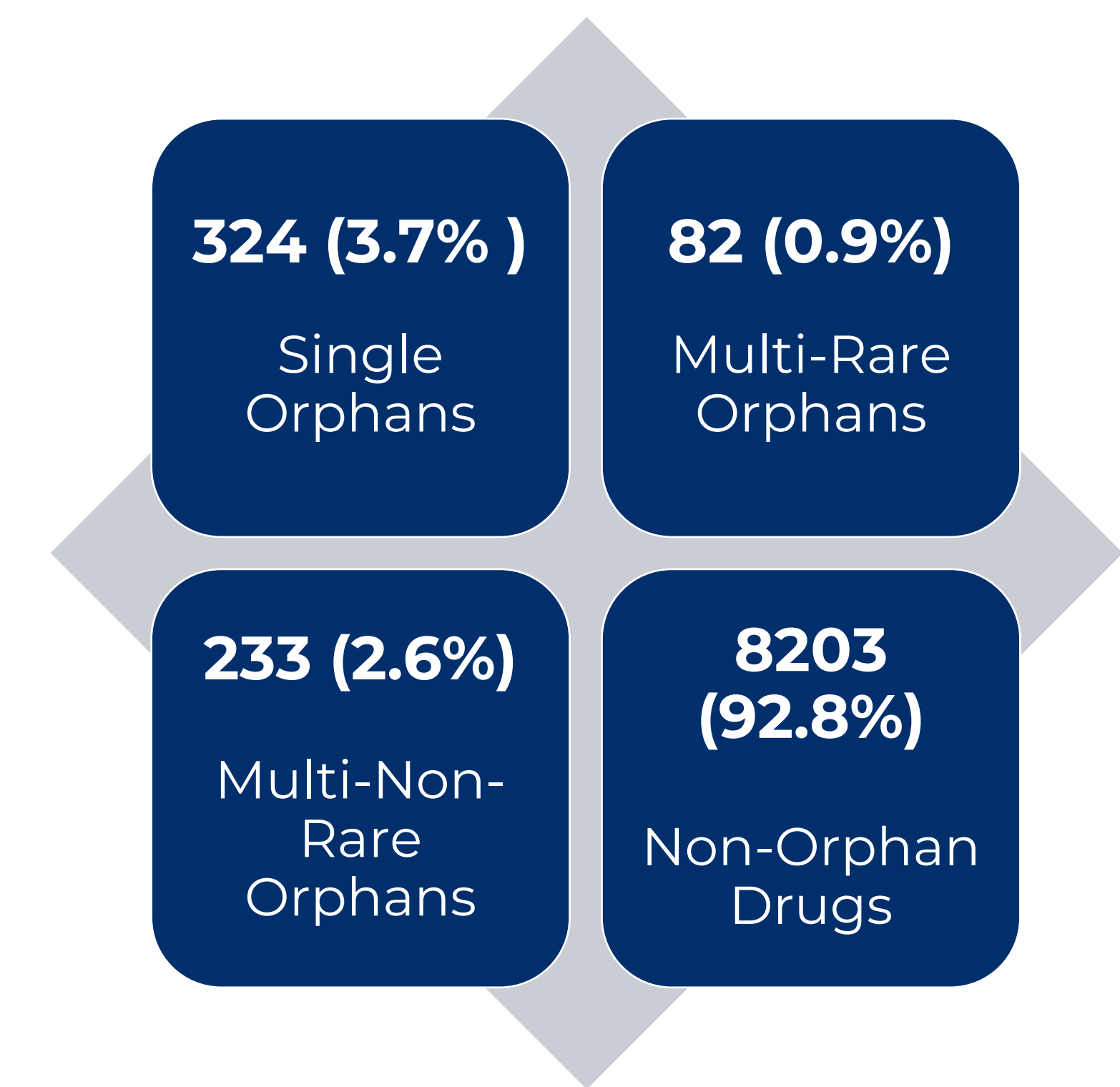
DRUG CATEGORIZATION

We categorized drugs into the following groups based on the FDA's Labeling database:³

- **Single Orphans:** orphan drugs that have a single indication
- **Multi-Rare Orphans:** orphan drugs with multiple indications where all are rare diseases
- **Multi-Non-Rare Orphans:** orphan drugs with multiple indications where some are non-rare diseases
- **Non-Orphan Drugs:** drugs treating only non-rare diseases

DRUG CATEGORIZATION

Out of **8,842** drugs analyzed:



RESULTS AND INTERPRETATION

2022 Medicare Spending

In 2022, the total spend associated with Medicare Part B (FFS) and D was \$481.65 billion and the drug spend was \$286.69 billion (Table 1).

