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Access to approved medicines is essential, but it is rarely the limiting factor on its own

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Rare diseases remain among the most complex and least visible challenges facing healthcare systems, particularly in regions where genetic factors amplify the burden. In this conversation, Ahmed Salem, Head of AstraZeneca Rare Diseases in the Gulf, reflects on how rare disease strategy is translated into regional action, spanning innovation, diagnostics, clinical research, genomics, and healthcare system readiness. The discussion offers a grounded perspective on what it takes to move beyond access and deliver earlier, more equitable care across fragmented pathways.

What role does rare disease play within AstraZeneca's strategy, and how does this translate into priorities for the Gulf region?

Rare diseases constitute a substantial yet persistently under-recognised public health challenge. Globally, it is estimated that around 400 million people live with a rare disease across up to 10,000 identified conditions, a number that continues to increase as genetic knowledge advances. Despite this scale, close to 90 percent of rare diseases still have no approved treatment. The impact is particularly acute because most rare diseases are genetic in origin, often presenting in early life, and many patients, especially children, face long and fragmented diagnostic journeys that place a heavy burden on families and caregivers.

In the Gulf, these challenges are often amplified by regional epidemiology. Higher rates of consanguinity increase the prevalence of inherited disorders, while demographic factors can compound the impact within families by the time a diagnosis is made. At the same time, it is important to recognise that rare disease extends beyond genetics alone, and our focus reflects this breadth across multiple therapy areas. Against this backdrop, AstraZeneca's acquisition of Alexion marked a deliberate strategic decision to build a dedicated rare disease platform. Rare Disease is now one of AstraZeneca's three core therapy areas, alongside Oncology and BioPharmaceuticals, supported by sustained investment in research and development and a growing pipeline of innovative medicines.

However, innovation alone is not sufficient. In rare disease, access to approved therapies is often not the primary constraint. Medicines may be available, yet patients remain undiagnosed or disconnected from care. Our priorities therefore extend to working closely with governments, medical societies, and healthcare systems to shorten diagnostic pathways, strengthen awareness, and build medical readiness. By addressing these systemic barriers alongside continued investment in innovation, we aim to move rare disease patients from being largely invisible within healthcare systems to receiving timely and equitable care across the region.

How does AstraZeneca Rare Disease's portfolio align with the scientific and epidemiological realities of the Gulf region?

AstraZeneca Rare Disease, built on the platform established by Alexion, has developed its leadership by pioneering complement biology. Since the acquisition of Alexion was completed in 2021, we have continued to invest in this core scientific franchise while expanding across a broader range of rare disease areas, including haematology, nephrology, neurology, metabolic disorders, and rare cancers. Today, the portfolio spans several therapy areas and is supported by a pipeline that combines lifecycle expansion of existing medicines with the development of new modalities, including gene and cell therapies, where we believe there is meaningful potential to address high unmet need.

At a global level, scientific priorities guide pipeline decisions, ensuring consistency and depth of focus. At the same time, regional epidemiology plays an important role in shaping how we deploy resources locally. In the Gulf, genetic factors can lead to higher prevalence of certain rare diseases, which influences how we prioritise education, partnerships, and diagnostic initiatives. This often requires targeted engagement beyond major urban centres to ensure that awareness and

diagnostic efforts reach the communities where the burden is greatest.

From a market access perspective, key Gulf countries provide timely regulatory pathways, and approved medicines are generally available. However, access to treatment does not automatically translate into impact. The more complex challenges sit earlier in the care pathway, where diagnosis can take many years and referral networks remain fragmented. In rare disease, patients may reach specialist care late, and clinicians may encounter conditions they have seen only once or not at all, while centres of excellence are still evolving.

This reality shapes how we think about our role in the region. Alongside bringing our full portfolio to the Gulf, we place equal emphasis on working with health authorities and medical societies to strengthen diagnostics, referral pathways, and specialist capability. By aligning innovation with system readiness, our objective is to ensure that scientific progress translates into earlier diagnosis and more effective care for patients living with rare diseases across the region.

How is AstraZeneca Rare Disease working to move from regulatory approval to effective patient identification and system readiness across the Gulf?

In rare disease, access to approved therapies is an important milestone, but it does not in itself solve the problem. Our focus is on helping to move care beyond availability towards earlier identification and more equitable management of patients who often remain unseen within healthcare systems. This is not something that AstraZeneca can address in isolation. Meaningful progress depends on sustained public private collaboration that links awareness, policy direction, and clinical practice.

A critical enabler is political prioritisation. When rare disease is recognised as a public health issue, it influences tangible system decisions, including the expansion of newborn screening, the development of recognised centres of excellence, and the creation of structured referral pathways that guide patients to appropriate expertise more efficiently. Data infrastructure also plays an increasingly important role. Where governance frameworks allow, integrated health information platforms can support earlier diagnosis and better coordination of care, reducing the risk that patients fall through gaps in fragmented systems.

