Racial and Ethnic Disparities in Orphan Drug Utilization Among Medicare Beneficiaries

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Poster HPR99

INTRODUCTION

Although racial and ethnic disparities in healthcare access and medication use are well-documented, little is known about their impact on the utilization of orphan drugs among Medicare beneficiaries. This study evaluates the racial/ethnicity disparities in the utilization of orphan drugs defined as those used exclusively for orphan disease indications and excluding drugs with both orphan and non-orphan indications, among Medicare Part B and D beneficiaries.

METHODS

DATA SOURCES:

We used 2022 100% Medicare Part B Fee-For-Service (FFS) claims and 100% Medicare Part D claims data. We also used FDA Orphan Drug Designation Database to identify orphan drugs.

OUTCOME:

Use of orphan drugs that are approved exclusively for orphan disease indications

STATISTICAL ANALYSIS:

Multivariable logistic regression models were used to assess the association between race/ethnicity and the use of any orphan drugs, adjusting for age, gender, dual eligibility, and comorbidities. We also conducted separate models stratified by urban and rural residence. All statistical analyses were performed using SAS 9.4.

DISCUSSION & CONCLUSION

This study identifies significant racial disparities in orphan drug utilization among Medicare Part B and D beneficiaries, with Black beneficiaries slightly less likely and Hispanic and Asian beneficiaries more likely to use orphan drugs compared to White beneficiaries. These differences may reflect variation in disease prevalence, provider prescribing patterns, or access to specialty care.

Rural residence further intensified these disparities, with minority groups, particularly Asian and Hispanic beneficiaries, showing higher odds of orphan drug use compared to rural Non-Hispanic Whites. These patterns may be influenced by geographic variation in care delivery or how rare diseases are diagnosed and managed in rural settings. Future research should explore the underlying drivers of both racial and geographic disparities and their implications for equitable access to high-cost therapies.

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RESULTS

Table 1. Sample Characteristics

Characteristics	Medicare Beneficiaries Using any Part B/D drugs	Beneficiaries Using Orphan Drugs	Urban-Beneficiaries using Orphan Drugs	Rural-Beneficiaries using Orphan Drugs
Total Beneficiaries	53.6M	1.5M	1.3M	0.2M
Mean Age	72.5	70.2	70.3	69.5
<65	11.8%	17.6%	16.9%	20.9%
65-74	47.3%	49.1%	49.5%	47.5%
75-84	29.7%	27.9%	28.2%	26.5%
85+	11.2%	5.4%	5.4%	5.2%
Male	43.0%	45.2%	45.2%	45.1%
Non-Hispanic White	78.6%	77.0%	75.1%	87.9%
Non-Hispanic Black	10.5%	11.8%	12.6%	7.6%
Hispanic	3.3%	3.6%	4.0%	0.9%
Non-Hispanic Asian	2.7%	2.6%	3.0%	0.3%
Non-Hispanic Other Race or Multiple Race	4.8%	5.0%	5.3%	3.2%
Rural	17.3%	15.0%	0%	100%
Dual	22.8%	27.6%	27.4%	28.9%
Low Income Subsidy	25.2%	30.3%	30.0%	32.5%
With at least one comorbidity	12.8%	17.2%	17.1%	18.2%
Cancer-Breast	2.5%	4.1%	4.1%	4.1%
Cancer-Colorectal	0.8%	1.7%	1.6%	2.1%
Cancer-Endometrial	0.3%	0.4%	0.4%	0.4%
Cancer-Lung	0.7%	1.9%	1.9%	2.0%
Cancer-Prostate	2.4%	3.2%	3.2%	3.2%
Cancer-Urologic	0.5%	0.8%	0.8%	0.8%
Alzheimer's disease	1.3%	0.7%	0.7%	0.7%
Non-Alzheimer's Dementia	4.0%	3.1%	3.0%	3.3%
Parkinson's Disease and Secondary Parkinsonism	1.0%	1.2%	1.1%	1.2%
Stroke/Transient Ischemic Attack	3.4%	4.9%	4.9%	5.1%

