RARE DISEASES STATUS & ORPHAN DRUG REIMBURSEMENT IN THE MENA REGION: ALARMING PRIORITY, HTA EVOLUTION, AND CALL FOR ACTION.

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ISPOR Saudi Arabia Chapter

Background

- A rare disease (RD) is a health condition with a low prevalence.(1) RDs affect approximately 6% of the worldwide population,(2) Globally, less than one-tenth of patients with RDs receive disease-specific treatment.(3) There is no unified, or universally accepted definition of RD.(4) Regionally, large family size, high maternal and paternal age, and high consanguinity rates in the range of 25-60% are contributing factors for higher levels of congenital and genetic disorders. (5)

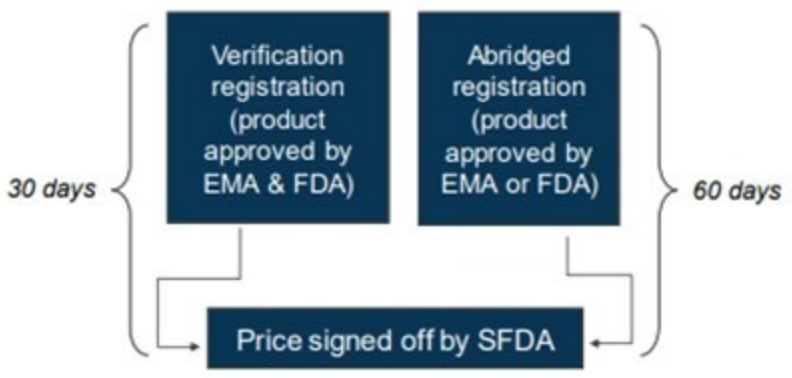
Objectives

- Defining the major hurdles in ODs reimbursement in MENA, focusing on equity, Healthcare system financing, HTA evolution within two major markets, Saudi Arabia & Egypt.

Methods and Materials

- An inter-subjective ethnographic study using hermeneutic dialogue was utilized during participant observation, in-depth interviews, focus groups, and literature review. An ecological framework was utilized to focus on the relationship between RDs financing and challenges in access & equity from cultural, geographical & economic perspectives.

Fig.1 *Saudi has an abridged regulatory and reimbursement pathways for ODs**



* FDA = US Food and Drug Administration, EMA = European Medicines Agency, SFDA = Saudi Food & Drug Authority

Results

- Egypt's healthcare system is largely financed by out-of-pocket (OOP) payments, which need to be reformed to achieve equitable financing. (6).

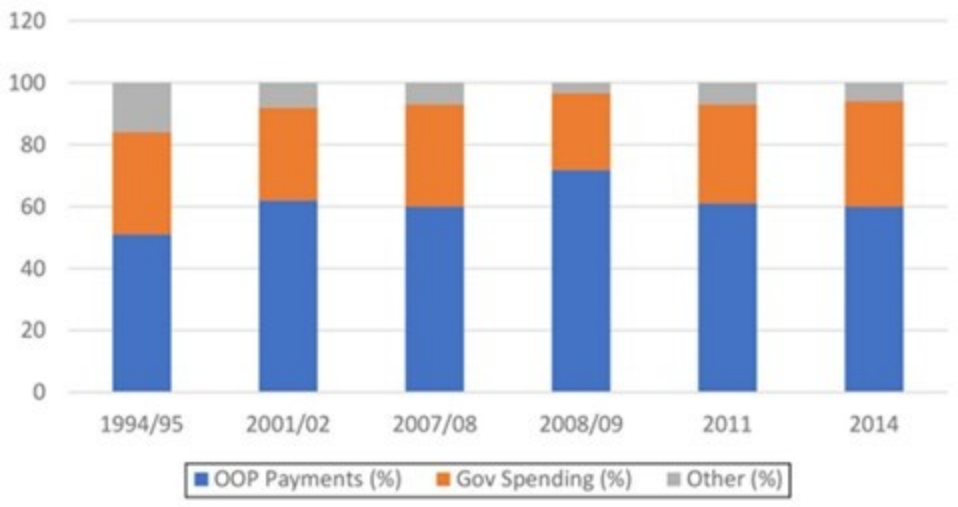


Fig.2 By authors using data from Egypt NHA (2011) and WHO and WB data bases

- In KSA, ODs public reimbursement is fragmented, and limited coverage within PHI. MOH/HTA center has been launched & ODD is under process to be implemented in 2023-end. Generally, The MENA region is facing healthcare equity challenges, part of it is access/regulatory, in addition to different cultural hurdles.
- Other barriers to access for rare disease patients: In the KSA to citizen's views on using modern medicine for genetic diseases was a notable barrier. For example, a study conducted in 2019 on a sample of people without epilepsy found that despite relatively high levels of education (42% with college or higher nonmedical), 55% agreed that patients with genetic, non-refractory epilepsies should be treated by a religious healer. (7)

Fig.3 Key Insights on Market Access Environment for Orphan Drugs in the MENA region*

MENA PMA ENVIRONMENT	KEY INSIGHTS
Registration	**Time to market are typically long**; most countries might offer Orphan Drug Developers a fast track approval though this is contingent on product profile and level of unmet needs; FDA and/or EMA approval is typically required
	KSA, Egypt and Algeria have the longest time to market though governments are trying to shorten timelines; UAE and Lebanon are among the fastest to market
	Early access programs are possible in all major markets, however no legal guidelines exist except for Algeria and Morocco. Drugs are ordered on patient name basis, which are generally paid OOP; though for KSA & UAE there is reimbursement provision for nationals
Need of clinical evidence	**Though not required, local clinical trials** are becoming increasingly important to show clinical evidence locally; it is unlikely this will become mandatory in the future
Pricing process	**Reference pricing is currently adopted** with **different level of maturity across MENA**. It is likely that reference pricing will evolve (i.e. expansion/change of reference basket) and **HTA assessment** might be considered in KSA, UAE & Egypt even for orphan drugs
Reimbursement status	**Reimbursement is granted** though **affordability will remain a challenge in several self-pay markets such as Lebanon, Morocco and Egypt**. Patient access programs help to ease the burden, especially as they are becoming more common
Key MENA trends	Trends towards **cost-containment measures, expansion of coverage, health technology assessment and increased importance of local manufacturing** were identified; to date, impact on orphan drug markets is likely to be minimal considering low budget impact

*Access environment may change based on fresh guidelines and updates to government policies
Source-Expert Interviews, Published Literature

Conclusions

- Lack of National policy is the fundamental challenge despite plenty of funding. Discussions should be more inclusive as NGOs; patient voice & caregivers' roles should be involved in decision-making. (8) EAPs programs are possible in the most of major MENA markets; however, no clear guidelines exist except for Algeria and Morocco. (9) Consequently, the historical underrepresentation in clinical trials has positioned MENA to provide a significant untapped and often treatment naïve patient population.

Contact

For more details:

Reference

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