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Gene Therapy Reimbursement Remains a Barrier to Commercialization

(Source: An article by Jessica Merrill for Scrip Citeline Commercial)

Multiple gene therapies have been approved in the last few years, but market access and reimbursement for gene therapies remain challenging. With several gene therapies now on the market, the industry's focus is turning from the development and regulatory landscape of the complex products to the commercial front, where payments for durable treatments with multimillion-dollar price tags continues to be an obstacle.

At the Cell & Gene Meeting on the Mesa, held in California on 7-9 October, the key takeaway was that much more problem-solving is needed to address outstanding challenges around reimbursement and to potentially get more value-based agreements or other alternative payment models in play.

During a panel session with Marianne Hamilton Lopez (Duke-Margolis Center for Health Policy senior research director for biomedical innovation), Keren Shani (executive director for cell and gene therapy at the consulting firm Trinity Life Sciences) spoke at length about how additional work still needs to be done by a broad range of stakeholders to devise a way to pay for these new breakthroughs.

"What we see is that the innovation is there. The patients are excited. They're seeing a future ahead that they didn't think they could dream about, but there's a lot of questions on the implementation still," said Lopez. "It's not just insurers and drug companies that need to be involved in discussions, but state Medicaid representatives and small employers, who need to be assured of the value of the medicines and can't risk financial peril," she said.

Shani offered that despite the approval of several new gene therapies, there has been little in the way of development when it comes to broad reimbursement policy.

"We've seen primarily payers reimbursing through single case agreements and we're seeing a little bit more interest in uptake in case rate negotiations," Shani said, adding that implementation of value-based reimbursement agreements by payers has been minimal despite a lot of interest and discussion.

The big issue with gene therapy is that the treatments are believed to be durable and long-lasting with high up-front pricing. However, it is still not certain how long the gene therapies might actually last and how different patients might respond. Whether the benefits last a lifetime or a few years remains to be seen.

Value-based reimbursement deals, i.e., reimbursement linked to certain performance metrics, were initially thought of as straightforward options for payers to pay for the expensive therapies over an extended period of time, but few have actively adopted those payment models due to complexity in development, the tracking of patients, and because so few

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In Brief...

- **Walgreens Boots Alliance** reported fourth quarter and fiscal 2023 earnings, with Q4 sales of US$35.4 billion up 8.3% on a constant currency basis year-over-year (reflecting sales growth in the U.S. retail pharmacy and international segments as well as sales contribution from the U.S healthcare segment), and fiscal 2023 year-end sales up 4.8% to US$139.1 billion, up 5.6% on a constant currency basis over the previous year.
- **Zuellig Pharma** announced that it has entered into an agreement with global biopharma company **GSK** to help set up its first vaccine distribution hub in the Asia-Pacific region. The hub will be headquartered in Singapore, serving 13 markets in the region. Under the partnership, Zuellig Pharma will provide GSK with warehousing and distribution services in cold-chain management for its vaccines. The hub will also leverage Zuellig's expertise in blockchain technology for China, one of GSK's largest Asia-Pacific markets.
- **Merck's** *Keytruda* has won approval from the **U.S. Food and Drug Administration (FDA)** for treatment of early-stage non-small cell lung cancer both before and after surgery. Up until this point, it was only approved for treatment for either before *OR* after, not both. The approval could meaningfully increase *Keytruda's* sales outlook, even though the Merck drug is already one of the best-selling therapies in the world.
- **Pfizer** plans to cut US$3.5 billion in costs with plans to transition the sale of the oral antiviral *Paxlovid* in November. The company has already begun the transition to the commercial market of *Comirnaty*, Pfizer's mRNA COVID vaccine. Utillization of both products in the first half of the year was lower than expected. CEO *Albert Bourla*

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Sustainability Crucial for Pharma's Supply Chain Future

(Source: An article by Catherine Eckford for European Pharmaceutical Review)

Evidence of sustainability metrics is expected to be important for companies in the pharmaceutical industry in the next few years, according to sustainability results of the CPHI Annual Survey 2023.

According to sustainability results from CPHI's Annual Survey 2023, sustainability goals and metrics are now being implemented across the pharma supply chain, with the rate of adoption accelerating quickly.

Most contract development and manufacturing organizations (CDMOs) are expected to use them within the next two years. This is a significant shift from last year's survey when the expectation was that changes would take up to five

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Gene Therapies (cont.)

patients have thus far been treated with gene therapies. The situation becomes even more fractured when it comes to tracking capabilities at the state level by state-run Medicaid programs.

Both Lopez and Shani agreed that outcomes are often subjective and may not always be trackable within existing capabilities, and they are not designed to measure the durability and benefit of a gene therapy.