Table 1. Medical and Drug Spending for Medicare Parts B and D

Category	2022 Spending (\$ Billions)
Total Part B and D Drug	\$ 286.69
Total Part B and D Medical and Drug	\$ 481.65

Multi-Non-Rare Orphans drove Orphan drug spend. (Figure 1)

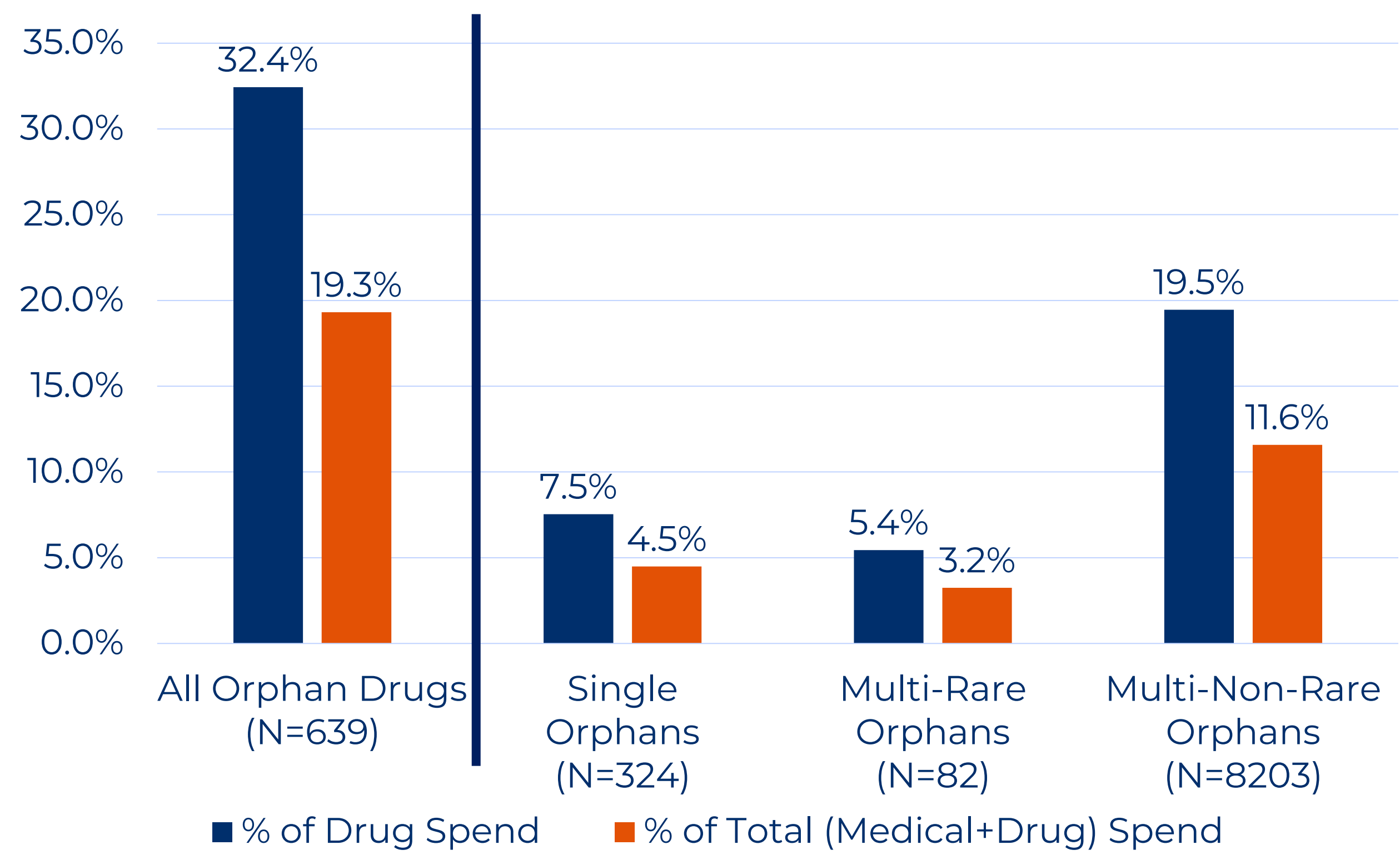
- Multi-Rare Orphans constituted a small proportion of the Medicare Parts B and D drug spend and an even smaller proportion of the total medical + drug spend (5.4% and 3.2%, respectively).
- By contrast, spending on Multi-Non-Rare Orphans was more than triple that of Multi-Orphans in both drug spend and total spend (19.5% and 11.6%, respectively).
- Single Orphans demonstrated similar trends as Multi-Rare Orphans.

