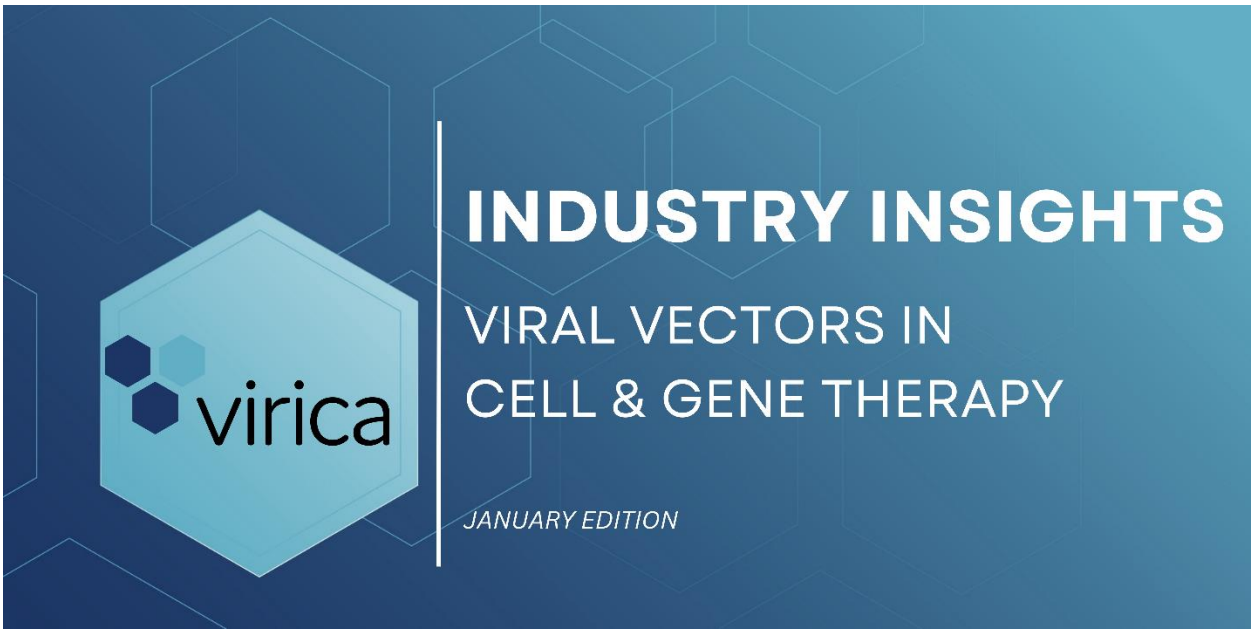

Report Overview:

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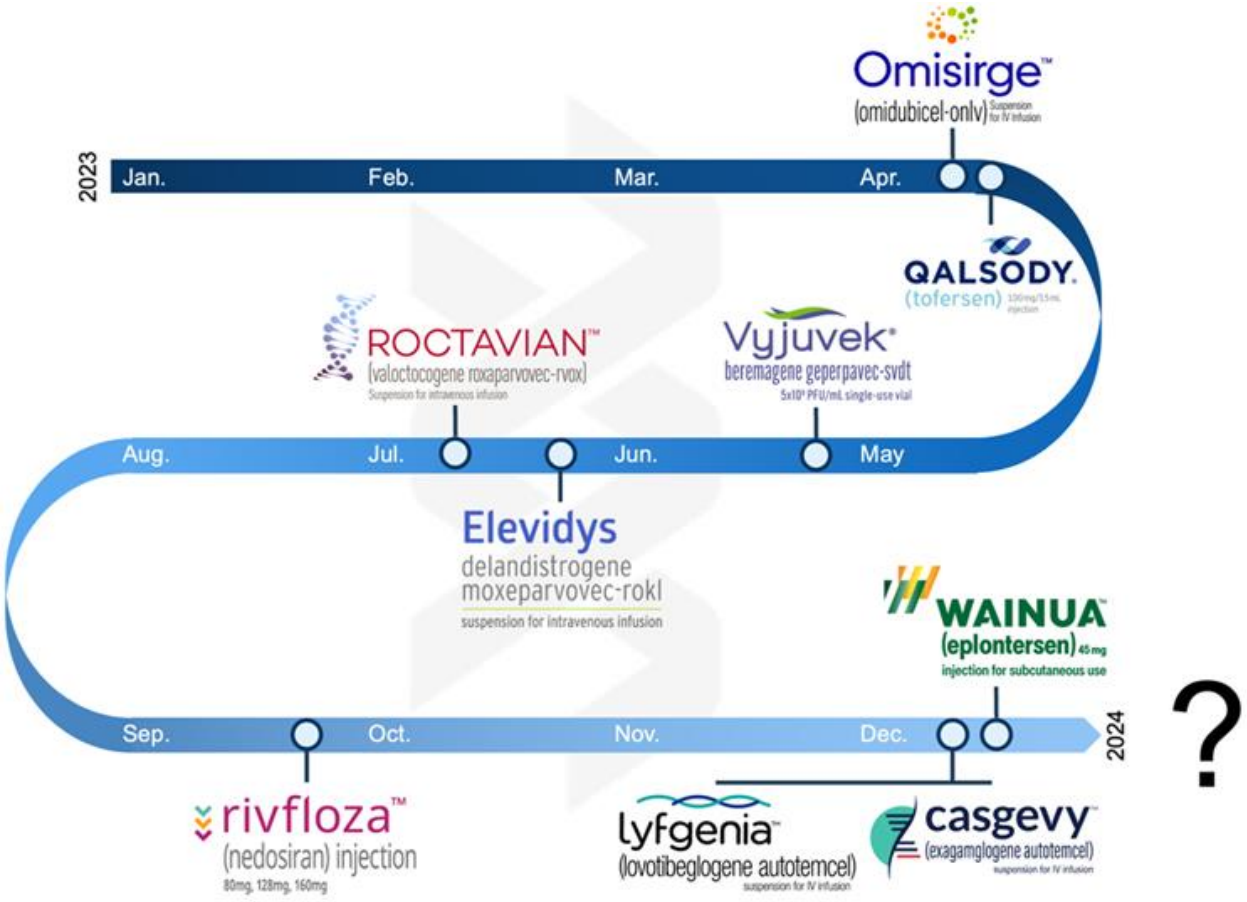
Virica's Insights

