A Comparative Study of the Current Status and Regulation of Orphan Drug Regulation in Saudi Arabia, USA, Europe, Australia and Japan

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Background

- Given their rarity, rare diseases had not been gaining a considerable attention by drug developers. This issue was attributed to the limited number of patients who can possibly enrol in clinical trials and the low probability of return on investment due to the low demand. Orphan drugs policies were created to tackle this problem.
- The goals of orphan drug policies include encouraging drug developers to develop treatment options for rare and diseases, accelerating availability of these drugs and facilitating accessibility.
- In regions that adopted a policy, the impact of the application of these regulations was measured by the number of medications and the effect of treatment on disease outcomes after implementation.
- By 2020, 46% of the world countries have orphan drugs policies. Rare diseases patients living in regions where there is no policy might remain untreated or experience delay in treatment which, in some cases, affects treatment outcomes.
- Saudi Arabia (SA) is one of the countries that does not implement a policy the landscape of orphan drugs in the region is unclear. So, this study was conducted to contribute to orphan drugs field in the region and assist in elucidating previously unmeasured aspect.

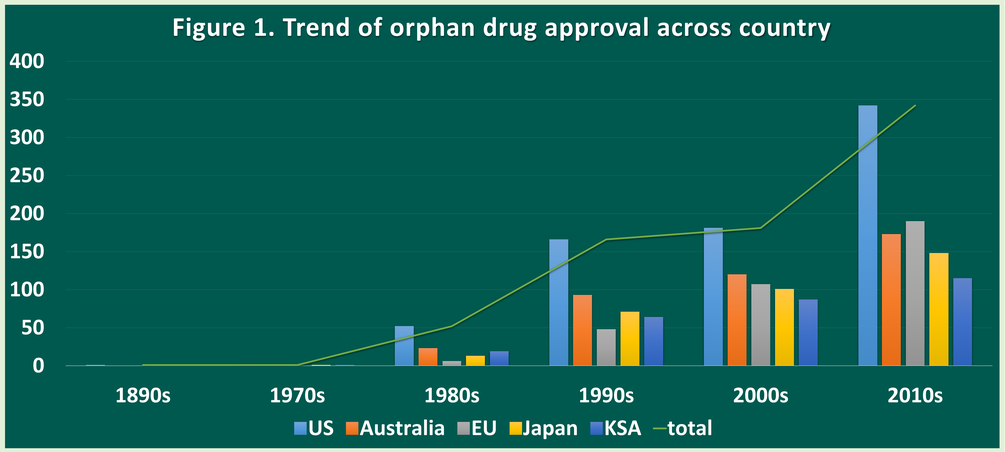
Objectives

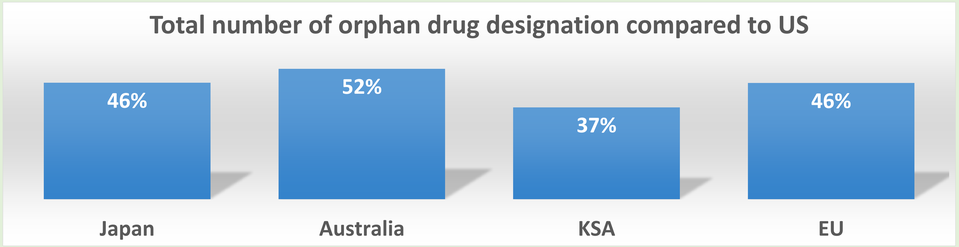
To examine availability of orphan drugs in Saudi Arabia (SA) compared to the United States (US), Japan, Australia and the European Union (EU).

Methods

- Orphan drugs with active designation status and with marketing authorization in the US, Japan, Australia, the EU and Saudi Arabia were accessed and collected using US Food and Drug Administration (US FDA), Japan's National Institute of Biomedical Innovation, Health and Nutrition registries, Australia's Therapeutic Goods Administration, the European Commission community registers as well as Saudi Food and Drug Authority (SFDA).
- Approved orphan indications, orphan routes of administration, pharmaceutical dosage forms used for the orphan indications and years of approval for each drug were collected from the same sources. Products that received orphan designations and have inactive, withdrawn or revoked designation status in the previously mentioned regions were excluded. The search was carried out in the period between February 2020 until July 2020 using the active ingredient's generic name based on either one of the four following product's nomenclatures; the International Non-proprietary Name (INN), the British Approved Name (BAN), the United States Adopted Names (USAN) or the brand name. If a product has more than one orphan designation, the date of marketing authorization for the first granted orphan designation was collected.

Figure 1. Trend of orphan drug approval across country





Results

- Orphan drug regulation and policy were vary across countries and that lead to variation in availability and marketing of these products. Figure 1 provide a trend analysis of all product approved in the 5 regions by date of approval.
- Out of 668 orphan drugs with orphan designations and active market exclusivity we identified in this study, 32% were approved and registered in SFDA compared to 91% in the US, 59% in Australia, 53% in the EU and 47% in Japan.
- Of the total products approved in SA, 41% are antineoplastics and immunomodulating agents and 11% are antinfectives.

Conclusion

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Refrences

- Mikami K. Orphans in the Market: The History of Orphan Drug Policy. Social History of Medicine. 2017;32(3):609–30.
- Lexchin J, Moroz N. Does an Orphan Drug Policy Make a Difference in Access? A Comparison of Canada and Australia. International Journal of Health Services. 2019;50(2):166–72
- Mikami K. Orphans in the Market: The History of Orphan Drug Policy. Social History of Medicine. 2017;32(3):609–30.
- Iqvia Institute. Orphan drugs in the United States. https://www.iqvia.com/-/media/iqvia/pdfs/institute-reports/orphan-drugs-in-the-unitedstates-growth-trends-in-rare-disease-treatments.pdf. Published October 2018. Accessed October 25,2020.
- Lichtenberg FR. The impact of new (orphan) drug approvals on premature mortality from rare diseases in the United States and France, 1999–2007. Eur J Heal Econ [Internet].
- Chan AYL, Chan VKY, Olsson S, Fan M, Jit M, Gong M, et al. Access and Unmet Needs of Orphan Drugs in 194 Countries and 6 Areas: A Global Policy Review With Content Analysis. Value in Health. 2020;23(12):1580–91.