Time Trend Analysis

- Trends of proportionate spend from 2018-2022 reveals stable trends over time, with Orphan Drugs representing 31-32% of Medicare drug spend per year (Figure 2).

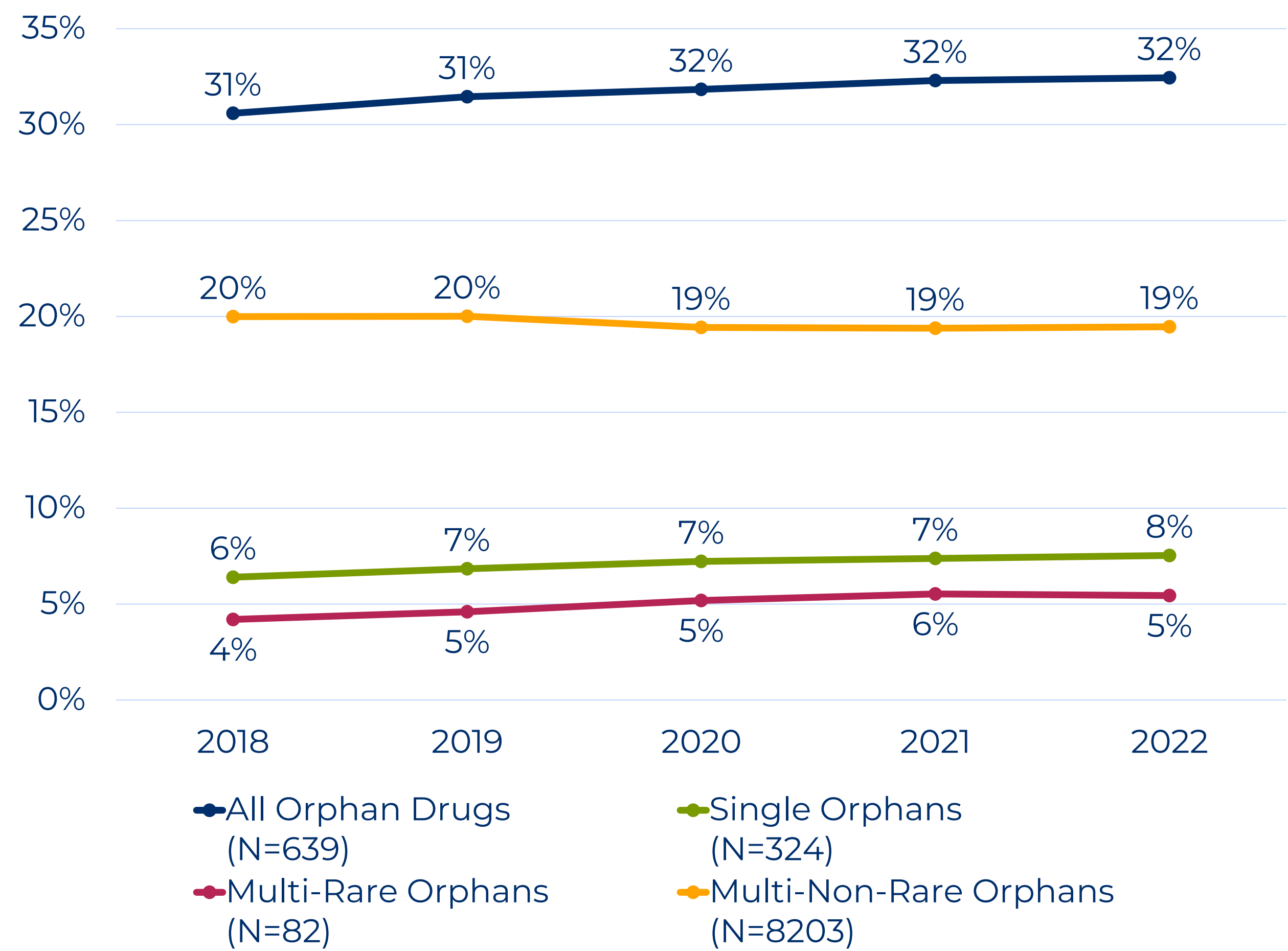
The proportion of Medicare drug spending on Orphan Drugs was stable over time. (Figure 2)

Figure 1. Proportion of Total Medicare Part B and D Drug Spend in 2022 Attributable to Orphan Drugs



Spending on Orphan Drugs was concentrated primarily in Multi-Non-Rare Orphans and not in Multi-Rare Orphans.

