

Driving patient access for treatments targeting rare genetic disorders in the Middle East



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Background & Objectives

In recent decades certain countries in the middle-east have seen substantial economic growth resulting in higher expectations being placed on their healthcare systems. With an increasing economic power, these countries are developing more modern and robust healthcare systems¹. This is demonstrated by the WHO's 2019 healthcare efficiency index where Saudi Arabia and the UAE were ranked 26th and 27th out of 191 countries, placing them ahead of Canada (30), Denmark (34), and the USA (37)².

Some of the countries in this region have a relatively higher prevalence of patients with inherited rare diseases because of practices such as marriage amongst cousins, and stigma towards prenatal screening and abortion³. For example, up to two thirds of marriages in some countries in this region are between cousins resulting in higher prevalence of genetic rare diseases such as sickle cell anaemia, Down's syndrome and many others³.

The objective of this study was to conduct a comparative analysis of the patient access opportunity for rare disease therapies in the region. To identify bright spots for Orphan Drug (OD) access and commercialisation opportunities, we compared healthcare coverage, the route to market and any associated incentives, pricing policies and any other barriers to access of rare disease therapies across three countries in this region. These were:

- The United Arab Emirates (UAE) which has a high per capita income and has the best healthcare infrastructure in the region^{4,5,1}
- The Kingdom of Saudi Arabia (KSA), which is an oil rich nation with the largest GDP in the region and almost 10 million people aged under 18 years old⁶, and
- Turkey although, not an oil-rich country and ranked 70th in the WHO's index, has the largest population in the region (80m) with a high proportion of people under 18 yrs old⁷

Methodology

Using secondary research supplemented by interviews with payers, clinical experts, and regional affiliates of biopharma companies, a comparative analysis to identify the opportunities and challenges of launching orphan drugs for rare disorders. This included:

- A) Targeted literature review, and
- B) Telephonic interviews with payers & KOLs (N= 4 per market) as well as commercial executives (N= 3) working in Middle Eastern affiliates of biopharma companies

Healthcare coverage for rare disease

- In the UAE and KSA citizens have extensive and fully reimbursed coverage for ODs from public providers. Expats on the other hand require private insurance, which rarely covers congenital diseases or which can potentially have high prescription drug co-pays (fig.1)⁸
- In Turkey, all citizens and expats with tax contributions for two years are covered by national health insurance. However, high-cost ODs are unlikely to be included in the list of drugs approved for routine national reimbursement (fig.1)⁹. Patients can only access these therapies through named patient imports that are reimbursed by the social security system

Orphan drug route to market

- In the UAE, the Ministry of Health and Disease Prevention (MOHAP) has a fast track regulatory and reimbursement process for ODs which has been used to approve numerous drugs within globally competitive timeframes (fig.2). For example, the UAE has one of the fastest access times with a worldwide rank of 4th for Ocaliva, 1st for Tagrisso, and ranged from 1st to 4th in 20 other analogues¹⁰
- The KSA does not have a specific access route for ODs but does allow for faster access (30-60 days) with EMA and/or FDA approval (fig.3)¹¹
- In Turkey, the standard access route requires 21 months (median), high discounts, and punitive exchange rate calculations which use a fixed Euro-Lira exchange rate of 70% the previous year's average rate. This is exacerbated by the year on year depreciation of the Lira over the last 5 years
 - However, named patient access is a viable route for ODs not marketed in Turkey (fig.4). This import route allows manufacturers to achieve EU like prices, avoid damaging their price in markets referencing Turkey, and bypass the punitive exchange rates as payments are made in currency of the country of origin¹². A large number of OD manufacturers have used this route to drive patient access over the last few years. A 2019 study found that of the 43 ODs studied, over 50% were accessed through the named patient import route¹³

Pricing policies for orphan drugs

- The UAE and KSA put a heavy emphasis on the price in the country of origin, unless lower External Reference Prices (ERPs)¹⁴. The healthcare systems in these countries are well funded, and payer willingness to pay is often substantially higher than in EU markets and more in line with the US
- The Turkish pricing policies for routine national reimbursement are; ERP and mandatory discounts on innovative products. Further downward pressure on prices are applied by the punitive official exchange rate mechanism. For named patient imports the healthcare system pays the list price of the drug from the country of origin which typically is an EU market¹²

