Orphan Drug Pricing Comparisons in Low-, Middle- and High-Income Countries

EE274

Scan QR code to access the poster

Charles Brown[1]; Dilip Choubey[1]; Burcu Kazazoglu Taylor[1]

[1]Novartis Gene Therapies Inc., Bannockburn, IL, USA

Introduction

- Of the 350 million people living with rare diseases worldwide, 50% are children and 30% are likely to die by 5 years of age[1,2]
- While pharmaceutical companies have increased research and development of orphan drugs for rare diseases, these efforts do not yet address availability and access challenges[3–6]
- Disparities observed in the scope and establishment of orphan drug policies by geographic location and income highlight ongoing global inequity for orphan drug access[5]
- Availability and access to orphan drug treatments is important to reduce morbidity and mortality of rare diseases and represents an unmet need worldwide[7]
- Challenges exist regarding orphan drug pricing to ensure availability and access in middle- and low-income countries with limited resources[2,3,8]
- To provide a better understanding of barriers to rare disease treatment worldwide, we sought to assess orphan drug availability and access patterns in LICs, MICs, and HICs

Objective

- We conducted a retrospective availability and pricing review of orphan drugs to observe country access and current published prices, segmented between HICs, MIC, and LICs

Methods

- Rare disease treatments were identified using the FDA's Orphan Drug Designations and Approvals list[9]
- Any drug developed under the US Orphan Drug Act is defined as treatment for a rare (orphan) disease that affects fewer than 200,000 people in the United States or is of low prevalence (<5 per 10,000 in the community)[10]
- Countries were classified as HICs, MICs, or LICs according to the World Bank's income classification criteria for the 2023 fiscal year, calculated using the World Bank Atlas method (**Table 1**)[11]

Table 1. Gross national income per capita range by income classification[11]

Income classification	GNI per capita range
HIC	$13,520 to $90,360
MIC	$1,086 to $13,205
LIC	$500 to $1,085

GNI, gross national income; HIC, high-income country; LIC, low-income country; MIC, middle-income country.

- Rare disease treatments that were approved between January 1, 2012, and January 1, 2022, and were available in more than 20 countries, including more than two MICs, were included in the pricing analysis
- Pricing data were obtained using NAVLIN by EVERSANA™ (a web-based pricing subscription service)[12]
- Prices were analyzed by country income classification (HIC, MIC, LIC) to identify potential pricing patterns between income classes
- Scatter plot and *t*-test were used to determine statistically significant differences in prices between income classes (originally planned as a chi-square analysis)

Results

- Twelve rare disease treatments with various orphan disease designations met the analysis criteria (**Table 2**)

Table 2. Rare disease treatments meeting analysis criteria

Rare disease treatment	Orphan disease designation
Agalsidase beta	Fabry disease
Alglucosidase alfa	Glycogen storage disease type II
Bosentan	Pulmonary arterial hypertension
Eculizumab	Neuromyelitis optica, myasthenia gravis, atypical hemolytic uremic syndrome, paroxysmal nocturnal hemoglobinuria
Eliglustat	Gaucher disease type 1
Idursulfase	Hunter syndrome (mucopolysaccharidosis type II)
Imiglucerase	Gaucher disease types 1, 2, and 3
Ivacaftor	Cystic fibrosis
Nusinersen	Spinal muscular atrophy
Onasemnogene abeparvovec	Spinal muscular atrophy
Ravulizumab	Paroxysmal nocturnal hemoglobinuria, myasthenia gravis
Risdiplam	Spinal muscular atrophy

- A total of 60 countries worldwide had at least one of the 12 orphan drugs meeting study criteria available. Most of these countries were HICs (HICs, n=43 [72%]; MICs, n=17 [28%]; LICs, n=0 [0%]) (**Table 3**).

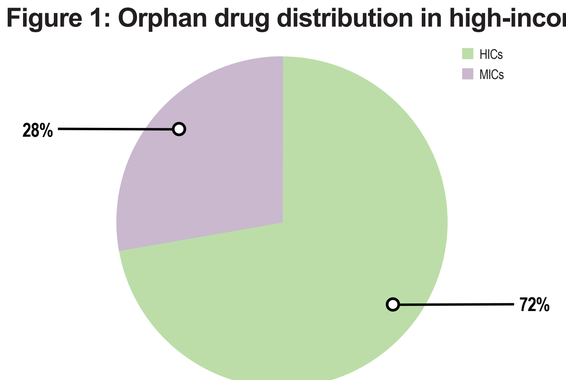
Table 3. Countries meeting orphan drug availability criteria stratified by income group

HICs	Argentina, Australia, Austria, Bahrain, Belgium, Canada, Chile, Croatia, Cyprus, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Israel, Italy, Japan, Latvia, Liechtenstein, Lithuania, Luxembourg, Netherlands, New Zealand, Norway, Oman, Poland, Qatar, Saudi Arabia, Slovakia, Slovenia, South Korea, Spain, Sweden, Switzerland, Taiwan, United Arab Emirates, United Kingdom, United States
MICs	Bosnia & Herzegovina, Brazil, Bulgaria, Colombia, Jordan, Lebanon, Montenegro, Morocco, North Macedonia, Peru, Romania, Russia, South Africa, Thailand, Turkey, Ukraine, Vietnam
LICs	None

HIC, high-income country; LIC, low-income country; MIC, middle-income country.

