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10.02.2026

Tags: UAE, MEA, PDC-CRO, CRO, Clinical Trials, Data, Rare Diseases

Dr Rania Alshami reflects on two decades in the Middle East and North Africa clinical research ecosystem, charting the region's regulatory maturation, rare disease opportunities, and growing role in early-phase trials. She discusses patient access, genomics integration, sponsor engagement, and why MENA is emerging as a credible hub for innovation in rare diseases and advanced therapies.

You have spent almost two decades in the Middle East clinical research space, with the last four years at PDC-CRO as chief business and strategy officer. Why did you decide to dedicate your career to the CRO industry rather than academia or pharma?

After completing my PhD in cell biology at the University of Delaware, I moved to Dubai with the intention of building an academic career. I joined what was then Dubai Biotechnology and Research Park – now Dubai Science Park – as part of its founding team, marking my first exposure to the contract research organisation world, as CROs were a target segment for the park. It allowed me to see the full continuum from bench to bedside and to understand how the various stakeholders fit together – from preclinical and clinical researchers to market access professionals and regulators.

I entered the CRO industry almost accidentally. Through my work with clients, I met the then CEO of Ergomed, and we supported the establishment of their Dubai operations as the first global CRO

to enter the region. After leaving Dubai Science Park, I joined Ergomed, learning regulations, processes, and execution from the ground up. My first study was a rare disease trial, and that set the trajectory. Nearly 20 years later, I have worked with multiple CROs across the region, consistently focusing on rare diseases and working to make the ecosystem more conducive to research – at the regulatory, site, and patient levels.

I joined PDC four years ago, which felt like a natural progression. I had known the CEO, Mohamed Mostafa, for many years, and he invited me to help grow the company and expand its capabilities. It was an opportunity I could not refuse.

Looking back over recent years, how has the region evolved – particularly in rare diseases? Could you comment on regulation, ecosystem readiness, and sponsor confidence?

One essential point when discussing MENA is its extraordinary diversity – in population, culture, infrastructure, and regulatory maturity. I began my career in the Gulf Cooperation Council (GCC) countries, particularly the UAE, while also working closely with Saudi Arabia. At that time, regulatory frameworks were developing and all stakeholders were working towards addressing some clear gaps in the process and capabilities– particularly around drug importation, sample exportation, sites experience, approvals timelines, contracting amog others.

In contrast, the Levant presented a different picture. Jordan's FDA was exceptionally well established and, at the time, represented the gold standard in the region in terms of regulatory readiness. Egypt had clear clinical research regulations under the Ministry of Health, while Lebanon, although lacking a regulatory authority providing approvals for clinical studies, offered an efficient framework with extremely short timelines based on sites IRBs approvals. These countries benefited from highly experienced sites, largely due to their role in running complex rescue trials when recruitment faltered elsewhere. In North Africa, Tunisia and Morocco demonstrated solid experience, while Algeria showed strong potential due to the market size but remained difficult to manage operationally.

Fast forward 20 years, and the transformation in the GCC – particularly Saudi Arabia and the UAE – has been remarkable. I have worked closely with regulators and sites, supporting the establishment of ethics committees, institutional review boards, standard operating procedures, and training programmes. On the regulatory side, we contributed to drafting new national frameworks, including those published by the UAE's Ministry of Health and prevention. Through

coordinated efforts by governments, CROs, pharmaceutical companies, and sites, these countries have reached a highly competitive regulatory standard. Orphan and rare disease pathways now exist in both countries; in the UAE, for example, drugs can be registered based on local data generation, enabling commercialisation prior to EMA or FDA approval. This is particularly attractive for small biotechs seeking early revenue streams.

Jordan remains exceptionally strong while Egypt has progressed, although sample exportation continues to pose challenges. Lebanon remains attractive for specific indications, particularly haematology and oncology.