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 healer¹⁵
- The UAE has the most liberal attitudes towards modern western medicine and treatment of rare genetic diseases in the region. However, whilst citizens do not face any conspicuous barriers to accessing OD treatments, expats are often refused coverage for genetic diseases by their private insurers
- In Turkey, once a reimbursement decision is made there are no other major barriers to patient access. Even the named patient import route is an established pathway with KOL requests for ODs often approved within 15 working days. This leaves the main barriers for ODs at the HTA/pricing assessment level

Results

Fig.1 Coverage for rare disease treatments



Fig.2 UAE offers fast-track regulatory and reimbursement approval*

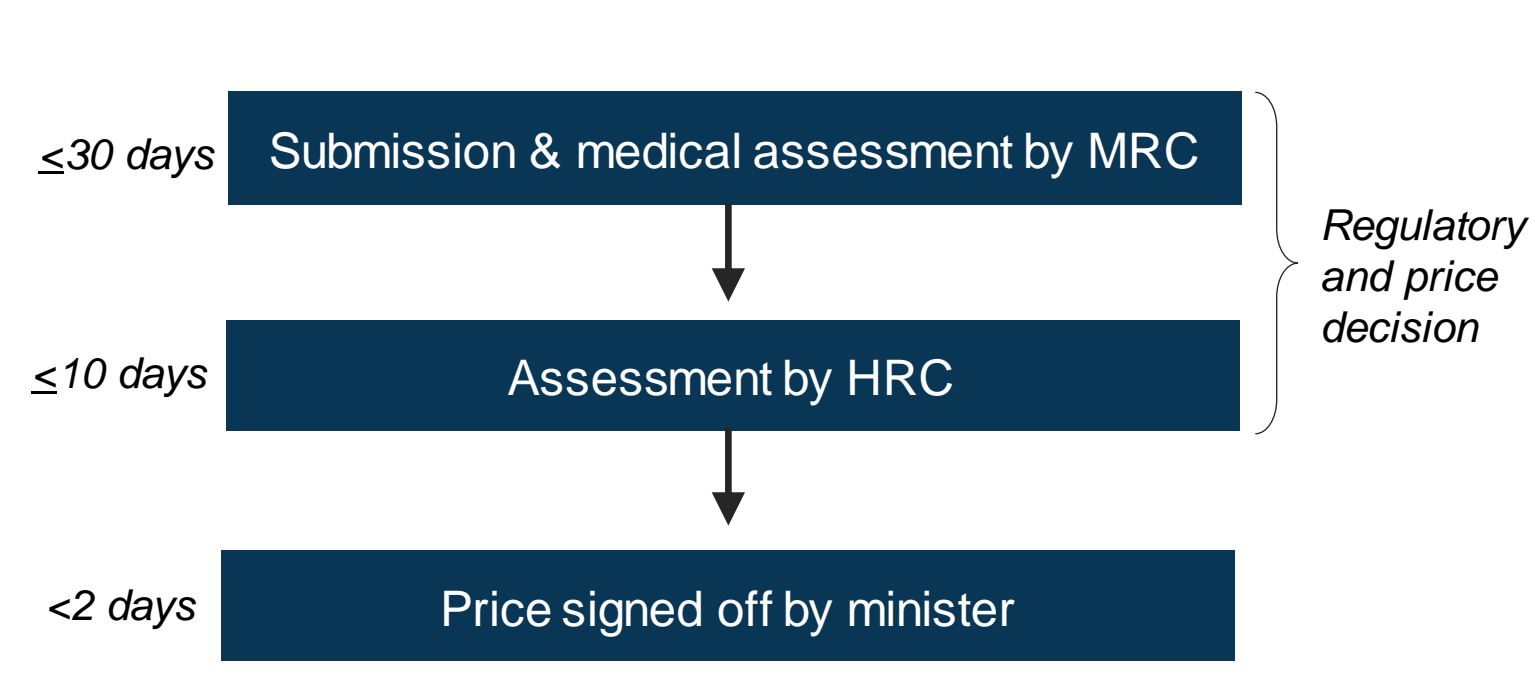


Fig.3 Saudi has an abridged regulatory and reimbursement pathways for ODs*

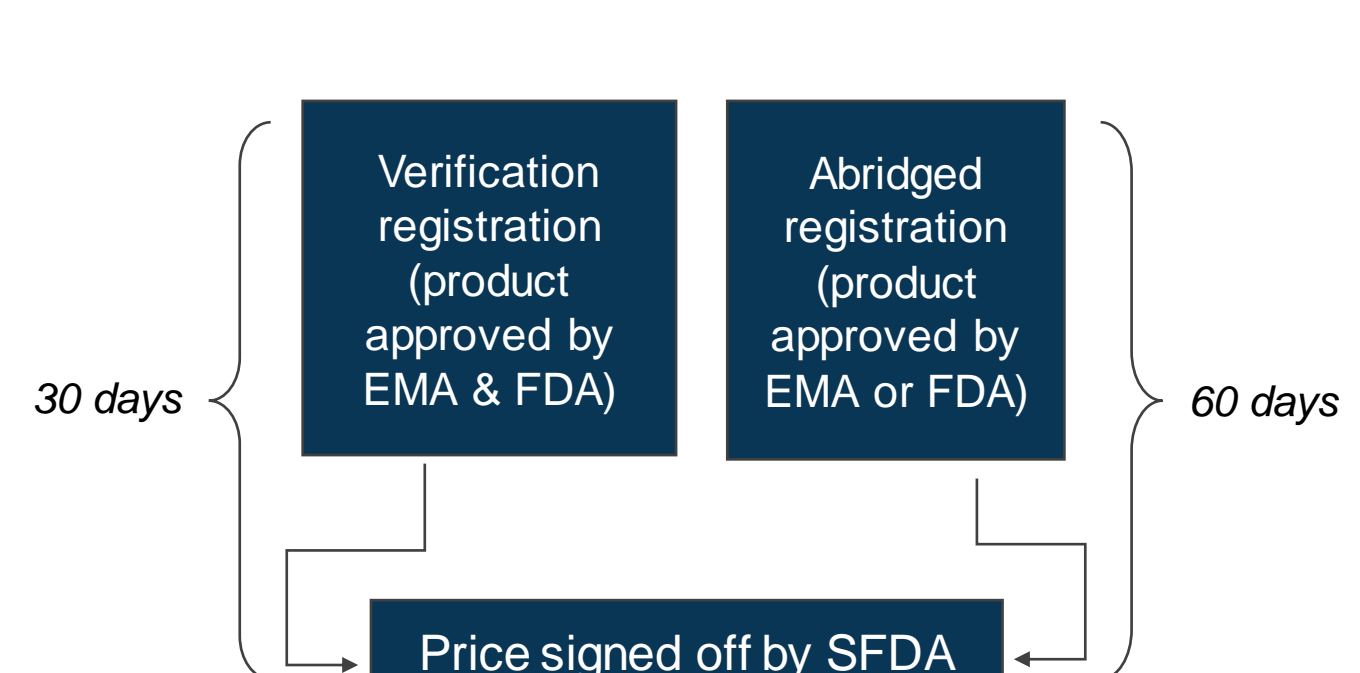
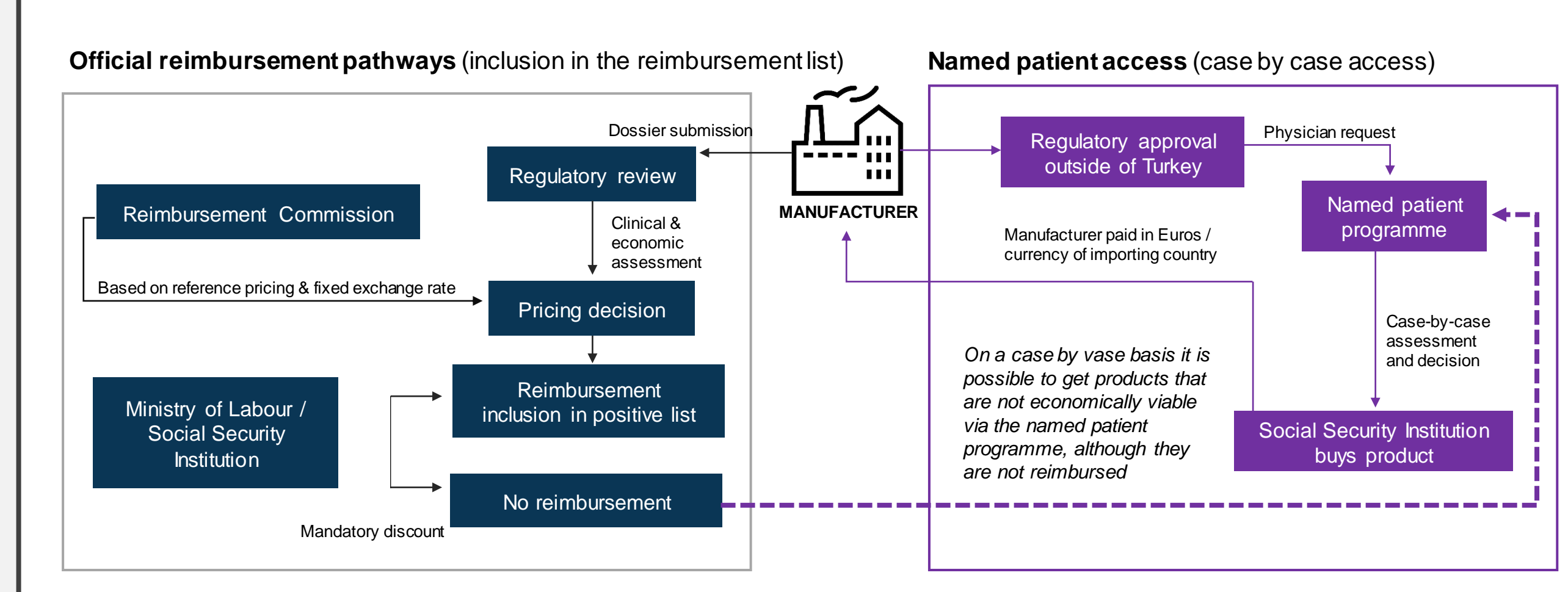


