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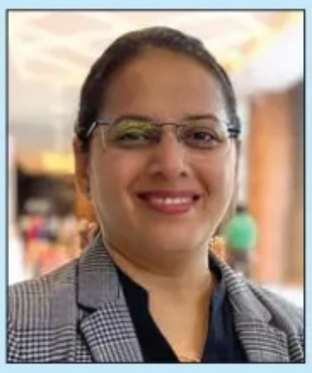
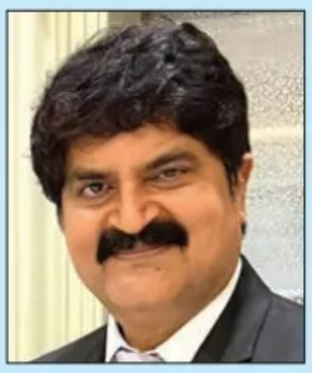
News AT A GLANCE



CPHI and PMEC India helping pharma industry...

Page No. 16

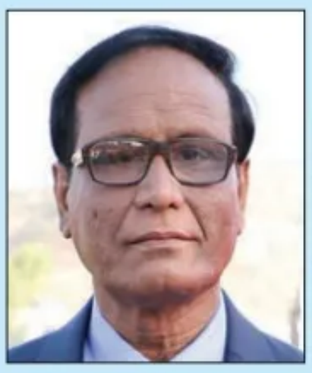


AI, IoT, block-chain shaping pharma...

Page No. 20

Bioaltus Pharma-ceuticals poised for...

Page No. 24



How to Commer-cialize an Ad-vanced...

Page No. 42

Advertiser Index - Pg 60

Sustainability takes centre stage on second day of CPHI India expo

LAXMI YADAV, MUMBAI

PHARMACEUTICAL stalwarts have underscored the necessity of a multifaceted approach to address significant sustainability issues facing the Indian pharmaceutical and biopharma industries, including energy efficiency, waste reduction, lifecycle assessments, eco-friendly packaging, supplier collaboration and transition to net-zero.

Speaking at a leadership panel discussion on "Addressing the Critical Sustainability Challenges in Indian Pharma & Biopharma" on second day of CPHI India expo in Noida on November 27, the industry captains also highlighted the need for charting the right roadmap towards sustainability with Environmental, Social, and Governance (ESG) goals.

Moderated by Chakravarthi AVPS, Global Ambassador, World Packaging Organisation, the panel discussion featured Jerome Gnanaprakasam, Vice President, Dr. Reddy's Laboratories; Sunila Sahasrabudhe, Head -ESG Finance, Biocon; Himanshu Gupta-Director Sales - South Asia, Veolia Water Technologies & Solutions; Chandi Prasad Ravipati, Head Packaging Development, Aurobindo Pharma.

CONTINUED ON p8▶

How to Commercialize an Advanced Therapy: Lessons Learned and Future Plans

DR. SANJAY AGRAWAL

THE commercialization of advanced therapies, such as cell and gene therapies, holds great promise for treating previously incurable diseases. However, these novel therapies present unique challenges in terms of development, production, and distribution. Bringing advanced therapies from the lab to the market requires a deep understanding of regulatory hurdles, supply chain complexities, manufacturing, pricing, and patient access.

This article delves into the critical lessons learned from the commercialization of advanced therapies and discusses future plans for scaling and optimizing this process to meet growing patient demand.

The Rise of Advanced Therapies

Over the past few years, advanced therapies have transformed the medical landscape. Cell therapies, gene therapies, and tissue-engineered products have brought new hope to patients suffering from rare genetic diseases, cancers, and degenerative conditions. These therapies, categorized as Advanced Therapy Medicinal Products (ATMPs), use groundbreaking technologies to target the root cause of diseases at the molecular level.

While the potential for these therapies is tremendous, their commercialization has been far from straightforward. From early R&D to patient access, advanced therapies require an entirely different approach compared to traditional pharmaceuticals or biologics. The lessons learned in navigating this space are crucial for the continued success of the industry.

Lesson 1: Streamlining Regulatory Pathways

One of the first hurdles in commercializing advanced therapies is regulatory approval. Given the novelty and complexity of ATMPs, traditional regulatory frameworks are often insufficient to address the unique challenges these therapies present. Companies have found that early and proactive engagement with regulatory authorities is critical to navigating this complex landscape.

