

RARE DISEASES

Emerging HEOR Research
Landscape in SAARC Nations

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Introduction

RARE DISEASES

Rare diseases are complex and heterogeneous. Less than 1% of rare diseases have effective treatments and they make up to 10% of all known diseases.

According to the World Health Organisation (WHO), rare diseases are defined as debilitating lifelong diseases or disorders with a prevalence of 1 or less, per 1000 population. Countries across the globe have their own set of definitions for rare diseases, which suit their specific population, health care system and resource requirements (see Figure 1).

In SAARC nations, country-specific categorization of rare diseases is a far-fetched aspect, as most of the nations are yet to legislate policies for rare diseases.

10% of all known diseases are rare diseases

Upto 80% Rare diseases are inherited

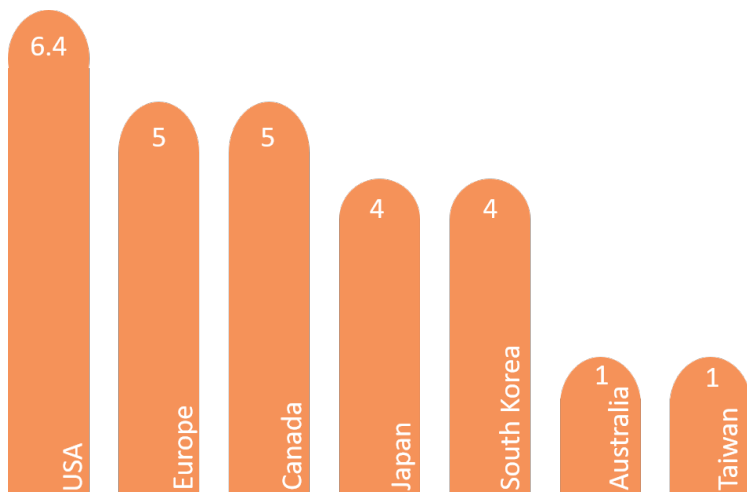
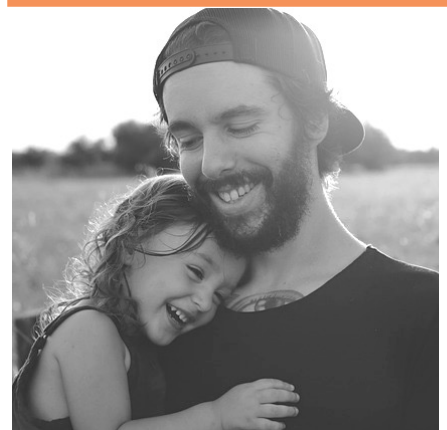


Figure 1 - Definition of rare disease in different countries (Prevalence per 10,000 population).

Source: National Policy for Rare diseases, 2021; Ministry of Health and Family Welfare, Govt. of India



SAARC Countries

The South Asian Association for Regional Cooperation (SAARC) aims to integrate the economics and improve the quality of life of the peoples of its eight member countries (Afghanistan, Bangladesh, Bhutan, India, Maldives, Nepal, Pakistan, and Sri Lanka) [1].



Awareness

With one of the poorest health indicators of the world, rare diseases in SAARC countries face fundamental challenges, ranging from access to basic health care systems, to the lack of funding.



Diagnosis

Social determinants like poverty, food insecurity, illiteracy, poor sanitation, and social stigma act as key constraints in health care. Therefore, there exists a need for a holistic and multi-sectoral approach to improve the public health situation in South Asia from the perspective of rare diseases.



Treatment options

Barriers for treatment

Moving Towards Universal Health Coverage

The United Nations adopted the Sustainable Development Goals (SDGs), also known as the Global Goals, in 2015 as a universal call to action to end poverty, protect the planet, and ensure that by 2030 all people enjoy peace and prosperity. To promote sustainable development, there are 17 linked SDGs, which in turn contain 169 objectives that must be met by 2030 [2]. All member states of the UN have committed to prioritize progress especially for the marginalized sections of society.



The United Nations adopted the Sustainable Development Goals (SDGs), also known as the Global Goals.

Health is interlinked with many other goals and targets of the SDGs, such as poverty, hunger, education, gender equality, water, sanitation, energy, climate change, biodiversity, peace, justice, and partnerships.



There are 17 integrated SDGs, including 169 targets, which are earmarked to be achieved by 2030 to ensure sustainable development.

While improving health can reduce poverty and hunger while enhancing education and gender equality; on the other hand, achieving other goals can also improve health outcomes by addressing social, economic, and environmental determinants of health.