Notably, the GCC now boasts truly state-of-the-art facilities. With the rise of cell and gene therapies, licenced, ready-to-operate centres have been established. We once compared the Middle East to Eastern Europe of a decade earlier, but the region has leapfrogged forward, particularly with genome initiatives and artificial intelligence programmes.

Despite the high disease burden that they represent, rare disease trials remain limited in the region. Where do you see the opportunity to change this?

This has been my focus for two decades. I often say that I look forward to the day when I am selling PDC’s services rather than selling the region itself. At global conferences, the conversation is still largely about explaining what the region can offer. Sponsors are frequently unaware that high-quality clinical research infrastructure exists in the Middle East. Once that hurdle is cleared, the focus shifts to two questions: do you know where the patients are, and are the investigators qualified?

The burden of rare disease is substantial. We are speaking about a rare patient population of approximately 25 million, comparable to the US and Europe, with a high incidence of metabolic, haematological, and neurological diseases. However, rare disease trials here represent at most three percent of global activity.

The gap is multi-layered. Awareness is a major issue – sponsors cannot value what they do not know exists. Political perceptions and regional instability contribute to a heightened risk perception, particularly among biotechs, which are inherently risk-averse. There is also a lack of structured data – registries, publications, and accessible datasets that clearly demonstrate where patients are.

Sponsors typically rely on global CROs, many of which have limited presence in the region. These CROs tend to work through local vendors, making it commercially unattractive to allocate studies here. Initial feasibility assessments are desk-based and exclude the region entirely due to a lack of internal data.

We regularly encounter sponsors who already know specific investigators in Saudi Arabia or elsewhere have sizeable patient cohorts, yet struggle to activate studies through their global CRO partners. In these cases, we reverse-engineer the process.

Raising awareness remains essential, but we have found that direct engagement with biotechs and principal investigators is most effective. Data is the decisive factor. When we demonstrate that sponsors can recruit 50 patients in one regional country versus five across Europe, the conversation changes. For biotechs in particular, the most critical milestone is first patient in. When we walk them through the regulatory process and demonstrate how efficiently start-up can be achieved, their interest grows rapidly

We have therefore focused on collaborating with global CROs, positioning ourselves as their regional execution partner. The ability to demonstrate access to patients in markets that also support future commercialisation is compelling.

Progress requires collective effort – from regulators, CROs, and, critically, principal investigators and key opinion leaders who must ensure the world knows where patients are.

Do you engage differently with biotechs versus large pharmaceutical companies?

Our approach varies significantly depending on whether we are working with large pharmaceutical companies, biotechs, or global CROs, as each enters the region through a different decision-making route.

For large pharmaceutical companies, engagement is typically driven by local and regional affiliates. These teams are embedded in the healthcare systems, habitually within dedicated rare disease units, and have a strong understanding of patient distribution, investigators, sites, and regulators. In many cases, they are already convinced of the region’s value and focus on building the internal case to headquarters. As headquarters are usually aligned with a global CRO, our role becomes one of coordination – strengthening the data, supporting the internal pitch, and, where mandated, acting as the regional execution partner alongside the global CRO.

With biotechs, the relationship is more direct and long-term. Building confidence in the region can take up to two years and involves extensive knowledge exchange around patients, regulation, and operations. Our role goes beyond clinical delivery, including support with investor connections where promising assets lack the financial runway. These engagements frequently evolve into true partnerships, particularly where patient populations are concentrated in the region or global recruitment has stalled.

Global CROs form a third group. As they are the primary interface with sponsors, a significant part of our work involves educating their teams about the region and equipping them with credible data. Their understanding is critical, as it directly determines whether MENA is even considered in global study allocation decisions.

The region is investing heavily in genomics and population-level data. What is still missing to translate this into clinical trials?

The issue is not a lack of activity, but fragmentation. Significant investments have been made across the region, yet genomic data is only valuable if it is connected to clinical action.

