The Landscape of Approved drugs with Pediatric Orphan indications in Saudi Arabia

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INTRODUCTION

Orphan drug development is vital for children affected by rare diseases. In Saudi Arabia, the high prevalence of rare diseases, due to frequent pathogenic alleles, presents a significant challenge despite government efforts. Additionally, there is limited data on recently approved pediatric orphan drugs, particularly regarding their availability and affordability.

OBJECTIVE

This study evaluates the availability, accessibility, and cost of orphan drugs for pediatric patients in Saudi Arabia (SA) compared to the United States (US). By examining approved drug types, and treatment costs, it aims to identify disparities and propose strategies to improve pediatric drug access and affordability in SA.

METHOD

- Data on all drugs approved with orphan designations and authorized for marketing with pediatric indications in **Saudi Arabia (KSA) and the United States (US)** from **2017 to 2023**, were derived from regulatory body databases This included data from the US Food and Drug Administration (FDA) and the Saudi Food and Drug Authority (SFDA).

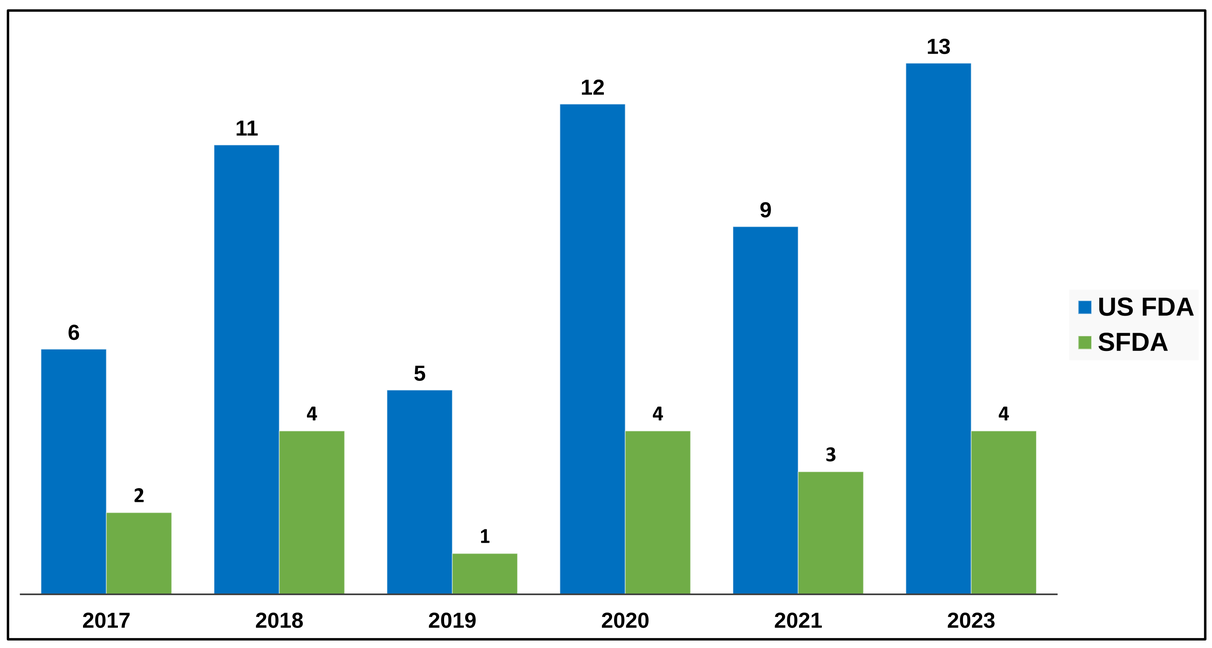
Pricing data was collected from the Red Book for wholesale acquisition cost (WAC), and the SFDA website for Saudi prices. All prices were converted from Saudi riyals to US dollars and adjusted to purchasing power parity dollars (PPP$) to enable direct comparison between regions.
Data was analyzed by summary descriptive statistics.

RESULTS

Table 1. Characteristics of Orphan Drug Approvals by the US Food and Drug Administration (FDA) and Saudi Food and Drug Authority (SFDA), 2017-2023

	USFDA Approvals (n, %)	SFDA Approvals (n, %)
Drug Type		
Biologics	20 (35.7%)	10 (55.6%)
Chemical entities	36 (64.3%)	8 (44.4%)
Therapeutic Area		
Cancer	13 (23.7%)	5 (27.8%)
Genetic	34 (60.7%)	11 (61.1%)
Infections	2 (3.6%)	0 (0.0%)
Transplant	1 (1.8%)	1 (5.6%)
Other	6 (10.7%)	1 (5.6%)
Patient Population Indication		
Adults and Pediatric	32 (57.1%)	14 (77.8%)
Pediatric	24 (42.9%)	4 (22.2%)
Total	56	18

Chart 1 . Comparison of Orphan Drug Approvals with Pediatric Indications: US FDA vs. SFDA



During the study period, the SFDA approved **18** drugs with **22** pediatric orphan indications, representing only **32%** of the pediatric orphan drug approvals granted in the US. **61%** of the approved orphan pediatric indications in SA were approved for genetic diseases. The average annual treatment cost for orphan drugs is substantially higher in KSA than in the US, with an average cost of $1,472,659.50 in KSA compared to $356,548.51 in the US.

This indicates a **4-fold difference in pricing**, primarily for certain high-cost biologics. Specific drugs show especially high treatment costs in KSA, such as Tagraxofusp-erzs ($945,007.91) and Avalglucosidase Alfa-ngpt ($1,094,460.03). These drugs are essential for treating rare conditions, but their high costs in KSA could limit access for patients and impose significant financial burdens on the healthcare system.

CONCLUSIONS

This analysis shows significant cost and access disparities for orphan drugs between KSA and US. Orphan drugs are notably more expensive in KSA, especially high-cost biologics, which may limit patient access to essential treatments. While KSA has increased approvals, the range of available drugs still lags behind the US, highlighting a need for policies to regulate pricing, improve insurance coverage, and foster local production.