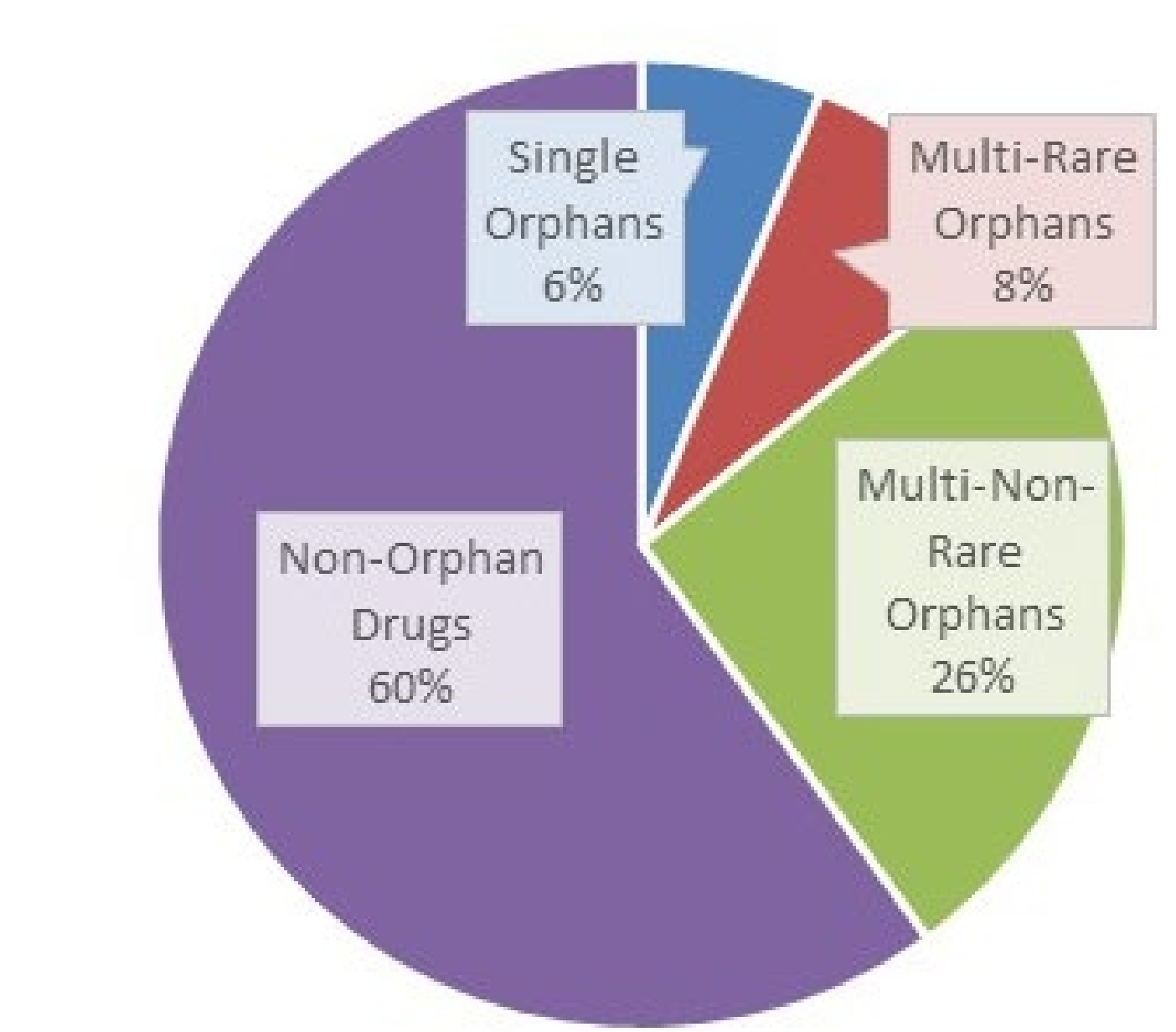
Figure 2. Time Trend - Proportion of Total Medicare Part B and D Drug Spend Attributable to Orphan Drugs



Top 50 Drugs with Highest 2022 Medicare Drug Spend

- Most (60%) of the top 50 Medicare drugs were non-orphan drugs, followed by Multi-Non-Rare Orphans (26%) (Figure 3).

Figure 3. Categorization of Top 50 Drugs with the Highest 2022 Medicare Drug Spend



Limitations

- This study included data available on Medicare Parts B and D but does not include Parts A and C.
- At the time this study was conducted, the most recent data cut available was 2022; more recent data is now available.

Disclosures

G Lyons, A Messali, and L Feng are employees of Alexion, AstraZeneca Rare Disease, and own stock in the company. T Olufade and J Eberle are employees of AstraZeneca and own stock in the company. Y Min and L Tran are employees of Health Management Associates and received funding to perform this study.

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