- None of the orphan drugs that met analysis criteria were available in LICs (**Figure 1**)

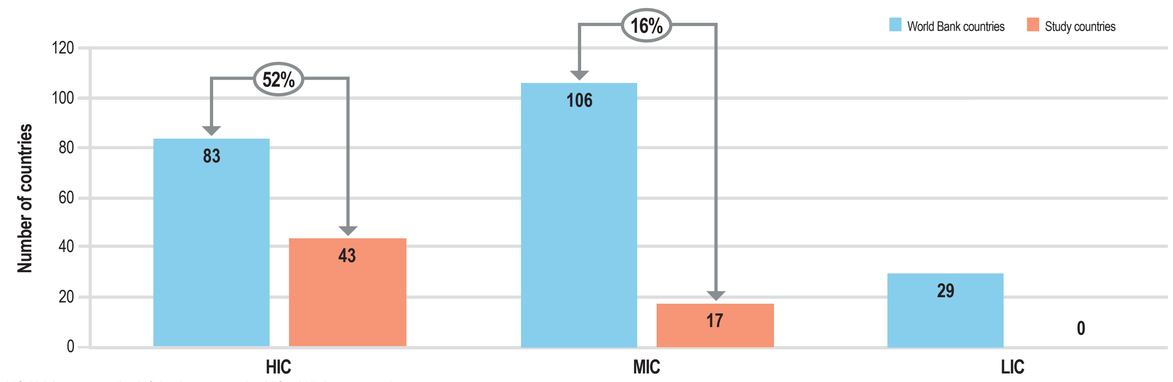
Figure 1: Orphan drug distribution in high-income, middle-income, and low-income countries



HIC, high-income country; MIC, middle-income country.

- The World Bank classifies a total of 218 countries (83 HIC, 106 MIC, and 29 LIC), and 60 countries in the current study met entry criteria (43 HIC, 17 MIC, and 0 LIC). This demonstrates that rare disease treatments have greater representation in HICs and are underrepresented in MICs and LICs (**Figure 2**).[11]

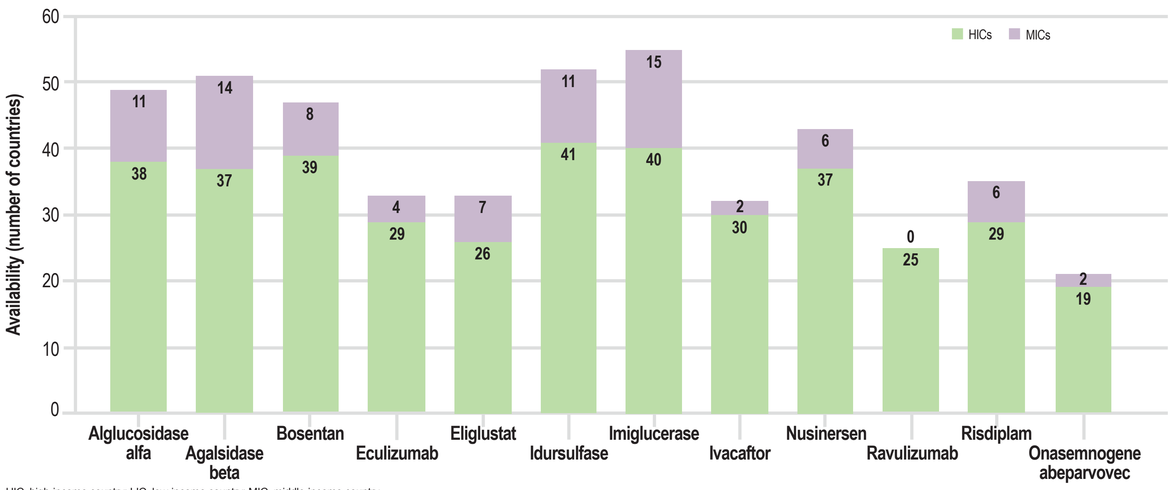
Figure 2. Study countries represented by income compared with World Bank classification



HIC, high-income countries; LIC, low-income countries; MIC, middle income countries.