Large genomic datasets now exist, but they tend to reflect national populations and exclude expatriate communities, which in countries such as the UAE represent the majority of residents. This means a substantial proportion of rare disease patients remains outside formal datasets. Even within local populations, there is a disconnect between genome projects and clinical care. Individuals may be identified with disease-associated mutations years before symptoms emerge, but there is no systematic mechanism to track them over time or link them to research opportunities when disease manifests.

What is needed is an integrated, multi-omics framework that connects genomic data with electronic medical records, diagnostics, and longitudinal clinical follow-up, supported by a robust consent model that enables research use and trial matching. Disease registries, natural history studies, and real-world evidence must be linked into a single pathway. While many of these elements exist, they are not yet connected. Without that integration, it remains difficult to confidently translate population-level insight into actionable clinical trial recruitment.

Access remains a challenge, even with orphan drug programmes. How do you assess the current landscape?

Access remains highly uneven. In the wealthier states, local populations typically have access to orphan drugs through government-funded healthcare systems. For expatriates, access is mediated through private insurance, where coverage of high-cost therapies is far from guaranteed. This creates significant uncertainty in countries where expatriates form the majority of the population.

In less wealthy countries across North Africa and the Levant, access is even more constrained. Orphan drugs are generally not reimbursed or only very selectively funded. In these contexts, clinical trials are not simply a research activity; they are frequently the only route to treatment and, in some cases, the only option for survival.

Gene therapies are forcing governments in the region to confront high upfront costs. How should value be assessed for these treatments, and how does this play out across different healthcare systems in the region?

Many gene therapies carry extraordinarily high price tags. This is not a regional issue; it is a global one. When governments act as the payer, the discussion inevitably turns to value.

If you assess the lifetime cost of caring for a rare disease patient – including repeated hospitalisations, chronic treatment, and long-term support – the economics begin to look different when compared with a one-time, high-cost intervention. Yet this analysis cannot be purely financial.

Quality of life must be central to the equation. Keeping a patient alive through ongoing medical intervention may cost governments millions over time, but it can come at the expense of quality of life for both patients and caregivers.

For now, the UAE and Saudi Arabia are relatively well positioned to absorb these costs. For other countries in the region, our role is to help bridge the gap through clinical research and access pathways, offering patients and families realistic hope.

Looking ahead, the region aims to move from importing therapies to generating early clinical evidence. What is driving this shift, and why does it matter strategically?

The region is rapidly establishing itself as a credible player in cell and gene therapy. This shift has been enabled by foundations that are now firmly in place – advanced infrastructure, increasingly capable clinical sites, and regulators who are both pragmatic and open to innovation. When

combined with new investment and funding mechanisms, this has translated into a marked increase in phase one and phase two activity.

We are directly involved in a growing number of these early-phase trials across the GCC, as well as in Lebanon and Jordan. Today, early phase studies account for roughly 70 percent of our portfolio at PDC. This evolution is still not widely recognised, yet it is strategically significant.

In rare diseases, competition is rarely singular. Multiple companies are often pursuing the same indication. Those that move early, recognise where patient populations are concentrated, generate local clinical data, and establish relationships with regulators and sites gain a decisive first-mover advantage. Those that delay risk watching competitors recruit patients in the region while they struggle to enrol one patient per year elsewhere. That is the practical reality, and it is the message we consistently convey to sponsors.

Finally, what excites you most about the future – both for the region and for PDC CRO?

For me, it feels like watching something you have nurtured for 20 years finally come of age. Despite challenges, the region has demonstrated resilience, ambition, and an unwavering commitment to raising standards.

The next frontier is patient empowerment. Patient advocacy groups are central drivers of progress globally, and while initiatives exist in the region, they require greater support and visibility. Many ultra-rare disease groups already know exactly where patients are – they simply lack a platform.

Our role at PDC is to help bridge that gap, supporting patient and investigator engagement and amplifying their voices globally. If we know a patient exists, they should be on the radar for potential treatment or trial participation.

The future is genuinely bright. The region has moved beyond adolescence and into maturity – confident, capable, and ready.

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