Partner Rare

Navigating Gene Therapy Commercialization in Europe

A Q&A with Joshi Venugopal, General Manager of Gene Therapies and Rare Diseases, Europe

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Gene therapies hold immense promise for revolutionizing the treatment of rare diseases, offering potential cures where previously only symptom management was possible. However, the path from groundbreaking research to patient access is fraught with complexities, particularly in navigating the distinct regulatory and commercial landscapes of Europe and the United States.

While the U.S. has pioneered gene therapy approvals, Europe presents unique challenges, including fragmented healthcare systems, varying reimbursement policies, and diverse patient access pathways. These complexities often lead to delays in bringing life-saving therapies to European patients.

In this exclusive Q&A, Partner Rare's Founder and Chief Executive Officer, San Godhania, sits down with Joshi Venugopal, General Manager of Europe for Novartis Gene Therapies & Rare Diseases, a global leader in innovative medicines. Drawing from his vast experience at the forefront of this rapidly evolving field, Dr. Venugopal offers a perspective on the hurdles faced by companies and the strategies that can pave the way for success.

Expert Insights:

A Q&A with Joshi Venugopal

San: What's your take on the future of gene therapies?

Joshi

In one word: bullish. Gene therapy has mainly tackled rare monogenic disorders so far, but these conditions aren't as rare as the name implies. They affect about 10% of the population collectively. With a plethora of rare diseases lacking treatment, there's a glaring medical need. The pipeline is booming, regulatory approvals are on the rise, and technological advancements like vector improvements and gene editing are boosting the field.

San: Europe is perceived as a challenging market for gene therapies. Do you see that changing?

Joshi

It's early days and it is a mixed bag at this point. While some have struggled with access issues and a few have even withdrawn, we've successfully gained broad access for the two therapies in our portfolio. So far, we have access in 30+ countries for our gene therapy for a neuromuscular disorder and 20+ countries for the gene therapy for a rare retinal disorder. Yes, one will face challenges, but it is not insurmountable if one is committed to find a way for our patients.

San: What's your key takeaway from working with payers?

Joshi

Traditional healthcare systems are geared towards chronic treatments with deferred payments. One-time gene therapies shake up this model. Payors face upfront costs for treatments based on small clinical trials, with long-term data still in the making. Collaboration between pharma companies and payors on innovative payment models like risk-sharing and outcome-based agreements are starting to take shape However, these are cumbersome to implement and as the number of reimbursed gene therapies increase, we will have to rethink how we approach this.

San: Any learnings other than innovative pricing models?

Joshi

Absolutely, leveraging early diagnosis and real-world evidence can significantly enhance patient outcomes and demonstrate the value of treatments. Early diagnosis allows for timely intervention, potentially improving the effectiveness of treatments and reducing the burden of the disease on patients and healthcare systems. Furthermore, real-world evidence provides valuable insights into how treatments perform in everyday clinical practice, beyond the controlled environment of clinical trials.This can help identify areas for improvement, optimize treatment strategies, and build confidence in the effectiveness and safety of treatments among healthcare professionals, patients, and payers. Linking reimbursement to real-world outcomes can incentivize pharmaceutical companies to develop treatments that demonstrate long-term benefits and encourage continuous monitoring of patient outcomes post-approval. It also promotes a collaborative approach between stakeholders, fostering a shared responsibility for patient well-being and optimal healthcare delivery.

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San: What advice do you have for smaller companies that aim to launch in Europe?

Joshi:

If you're serious about Europe, start gathering insights before designing your pivotal trials. Understand the specific access pathways for one-time therapies, like the Highly Specialized Technologies Appraisal (HSTA) in the UK, and how drugs will be rated by HTA bodies, such as ASMR ratings in France. Learn which countries allow real-world data in P&R decisions and which permit indication-based net realized prices. Incorporate these factors into your trial design, post-launch registry setup, and access strategy.

Smaller companies with limited resources will need to prioritize where to launch first. Engaging with rare disease industry experts who have specialized knowledge can be very helpful early on. Eventually, you'll need to decide whether to proceed independently or partner with companies that have an established presence and local expertise.

Also, pay close attention to evolving policies like EU-wide pharmaceutical legislation and Joint Clinical Assessments, as well as country-specific laws, such as those proposed in France. With a population of 740 million, I presume Europe is crucial for delivering the promise of your medicine

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Your Trusted Partner in Rare Disease Commercialization

Partner Rare would like to thank Joshi Venugopal for his valuable contributions to the field of gene therapy and his commitment to improving the lives of patients with rare diseases. As this Q&A highlights, navigating the complexities of gene therapy commercialization requires deep expertise and a strategic approach.

Partner Rare is uniquely positioned to guide biotech and pharmaceutical companies through this journey, offering tailored solutions for regulatory affairs, market access, clinical trial design, and commercialization strategies. Our team of seasoned experts understands the unique challenges of the European market and can help you unlock the full potential of your rare disease therapy.

Contact Partner Rare today to learn how we can support your gene therapy development and commercialization goals.

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About Joshi Venugopal

Joshi Venugopal is a seasoned pharmaceutical executive with extensive experience in gene therapy commercialization, new product development, and general management across three continents. His leadership roles include leading the industry-leading Gene Therapy Commercialization efforts for Novartis in Europe and serving as their Global Head of New Products, responsible for the early pipeline.

Joshi has a strong background in research and development, having contributed to Novartis' R&D organizations and holding a PhD in Cell Biology from the University of Basel. He is also an alumnus of the General Management Program at Harvard Business School.