- Orphan drugs are available in HICs, have a very limited presence in MICs, and have no presence in LICs (**Figure 3**)

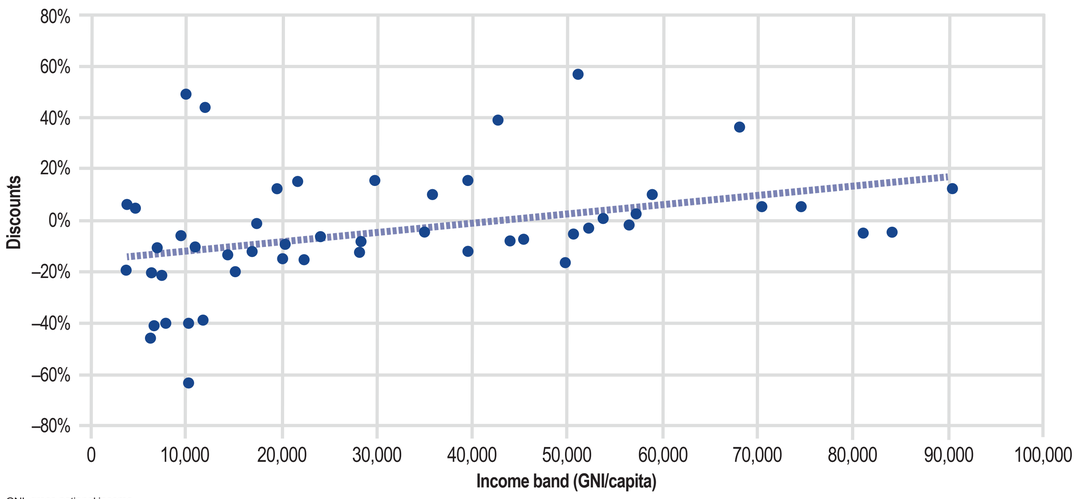
Figure 3. Orphan drug availability: number of countries per income classification



HIC, high-income country; LIC, low-income country; MIC, middle-income country.

- Although there was a positive correlation (correlation coefficient: 0.368346), no statistically significant differences in prices for orphan drugs between MICs and HICs were identified (**Figure 4**)

Figure 4. Percentage discount compared with high-income and middle-income countries



GNI, gross national income.

Conclusions

- There is a gap in terms of orphan drug access and availability for HICs compared with MICs and LICs
- Orphan drugs have a very limited presence in MICs and no presence in LICs
- When rare disease treatments are available in MICs, their prices are similar to the prices for HICs
- Although orphan drug policies and the pharmaceutical industry have emphasized drug development for rare diseases, the benefits of these advancements have not yet reached many patients
- While the pharmaceutical industry has put more focus on drug development for rare diseases,[13] the benefits of such innovation are not yet reaching the large share of patient populations in MICs and LICs. As an industry, availability and access to orphan drugs is the next challenge in rare diseases: designing innovative approaches in partnership with health care ecosystem and stakeholders that will enable broader access to orphan drugs in LICs and MICs.

References

1. The Lancet Diabetes Endocrinology. Spotlight on rare diseases. *Lancet Diabetes Endocrinol*. 2019;7:75.
2. NORD. Updated rare disease facts and figures. 2017. Available at: https://rareundiagnosed.org/rare-disease-facts. Accessed September 30, 2022.
3. Weerasooriya SU. *Health Info Libr J*. 2019;36:179–84.
4. Roberts AD, et al. Orphan drug approval laws. 2022. Available at: https://www.ncbi.nlm.nih.gov/books/NBK572052/. Accessed September 30, 2022.
5. Chan AY, et al. *Value Health*. 2020;23:1580–91.
6. Sharma A, et al. *J Pharm Bioallied Sci*. 2010;2:290–9.
7. Gammie T, et al. *PLoS One*. 2015;10:e0140002.
8. Kheirandish M. *East Mediterr Health J*. 2020;26:372–3.
9. US Food and Drug Administration, US Department of Health and Human Services. Search orphan drug designations and approvals. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/. Accessed September 30, 2022.
10. Health Promotion and Disease Prevention Amendments of 1984, § 4, Pub. L. 98–551, 98 Stat 2815 (1984).
11. World Bank Country and Lending Groups: country classifications. https://datahelpdesk.worldbank.org/knowledgebase/articles/906519-world-bank-country-and-lending-groups. Accessed September 30, 2022.
12. NAVLIN by EVERSANA. https://data.navlin.com/alspc/#!/. Accessed September 30, 2022.
13. PhRMA. Medicines in Development: Rare Diseases, A Report on Orphan Medicines in the Pipeline. 2021. https://phrma.org/resource-center/Topics/Medicines-in-Development/Medicines-in-Development-for-Rare-Diseases-2021-Report. Accessed September 30, 2022.

Abbreviations

FDA, US Food and Drug Administration; GNI, gross national income; HIC, high-income country; LIC, low-income country.

Acknowledgments and Disclosures

This study was funded by Novartis Gene Therapies, Inc.

Editorial support was provided by Wynne Dillon, MS, of Kay Square Scientific, Newtown Square, PA. This support was funded by Novartis Gene Therapies, Inc.

Disclosures: CB, **DC**, and **BKT** are employees of Novartis Gene Therapies, Inc., and own Novartis stock or other equities.

Presented at ISPOR Europe 2022, November 6–9, 2022, Vienna, Austria.