Fig.4 Turkey: Patients may get reimbursed access through named patient imports if the OD isn't on the official reimbursement list / not launched in the market



Conclusion

- In the oil rich countries such as the UAE and KSA, ODs can achieve speedy market entry and can command relatively high reimbursed prices. Moreover these markets tend to have a relatively higher prevalence for many genetic diseases. However, sociocultural barriers around diagnosing and treating genetic diseases can limit uptake
- In Turkey, to pursue the standard market access process is slow and results in punitive prices. However, the named patient access route looks promising but limits the volume potential for ODs
- Therefore, it is vitally important for manufacturers to have a well thought-out strategy when launching an OD in this region. For biopharma manufacturers to drive access for their ODs while optimising commercial outcomes requires careful selection of the route to market, a favourable launch sequence, and early stakeholder engagement to shape local sociocultural attitudes towards rare disease treatments

References

- 1) Kohja et al., Health Care in Gulf Cooperation Council Countries: A Review of Challenges and Opportunities 2017 pp3-5 | 2) Tandon et al., MEASURING OVERALL HEALTH SYSTEM PERFORMANCE FOR 191 COUNTRIES. WHO pp18)
- 3) Bayoumi et al., Genetic disease in the Arab world 2006 | 4) https://www.cia.gov
- 5) https://www.khaleejtimes.com/news/uae-health/dubai-to-map-genome-of-all-its-residents | 6) Worldpopulationreview.com | 7) worldpopulationview.com
- 8) https://www.thiqa.ae/en | 9) https://www.allianzcare.com/en/support/health-and-wellness/national-healthcare-systems/healthcare-in-turkey.html | 10) MOHAP-U.S.-U.A.E Business Council January 2019 roundtable p11 | 11) Saudi Food and Drug Administration | 12) https://pharmaintelligence.informa.com/~media/informa-shop-window/pharma/files/pdfs/whitepapers/turkey-white-paper-final_11-2016.pdf
- 13) Czech et al., A Review of Rare Disease Policies and Orphan Drug Reimbursement Systems in 12 Eurasian Countries p8 | 14) Kanavos et al., 2018 p17
- 15) Tayeb 2019 p2