For example, the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) have established specific programs for ATMPs, such as the Regenerative Medicine Advanced Therapy (RMAT) designation in the U.S. and the Priority Medicines (PRIME) scheme in the EU. These programs allow

CONTINUED ON p43▶

How to Commercialize an Advanced...

CONTINUED FROM p42▶

for accelerated approval pathways, but they also require companies to engage with regulators early in the process to ensure clinical trial designs meet the necessary standards.

Companies like Spark Therapeutics, which developed Luxturna (a gene therapy for a rare form of blindness), successfully leveraged these accelerated pathways. Early discussions with the FDA helped Spark align its clinical trials with regulatory expectations, leading to the timely approval of the therapy.

Future Plan: Collaboration with Global Regulators

As the demand for advanced therapies increases globally, companies must expand their regulatory engagement beyond key markets like the U.S. and Europe. Collaborating with regulators in emerging markets will be essential to make these therapies more accessible. Additionally, harmonizing regulatory requirements across regions could help streamline global approval processes.

Lesson 2: Overcoming Manufacturing Challenges

Manufacturing advanced therapies is notoriously complex. Unlike traditional drugs, where chemical synthesis is the primary production method, advanced therapies often require the manipulation of living cells or genes, making the production process much more delicate. Variability in the source material (such as patient-derived cells) and the need for stringent quality control add further complexity.

The scalability of manufacturing is another major challenge. For instance, cell therapies often require personalized manufacturing for each patient, meaning each batch is unique. This "one patient, one batch" approach is time-consuming and costly, posing a barrier to large-scale commercialization.

Companies like Novartis, which developed Kymriah (a CAR-T cell therapy for cancer), have invested heavily in building specialized manufacturing facilities and developing robust processes to ensure product consistency and scalability. However, the high costs of such manufacturing approaches remain a significant barrier to broad commercialization.

Future Plan: Automation and Modular Manufacturing

Looking ahead, the industry must invest in automation and modular manufacturing to scale advanced therapies efficiently. Automated systems for cell expansion, gene editing, and quality control could significantly reduce costs and improve consistency. Additionally,

CONTINUED ON p44▶

How to Commercialize an Advanced...

CONTINUED FROM p43▶

modular manufacturing platforms that can be easily replicated across different locations will help meet growing global demand.

Lesson 3: Addressing Supply Chain Complexities

The supply chain for advanced therapies is radically different from traditional pharmaceuticals. With some therapies requiring patient-specific material (such as cells), the supply chain becomes a "vein-to-vein" process, where patient cells are harvested, shipped to manufacturing facilities, manipulated, and then returned to the patient.

This process requires precise coordination to ensure the timely delivery of the therapy, as well as the maintenance of product integrity throughout the journey. Temperature control, transportation logistics, and handling of biologic materials are critical factors that can make or break the success of the therapy.

For example, the commercial success of Gilead's Yescarta (another CAR-T cell therapy) hinged on establishing a reliable and efficient supply chain. Gilead invested in a global logistics network, including specialized cold-chain transportation, to ensure the therapy could be delivered to patients within a narrow timeframe.

Future Plan: Digitalized Supply Chain and Data Integration

The future of advanced therapy commercialization will require fully digitalized supply chains, integrating data from collection, manufacturing, and delivery points to create real-time visibility. Digital tools, such as blockchain, can help ensure product traceability and improve the efficiency of the vein-to-vein process. Furthermore, collaborations with third-party logistics providers specializing in cold-chain transportation will become more prevalent to support global distribution.

Lesson 4: Navigating Pricing and Reimbursement

The cost of developing and manufacturing advanced therapies is exceptionally high, which is reflected in their price tags. For example, Zolgensma, a gene therapy for spinal muscular atrophy, has a list price of over $2 million. These high prices present a significant challenge for payers, particularly in publicly funded healthcare systems, and have led to debates around the affordability and value of advanced therapies.

To address these concerns, companies are exploring innovative pricing models. Outcomes-based pricing, where payment is tied to the therapy's long-term effectiveness, has gained traction. For instance, Novartis offers outcomes-based contracts for Zolgensma, allowing payers to spread the cost over time or receive refunds if the therapy does not meet specific clinical benchmarks.

Future Plan: Expanding Innovative Pricing Models

As more advanced therapies enter the market, the industry must continue developing and refining innovative pricing and reimbursement models. Pay-for-performance contracts, subscription-based models, and value-based pricing will become essential tools for ensuring patient access while balancing the financial burden on healthcare systems.