The cell & gene therapy industry had a breakthrough year in for approvals in 2023, with seven FDA approvals in the US and one in the EU. According to the Alliance of Regenerative Medicine (ARM), breakthroughs are becoming the norm as growing approvals reflect the innovation in the cell & gene therapy industry and the confidence of regulatory authorities in these life-saving therapies. It represents a milestone year for CRISPR-based gene therapy, with the UK's approval of Casgevy, the first approved CRISPR-based therapy to tackle sickle disease alongside Bluebird Bio's lentivirus-based gene therapy, Lyfgenia. The industry anticipates continued momentum with U.S. approval of an allogenic T-cell therapy and predictions for the first-ever adoptive cell therapy for solid tumours alongside five gene therapies in 2024.

Despite this promising news, cell and gene therapy is at a critical juncture due to manufacturing bottlenecks and exorbitant manufacturing costs. Casgevy's $2.2 million and Lyfgenia's $ 3.1 million cost per patient highlight the cost pressures and severely undermine the accessibility of innovative life-saving therapies. The manufacturing constraints signal a potential shift in the 2024 industry focus to manufacturability and scalability to ensure groundbreaking therapies reach a broader patient base. ARM's State of the Industry briefing captures the manufacturing challenges, indicating manufacturing automation and standardization as key strategies. Cell and gene therapy manufacturing is complex and needs solutions to tackle the challenge from multiple angles using complementary and synergistic technologies. The recent JPM conference echoed the industry's innovation, resilience, and transformative strides in the dynamic field of cell and gene therapy.



Top Headlines:

Health Canada green lights Pfizer Canada's Gene Therapy for Hemophilia B.

Pfizer Canada ULC announced Health Canada's approval of BEQVEZ, a novel AAV vector-based gene therapy for the treatment of adults with moderate to severe Hemophilia B who are negative for neutralizing antibodies to variant AAV serotype Rh74. The approval is based on the open label, single arm Phase 3 BENEGENE-2 study evaluating the safety and efficacy for BEQVEZ in adult male participants with moderate to severe Hemophilia B.

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Significant Milestone for Sickle Cell Disease: FDA Approves Two Gene Therapies to Treat Patient with Sickle Cell Disease.

The FDA approved two milestone gene therapies, Vertex Pharmaceutical's CRISPR-based gene editing therapy Casgevy and Bluebird Bio's lentivirus-based gene therapy, Lyfgenia, to treat sickle cell disease (SCD). Casgevy is the first approved therapy utilizing CRISPR-Cas9 technology, where patients' hematopoietic stem cells are modified to increase production of fetal hemoglobin (HbF), preventing sickling of red

blood cells. Lyfgenia on the other hand uses a lentiviral vector for genetically introducing HbAT87Q, a gene-therapy derived hemoglobin that functions similar to healthy adult hemoglobin. Both studies demonstrated strong safety and efficacy results, receiving Priority Review, Orphan Drug, Fast Track, and Regenerative Medicine Advanced Therapy designations.

Read Full Article

FDA, Industry Officials Predict 2024 will be a 'Breakthrough Year' for Gene Therapy Approvals.

The gene therapy landscape is experiencing a significant breakthrough, with an optimistic outlook for up to 17 new approvals in 2024, FDA preparations through the new pilot STRAT program and new Office of Therapeutic Products, growing collaborations, efficiency considerations and transformative therapies raising both hopes and questions about accessibility and affordability.

Read Full Article

News at a Glance

- BioCentriq successfully closes $29.2 million Series A to bolster cell therapy manufacturing by translating, optimizing, and scaling processes for GMP manufacture.

- Bayer inaugurated its $250 million Cell Therapy Launch Facility in Berkley, focusing on transformative global scale cell and gene therapies and supporting commercial manufacturing demands.

- Voyager Therapeutics announces strategic collaboration with Novartis to advance potential gene therapies for Huntington's Disease and Spinal Muscular Atrophy


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