Figure 1.
Orphan Drug Use by Race/Ethnicity and Urban/Rural

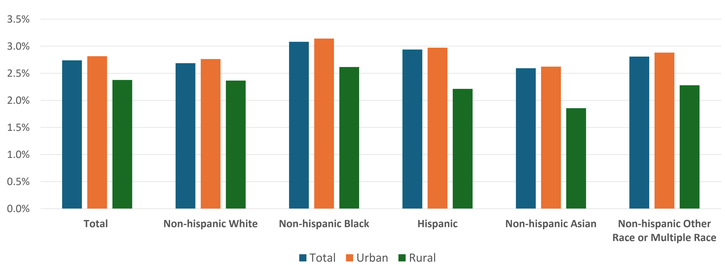

Table 2. Adjusted Odds Ratios for Orphan Drug Use

Variable	Adjusted OR	95% CI
<65	0.89	0.83-0.94**
75-84	1.13	1.13-1.14**
85+	2.26	2.24-2.28**
Male	0.93	0.93-0.93**
Non-Hispanic Black	0.99	0.99-1.00*
Hispanic	1.06	1.05-1.07**
Non-Hispanic Asian	1.09	1.08- 1.10**
Non-Hispanic Other Race or Multiple Race	1.02	1.01-1.03**
Rural	1.23	1.22-1.24**
Dual	0.98	0.97-0.99**
Low Income Subsidy	0.86	0.85-0.87**
Cancer-Breast	0.55	0.55-0.56**
Cancer-Colorectal	0.48	0.47-0.49**
Cancer-Endometrial	0.81	0.79-0.83**
Cancer-Lung	0.40	0.40-0.41**
Cancer-Prostate	0.71	0.71-0.72**
Cancer-Urologic	0.67	0.66-0.69**
Alzheimer's disease	1.42	1.39-1.45**
Non-Alzheimer's Dementia	1.15	1.14-1.16**
Parkinson's Disease and Secondary Parkinsonism	0.77	0.76-0.78**
Stroke/Transient Ischemic Attack	0.64	0.64-0.65**

*p<0.01 **p<0.0001

Table 3.
Adjusted Odds Ratios for Orphan Drug Use Stratified by Urban/Rural

Race/Ethnicity	Urban		Rural	
	Adjusted OR	95% CI	Adjusted OR	95% CI
Non-Hispanic Black	0.99	0.98-0.99**	1.02	1.00-1.03*
Hispanic	1.05	1.04-1.06**	1.24	1.18-1.29**
Non-Hispanic Asian	1.08	1.07-1.09**	1.33	1.24-1.43**
Non-Hispanic Other Race or Multiple Race	1.01	1.00-1.02*	1.11	1.09-1.14**

*p<0.01 **p<0.0001

- Among Medicare Part B and D beneficiaries who used at least one orphan drug, 77.0% were White, 11.8% Black, 3.6% Hispanic, 2.6% Asian, and 5.0% Other Race/Multiple Race.
- **Figure 1** displays the percentage of Medicare Part B and D beneficiaries using orphan drugs by race/ethnicity, comparing overall, urban, and rural populations. Non-Hispanic Black beneficiaries had the highest overall orphan drug use, primarily driven by higher usage in urban areas.
- **Table 2** presents results from multivariable logistic regression, showing significant associations between race/ethnicity and orphan drug use:
- Black beneficiaries had 1% lower odds of utilizing orphan drugs (aOR: 0.99, 95% CI: 0.99–1.00)
- Hispanic beneficiaries had 6% higher odds (aOR: 1.06, 95% CI: 1.05–1.07)
- Asian beneficiaries had 9% higher odds (aOR: 1.09, 95% CI:1.08–1.10)
- **Table 3** shows that in rural areas, all racial and ethnic minority groups had higher odds of orphan drug use compared to Non-Hispanic White beneficiaries. The highest odds were among Asian (33% higher), followed by Hispanic (24%), Other Race/Multiple Race (11%), and Black (2%) beneficiaries.

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