Is innovation reaching everyone?

An assessment of healthcare equity in access to orphan drugs that represent a public health concern and therapeutic innovation in LATAM

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Background

The accelerated approval of orphan drugs is based on public health concern and therapeutic innovation. While these drugs should be equitably accessible to everyone, people in Latin America (LATAM) often face higher access struggles than European patients. This study aimed to compare the time from marketing authorization until health technology assessment (HTA) decision and coverage restrictions for orphan drugs in LATAM compared to Europe.

Methods

We evaluated orphan drugs with accelerated assessment approval by the European Medicines Agency (EMA) and Medicines and Healthcare Products Regulatory Agency (MHRA) in the last five years. The marketing authorization dates and HTA decisions and dates were collected for Germany (EMA, G-BA), Sweden (EMA, TLV), UK (MHRA, NICE), Brazil (ANVISA, CONITEC), and Chile (ISP, Ricarte Soto Law). The median time from marketing authorization to HTA decision was calculated.

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Results

Between 2017 and 2022, nine orphan drugs received accelerated approval by the EMA. Of these, only four and six received marketing authorization in Chile and Brazil, respectively. In total, 24 HTAs were identified for eight of these orphan drugs (Europe: 19, LATAM: 5), with a total of 20 positive HTA decisions (**Table 1**). Differences were observed in coverage scope, with higher restrictions in LATAM (e.g., coverage restriction by disease categorization).

Table 1: HTA outcomes for the select orphan drugs (2017–2022)

Country	Product	Indication	Recommendation	Registration Date	Time (months to reimbursement)
Germany	Cenegermin	Neurotrophic keratitis	RECOMMENDED	July 2017	46
Germany	Cerliponase alfa	Neuronal ceroid lipofuscinosis type 2	RECOMMENDED	May 2017	7
Brazil	Cerliponase alfa	Neuronal ceroid lipofuscinosis type 2	RECOMMENDED	July 2018	45
Germany	Nusinersen sodium	Spinal Muscular Atrophy	RECOMMENDED	May 2017	7
Brazil	Nusinersen sodium	Spinal Muscular Atrophy type I	RECOMMENDED	August 2017	20
Brazil	Nusinersen sodium	Spinal Muscular Atrophy	RESTRICTED (TYPE II)		45
Germany	Risdiplam	Spinal Muscular Atrophy	NOT RECOMMENDED	March 2021	7
Sweden	Risdiplam	Spinal Muscular Atrophy	NOT RECOMMENDED	March 2021	7
Brazil	Risdiplam	Spinal Muscular Atrophy	RESTRICTED (TYPES I & II)	October 2020	17
UK	Budesonide	Eosinophilic esophagitis	NOT RECOMMENDED	January 2018	41
Sweden	Budesonide	Eosinophilic esophagitis	RECOMMENDED	January 2018	17
Sweden	Lanadelumab	Hereditary angioedema	NOT RECOMMENDED	November 2018	24
UK	Lanadelumab	Hereditary angioedema	RECOMMENDED	November 2018	11
Germany	Lanadelumab	Hereditary angioedema	NOT RECOMMENDED	November 2018	8
Brazil	Lanadelumab	Hereditary angioedema	NOT RECOMMENDED	October 2019	20
Germany	Inotersen sodium	hATTR with polyneuropathy	RECOMMENDED	July 2018	8
Sweden	Inotersen sodium	hATTR with polyneuropathy	RECOMMENDED	July 2018	16
UK	Patisiran sodium	hATTR with polyneuropathy	RECOMMENDED	August 2018	12
Germany	Patisiran sodium	hATTR with polyneuropathy	RECOMMENDED	August 2018	7
Sweden	Patisiran sodium	hATTR with polyneuropathy	RECOMMENDED	August 2018	14

Additionally, the median time from marketing authorization to HTA decision in Europe was 10.75 months, compared with 20.39 months in LATAM (**Figure 1**). This represents an equity gap score of 1.90 when comparing time to HTA decision between LATAM and Europe (**Figure 2**).

Figure 1: Median time to reimbursement (months)

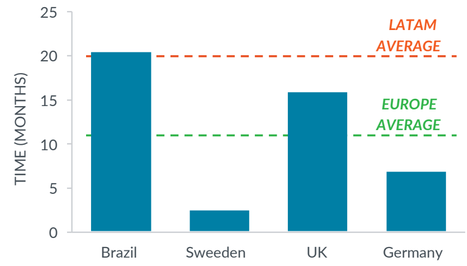

Figure 2: HTA equity gap

$$\frac{\text{Time to reimbursement Europe}}{\text{Time to reimbursement LATAM}} = 1.90$$

<1	1	>1
Inequity, lower in **LATAM**	Equal	Inequity, lower in **Europe**

Conclusions

There is a considerable equity gap in the HTA of orphan drugs that represents a public health concern and therapeutic innovation between Europe and LATAM. This likely follows delays in registration and launch (~average of 6 months), which relate to a lower profitability of the LATAM market driven by a lower ability-to-pay when compared to Europe, and to reduced local capabilities in LATAM when compared to Europe. Financing decision-makers should work closely with manufacturers to increase the attractiveness of the LATAM market without altering the financial stability of the region.

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