One payment model that could gain traction is a warranty model, which was presented by BioMarin Pharmaceutical for its new hemophilia A gene therapy *Roctavian*, which was approved by the U.S. Food and Drug Administration (FDA) in June of 2023 and is currently priced at US$2.9 million. BioMarin believes that the warranty model is more streamlined and straightforward in that the company will reimburse a payer a portion of the cost if a patient treated with *Roctavian* has to revert to prophylaxis treatment within a pre-specified number of years.

The U.S. Centers for Medicare and Medicaid Services is working on its own program to test new outcomes-based approaches for cell and gene therapies through the Center for Medicare and Medicaid Innovation (CMMI). The agency expects to implement the multi-state program in 2025, according to an update issued by the agency on October 11th of this year.

Sustainability (cont.)...

years, the data showed.

The new findings are being announced at this year's CPHI Barcelona (October 24th to 26th), with 93 percent of executives stating that visibility on supply chain partner's sustainability record' is either "extremely important" or "important".

Implementing ESG measures. "The rate of change is increasing rapidly and we expect this to be one of the biggest themes for manufacturing in 2024," stated Silvia Forroova, Director of Partnerships & Sustainability at Informa Markets. Sixty percent of executives forecast that innovators will require CDMOs to implement sustainability metrics (eg, full waste recycling, green power use, low PMI targets, and/or green chemistries) as a part of contracts with the next two years. A further 20 percent anticipate that CDMOs will be expected to show environmental, social, and governance (ESG) goals as part of any supplier deals. Only 20 percent believe that only "cost and capabilities" will remain an innovator's single factor in assigning new contract decisions.

"The 2023 CPHI event in Barcelona is our greenest to date, but we are also increasingly using the event as a platform to centralize debate, propagate best sustainability practices, and help our partners accelerate towards greener manufacturing methods," commented Forroova.

Sustainability in the global pharmaceutical industry. Earlier this year, CPHI also launched its first sustainability report. This evaluated the sustainability developments across supply chain partners and details global initiatives currently underway. The Barcelona event hosted a dedicated sustainability track on the 25th of October, as well as its first ever Sustainability Centre.

"The trend in the industry is very clear, and we are seeing all supply chain partners exploring more efficient manufacturing processes… which also often brings cost benefits, as a more efficient process is typically cheaper," Forroova added.

Further analysis of the findings released from CPHI Barcelona showed that only nine percent of the pharma companies stated they had 'no current green manufacturing activities'.

At forty percent, the most popular initiatives currently in use across the industry are: 'waste and water reduction programmes'. Other initiatives commonly being harnessed by the industry were: process improvement working groups – including green chemistries, metal catalysis, continuous processing (33 percent); manufacturing equipment optimisation, eg, intelligent energy programmes and machine learning for process efficiency (32 percent), and decarbonizing company supply chains (29 percent).

In Brief (cont.)

announced that the company was preparing cost-cutting reduction measures during the Q2 sales and earnings call in August due to the anticipated lower utilization rates.

- The **European Medicines Agency** has been notified by relevant national competent authorities that pre-filled pens falsely labelled as the diabetes medicine *Ozempic (semaglutide)* have been identified at wholesalers in the EU and the U.K. The pens, with labels in German, originated from wholesalers in Austria and Germany, and have batch numbers, 2D bar codes and unique serial numbers from genuine *Ozempic* packs. When the packs of the falsified *Ozempic* were scanned, the serial numbers were shown to be inactive, thereby alerting operators to a potential falsification. There are also differences in the appearance between the falsified and original pens. There is no indication that any falsified pens have been dispensed to patients from legal pharmacies.
- French manufacturer **Sanofi** announced that it was seeing an "unprecedented level" of demand for its respiratory syncytial virus (RSV) antibody therapy it co-developed with **AstraZeneca**. The therapy, branded *Beyfortus*, was approved in July for the prevention of RSV in infants and toddlers. Prior to the approval of Beyfo*rtus*, Swedish Orphan Biovitrum's treatment, *Synagis*, was the only approved preventive therapy in the United States for high-risk infants. Sanofi said it is working with AstraZeneca to identify further solutions to meeting the surging demand for the drug therapy.
- Japan's largest R&D hub for bioventures and pharma startups, **Shonan Health Innovation Park (Shonan iPark)** is in active talks with Korean bioventures and government groups over the formation of a new ecosystem to incubate new companies. The two organizations plan to sign a memorandum of understanding in November. The plan is to create synergy between fast-growing Korean startups, mainly in the cell and gene therapy area, and large Japanese pharma firms experienced in international markets to help globalize such assets.

(Sources: Company Press Releases, Drug Store News, FiercePharma, Reuters and Scrip Intelligence)