Alongside policy, medical readiness remains a central challenge. Diagnostic capacity is uneven, and many frontline physicians encounter rare diseases too infrequently to recognise them early. As a result, patients may move between specialties for years before reaching the right clinic.

Addressing this requires targeted education for frontline clinicians, clearer guidance on when to suspect a rare condition, and practical support to improve access to specialised diagnostic tests.

Even when patients reach specialist care, expertise and capacity must be available at scale.

Centres of excellence are emerging, but coverage remains limited. This is why, at AstraZeneca, our role extends beyond bringing innovation to market to supporting health authorities and medical societies in strengthening diagnostics, referral networks, and specialist capability. The priority now is to translate intent into coordinated action that delivers earlier diagnosis and more consistent care for patients living with rare diseases.

How does clinical research and evidence generation support access and decision making for rare diseases in the Gulf?

Clinical research sits at the core of how we advance rare disease care, and the Gulf is increasingly positioned to contribute at a global level. Across the UAE, Saudi Arabia, and neighbouring markets, there is clear momentum to expand participation in Phase II and Phase III studies, supported by government ambition to strengthen research capability and ensure regional patient populations are represented in international development programmes. We are working closely with health authorities to broaden this footprint and embed the region more firmly within global clinical trial networks.

That said, late-stage trials, while essential for establishing efficacy, safety, and regulatory approval, do not on their own address the practical questions faced by health authorities and payers in rare disease. Patient numbers are limited, prevalence data are often incomplete, and care pathways vary across settings. Local evidence generation therefore becomes critical. Registries, observational studies, and structured collection of real-world outcomes help build a clearer picture of how patients are diagnosed, treated, and followed in routine practice, and where gaps in care persist.

This type of real-world evidence complements clinical trial data by translating scientific results into locally relevant insight for health technology assessment, reimbursement, and service design. In markets such as the UAE, where formal HTA frameworks are in place, these data are increasingly central to policy and coverage decisions. By advancing global clinical research alongside robust local evidence generation, our objective is to support more informed decision making and ensure that innovation in rare disease delivers measurable impact for patients across the region.

How do national genomics initiatives in the Gulf reshape the outlook for rare disease diagnosis and care?

National genomics programmes are advancing at pace across the Gulf and are beginning to reshape how rare diseases are understood and managed. Initiatives such as the Saudi Human Genome Program, the Emirati Genome Program, and the Qatar Genome Program have already generated substantial genomic datasets, reflecting a clear commitment by governments to precision medicine, earlier diagnosis, and a deeper understanding of genetic disease patterns within local populations. In a region where inherited conditions are more prevalent, this scale of genomic insight has particular relevance for rare disease.

The real significance of these programmes lies in their ability to shift care upstream. Genome sequencing enables earlier risk identification and, in some cases, prevention, rather than waiting for symptoms to emerge after years of uncertainty. Governance frameworks are still evolving, and unlocking the full value of these datasets depends on close collaboration between public institutions and industry, particularly around responsible data access for research and clinical development. Where this alignment exists, genomics can also strengthen clinical trial readiness and improve the ability to identify and stratify patients earlier and more accurately.

Newborn screening provides a clear illustration of this progress. In Qatar, genome-based newborn screening initiatives are already being implemented to identify hundreds of treatable rare diseases at birth, extending well beyond traditional screening models. Similar capabilities are emerging in the UAE, supported by national genomics infrastructure. These developments lay the groundwork for earlier diagnosis, more timely intervention, and a healthcare system that is increasingly equipped to address rare disease proactively rather than reactively across the region.

What does meaningful progress in rare disease look like over the coming years, and where will AstraZeneca focus its efforts?

Rare disease progress should be viewed as a long-term transformation rather than a series of isolated achievements. While tangible advances are already evident, the central challenge remains moving patients from being largely invisible within healthcare systems to receiving care that is timely, coordinated, and equitable. Meaningful progress will depend on sustained prioritisation and system-level awareness, because when rare disease is consistently recognised as a public health

priority, it shapes decisions that influence diagnosis, referral pathways, and the overall organisation of care.

In practical terms, success will be reflected in shorter diagnostic journeys and the development of centres of excellence that can operate at scale. Access to approved medicines is essential, but it is rarely the limiting factor on its own. Without robust diagnostics, clear referral networks, and sufficient specialist capability, availability does not translate into real-world impact. Through AstraZeneca, our focus will remain on partnership across the ecosystem, working alongside health authorities and other stakeholders to strengthen system readiness, support health equity, and ensure that scientific progress delivers lasting benefit for patients and their families across the region.

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