The Center of Health Ethics, University of Missouri aptly defines 'health care access' as the ability to obtain healthcare services such as prevention, diagnosis, treatment, management of diseases, and other health-impacting conditions.

Affordability and convenience are necessary for healthcare to be affordable [3]. Though the last few decades have seen rapid progress in healthcare, a large portion of the global population residing in developing countries, does not have access to adequate healthcare. As per a 2022 estimate by the United Nations Conference on Trade and Development, the percentage of people living in developing countries has gone up from 66% in 1950 to 83% and is expected to reach 86% by 2050 [4].

Interestingly, the challenges in ensuring access to healthcare facilities are also faced by developed nations such as the United States, despite Medicare and Medicaid programs. Inaccessibility to healthcare has been defined as a public policy issue and there are strong opinions of it qualifying as a moral issue as well.

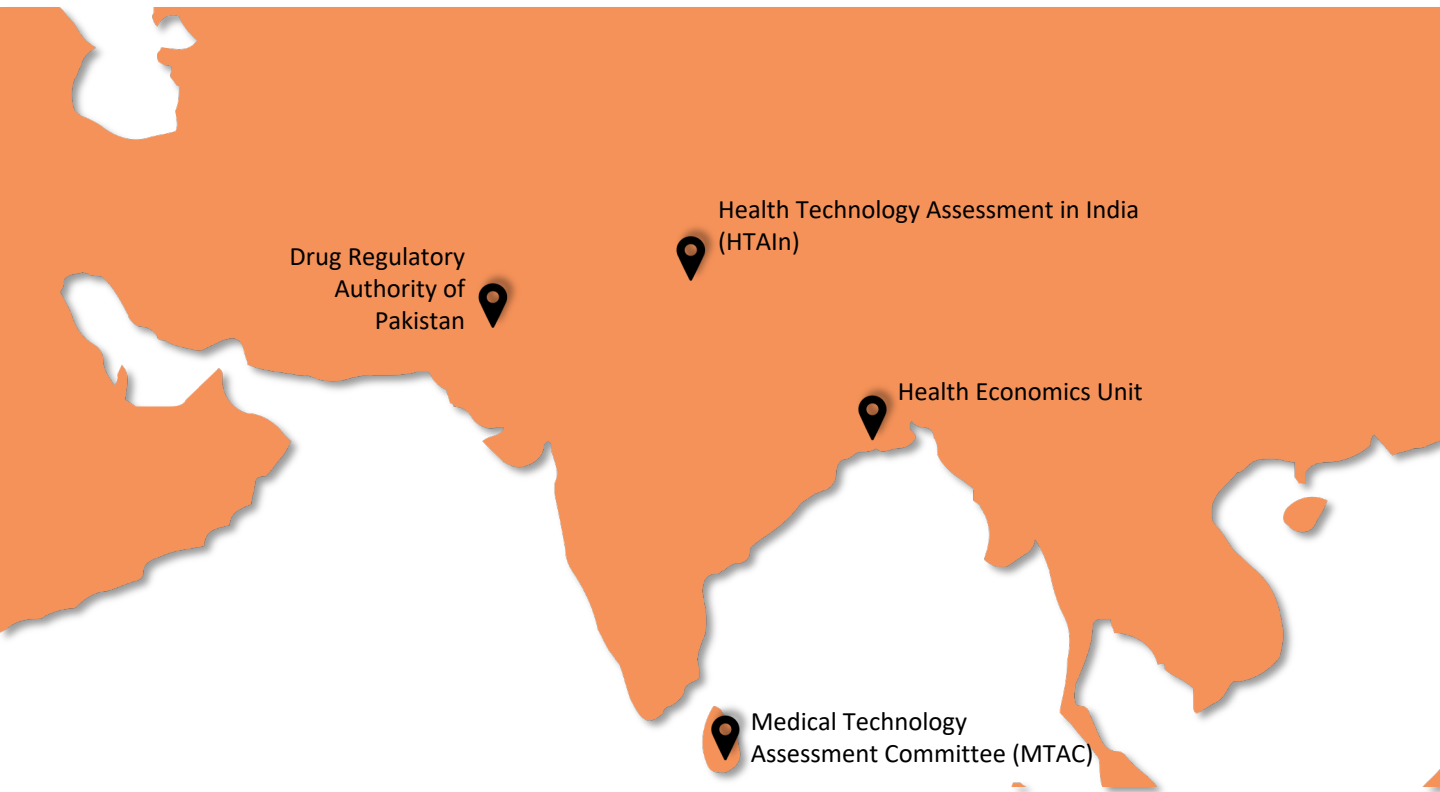


Poverty, barriers to healthcare access, and allocation of resources are three important themes identified in healthcare access. Navigating these challenges can be done through different thought processes.

