ACCEPTANCE CODE:

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Assessing Access to Innovative Orphan Medicines in Latin America: A Cross-Country Value and Access Analysis

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Objectives

To assess the availability and time to access of innovative orphan medicines across Latin America and identify system-level barriers a ecting equitable access for rare disease patients.

Methods

A total of 271 orphan medicines approved globally between 2014 and 2024 were analyzed across ten Latin American countries. Availability and time-to-access definitions were standardized to allow cross-country comparison. Extended availability was defined as the combined presence of full, limited, and private access. Data were collected through national pharmaceutical associations, research-based companies, and publicly available sources. Descriptive analyses were conducted.

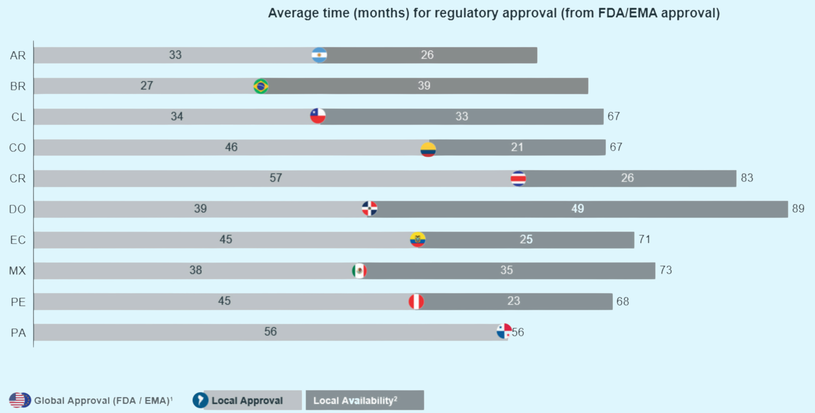
Results

Average extended availability of orphan medicines was 16% of those globally approved. Argentina showed the highest availability (29%), while Ecuador Dominican Republic presented the lowest (6%). Time to availability ranged from 21 to 49 months. Benchmarking against European datasets highlights substantially higher availability levels outside the region.

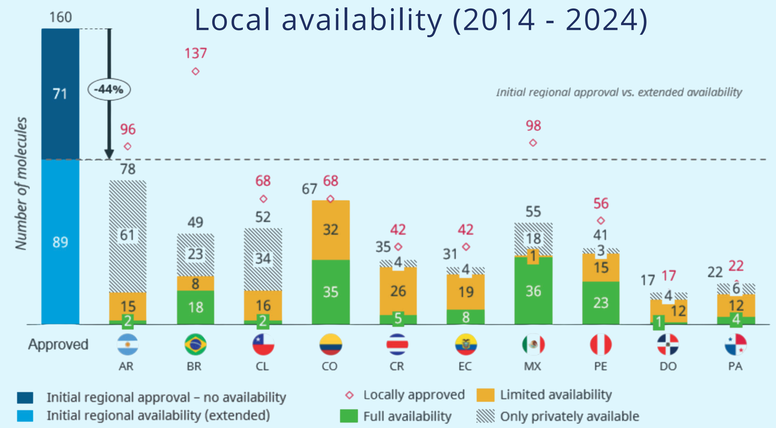
Conclusions

Access to orphan medicines remains extremely limited across Latin America, reflecting structural constraints in regulation, financing, and HTA processes. Strengthening rare-disease-specific policies and coordinated multi-stakeholder action is essential to improve value-based decision-making and patient outcomes.

Time to availability (2014 - 2024)



Local availability (2014 - 2024)



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