Health Economics and Outcomes Research (HEOR) is a field of study that evaluates the cost-effectiveness, quality, and impact of health care interventions and policies which go a long way in ensuring healthcare access and reducing inequalities [5]. HEOR is significant for informed health care decision making, especially in the context of limited resources and competing priorities. Of the several challenges faced by these nations, the importance of ensuring access to health services is paramount.

Health, directly addressed by Goal 3 is a key component of the SDGs. Goal 3 has 13 targets and 28 indicators and encompasses a wide range of health issues, such as maternal and child health, communicable and non-communicable diseases, mental health, universal health coverage, access to medicines and vaccines, environmental health, and health financing.

Assessing Health Economic Outcomes



The World Bank classified SAARC nations as 'low- and middle- income countries (LMICs)' [6]. The UN had placed four SAARC countries under 'least developed countries' in February 2021 (Afghanistan, Bangladesh, Bhutan, Nepal) for the next three years. In comparison with developed nations where HEOR is widely practiced, the challenges and inequalities faced by SAARC member states, with respect to healthcare access, are diverse, yet unique.

There is no single HTA authority in SAARC nations and different agencies and institutions conduct HTAs according to country-specific compulsions. Out of the eight SAARC nations, four national HTA bodies have been constituted until now. In this context, it is interesting to note that institutionalization of HTA processes has shown significant advances in the neighboring Association of South-East Asian Nations (ASEAN) [7].

Global efforts to accelerate progress towards universal health coverage (UHC) was escalated following the United Nations General Assembly (UNGA) resolution on 12th December 2012. In simple words, UHC implies that every person irrespective of location should have access to quality, affordable health care. The three dimensions of UHC are population coverage, service coverage and financial protection. The idea of UHC is embedded in the SDG target 3.8, spanning health promotion, prevention, and treatment.

Challenges

Accelerated focus on UHC in Asia has resulted in the rapid advances in science and technology which has made more health technologies available in the market. On the contrary, countries have witnessed increased healthcare spending due to economic development and adoption of high-cost technologies. This presents a problem for the government or payer who must decide whether to include a new technology in the UHC benefits package as well as for medical professionals who want to use the best clinical practices and for patients who may realize that a better treatment option exists but is out of their reach.

HTA development in Asia: Challenges [8, 9]

- ❑ Increasing need and demand for health priority setting
- ❑ Lack of resources, infrastructure, and technical capacity to cope with the increasing demand
- ❑ Inadequate involvement of stakeholders in the HTA process

Approach to HTA

Instead of a 'one-size-fits-all' approach, experts have suggested that countries need customized efforts to fit existing and often complex coverage, reimbursement, and pricing schemes, keeping in mind country-specific socio-cultural aspects.



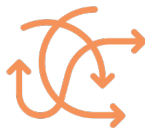
Population

Assessing treatment effect: Small sample sizes; Population heterogeneity; Lack of comparators



Instruments

Lack of validated tools to assess efficacy and effectiveness endpoints



Generalization

Difficulty in applying cost — effectiveness thresholds or value framework

To deal with these challenges, many countries rely on health technology assessments (HTA) for determining priorities; however, the mode of implementation depends on the health system design and local social values. Historically, outcomes research and pharmacoeconomic, which provide the foundation of HTA, were introduced in Asia in the late 1990s whereas many European countries, Canada, and Australia had already institutionalized HTA around the same time. It is interesting to study the differential progress made by Asian countries towards institutionalizing healthcare priority setting through HTAs.

In December 2021, a resolution was adopted by the UNGA [10] on Persons Living with a Rare Disease, which calls on countries to strengthen health systems to provide universal access to healthcare services without financial hardship. This call has multiple implications in SAARC nations where the HTA scenario is still evolving. Further, these nations have their own challenges in terms of geo-political and socio-economic aspects which in turn impacts setting of priorities.

While the global scenario on rare diseases has witnessed multiple initiatives since the late 90s with the emergence of global multi-stakeholder networks such as Orphanet, sadly, none of the SAARC nations have been looped in such initiatives. As a result, there remains a significant gap in SAARC nations, both in terms of evaluating health economic outcomes in general and rare diseases, in particular.



Diagnostic delays

Lack of awareness, expertise, and infrastructure; rare disease patients face long and frustrating journeys to get a correct diagnosis



Social and Economic disparities

Rare disease patients and their families often face social and cultural exclusion, stigma, discrimination, and isolations



Lack of available treatments

Many rare diseases have no effective treatments or cure



Evidence synthesis and patient advocacy

Lack of evidence-based guidelines and multi-stakeholder networks



Lack of access

Rare diseases which have treatment options may not be accessible or affordable for patients in South Asia



Priority settings

Many nations are yet to legislate policies for tackling rare diseases



Appropriate health services

Rare disease patients often require specialized and multidisciplinary care, which are not available or easily accessible in South Asia



Limited political visibility and prioritization

Persuade a customer to purchase a product that fulfills their needs

Multiple challenges in evaluating health economic outcomes for rare diseases are faced by SAARC nations where evidence generation, synthesis and value communication processes are in nascent stages at present.

Despite these challenges, member states have also prioritized rare diseases, albeit at an individual level. For example, India has legislated a National Policy for Rare Diseases (NPRD), recently in 2021.

A focus on Rare Cancers



Rare cancers, are a subset of “forgotten cancers” that requires special care as the treatment outcomes are worse due to persistent lack of attention. Rare cancers represent around 20% of all cancers in Asia as well in Europe [11]. Similar to rare diseases, barriers in treatment of rare cancers include diagnostic issues, lack of randomized data on treatment and uncertainty in treatment protocols. Data on the burden and epidemiology for rare cancers in SAARC countries is lacking at present.

The European group RARECAREnet has identified a three-tier list of 198 cancers based on the

incidence of cancers $\leq 6/100,000$ populations. A recent study has reported similarities and differences between rare cancers in Europe and four SAARC nations (India, Nepal, Bhutan and Sri Lanka) [12].

The findings highlighted that cancer epidemiology was distinct in SAARC countries, underlining the need for separate recognition of rare cancers in this region based on the realistic standard definition and arbitrary lower cut-offs [12]. In SAARC nations, rare cancers merit greater attention from the oncology community and funding organizations. Furthermore, there is a requirement for data on rare cancer histologies, care and survival patterns, to enable better comparison with international counterparts.

Tackling Rare Cancers in Asia

The Asian National Cancer Centers Alliance (ANCCA) was established in 2005, involving nine national cancer centers (NCCs) in the region (Bangladesh, China, Japan, Korea, Mongolia, Pakistan, Singapore, Thailand and Vietnam) to address common issues and concerns in the fight against cancer. The alliance now consists of 12 full members and 7 associate members. Interestingly, five SAARC member states, Bhutan, Nepal, Pakistan, India, and Sri Lanka are ANCCA members. ANCCA has earmarked improvements in medical standards (from diagnosis to supportive care, for all types of cancers, including rare and pediatric cancers) as goals to be achieved under the third phase (2028-2030).

In recent times, the National Cancer Center Hospital, Japan launched the ATLAS (Asian clinical TrialS network for cAnCerS) project, in 2020, funded by the Japan Agency for Medical Research and Development (AMED). The project aims to establish a clinical research network across Asia (Malaysia, Vietnam, Thailand, the Philippines and Indonesia) which will improve oncology drug access in Asia, establish cancer genomic medicine, drug development, and also focus on trials for rare cancers. The project is envisaged to instigate collaborative investigator-initiated trials, and address unmet needs in the region.



Conclusion

It is estimated that 300 million people in the world live with a rare disease and over 45 million people belong from the Southeast Asia. Although the challenges associated with rare diseases (including rare cancers) have been well documented over the last couple of decades, yet, its translation into actionable initiatives is still far-fetched from the perspective of SAARC nations.

Due to the lack of authentic epidemiological data for SAARC nations, they fail to legislate policies and implement necessary rare diseases related counter-strategies, along with inappropriate categorization and definition of rare diseases. Some strategies commonly employed by developed countries include meticulous and vigilant development of drugs by incentivizing industry ensuring patients access to subsidized drugs, grants to researchers and tax incentives on expenditure. Similar approaches can be adopted in SAARC nations to fast-track research, development and universal health coverage.

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Our Team

Leadership



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About Indence

Indence Health® is a full-service healthcare and life sciences consulting firm whose practice area straddles across Health Economics, Market Access, Competitive Intelligence, Medical affairs, Regulatory writing and go-to-market (GTM) strategies. Indence Health is a passionate young start-up and has been founded by partners having an association of nearly two decades. The founding team has an overall enriched experience of more than twenty-five years spread across med-tech, pharmaceutical and HEOR consulting, which we desire to leverage upon for servicing the best-in-class solutions to our customers.