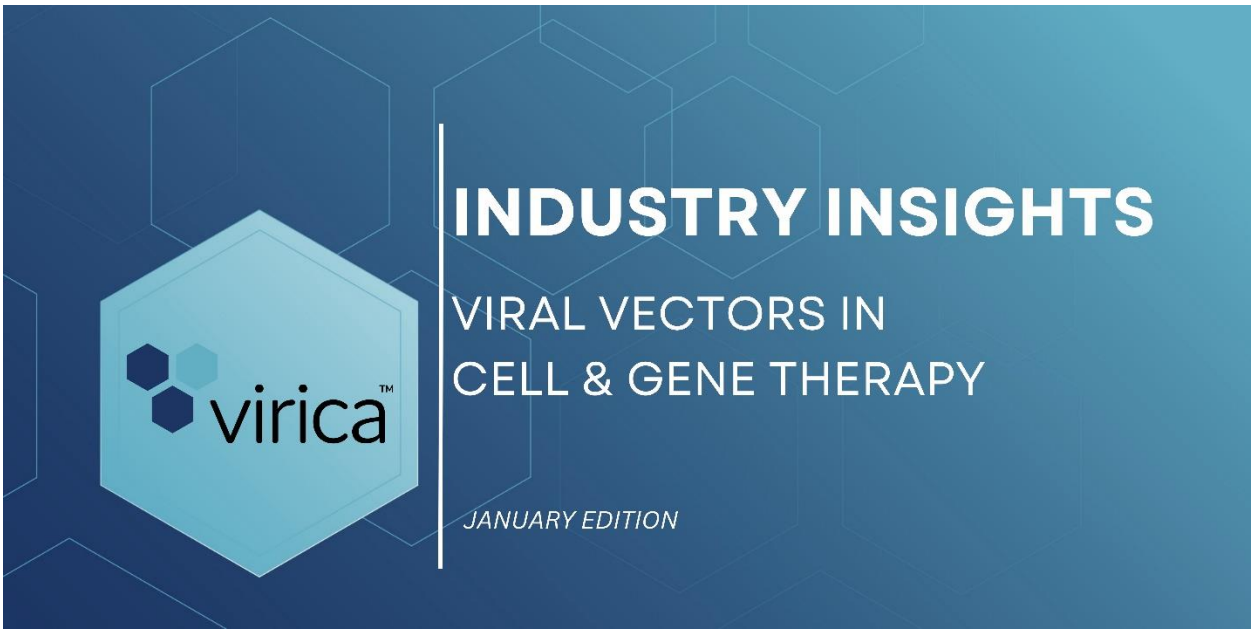

Report Overview:

- Virica's Insights
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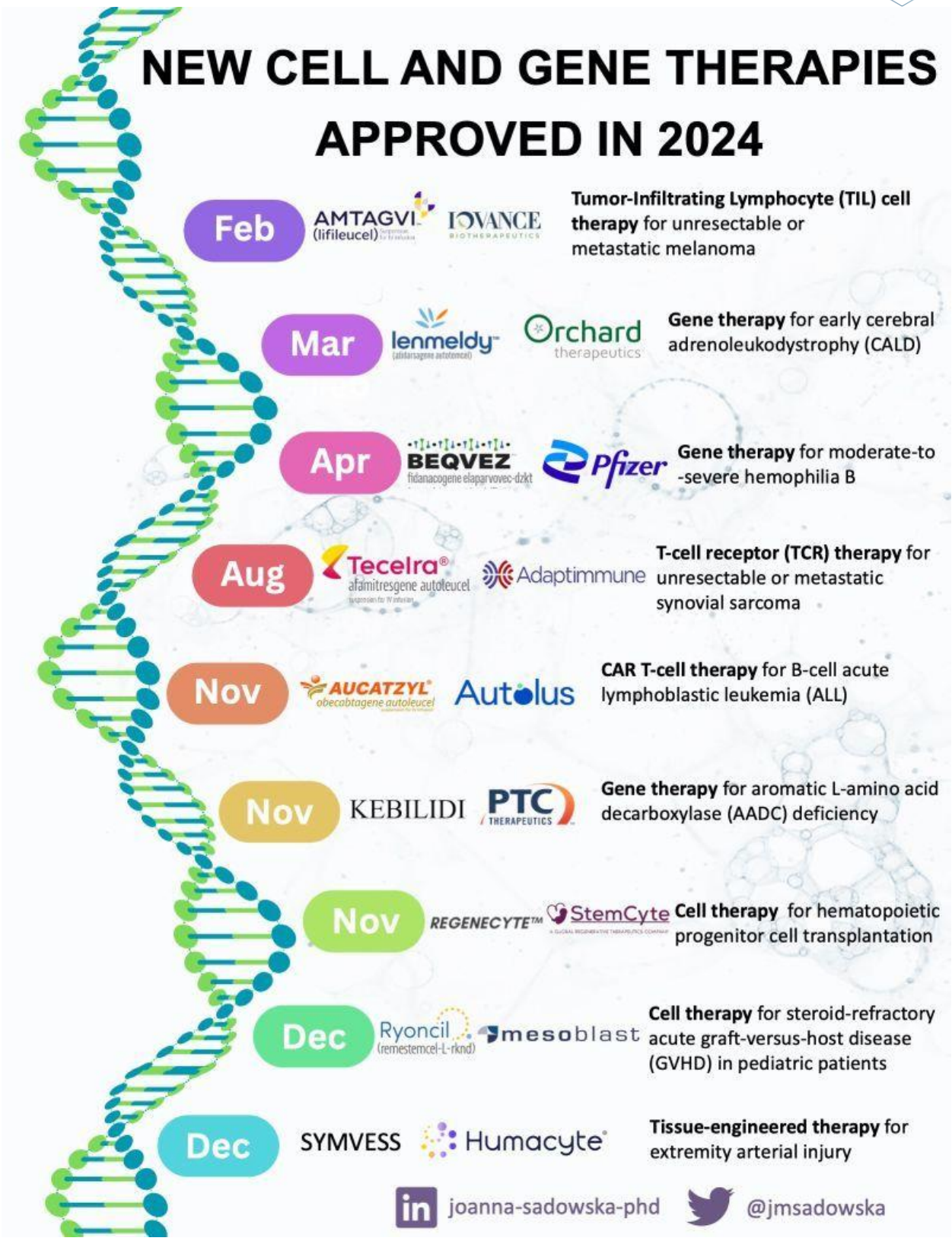
Virica's Insights

Cell and gene therapy (CGT) continues to build on the momentum with nine new FDA approvals crossing the regulatory finish line in 2024. Notable approvals include the first mesenchymal stromal cell therapy for GVHD and PTC Therapeutic's Kebilidi as the first gene therapy delivered directly to the brain to treat AADC deficiency. 2024 was a banner year for cell therapy with first-in-class drugs such as Iovance's Amtagvi and Adaptimmunes Tecelra standing out as significant advancements targeting solid tumours. The cell therapy field has also steadily diversified from an oncology focus with autoimmune diseases emerging as a promising new frontier. Over 20 CAR T programs are now in autoimmune trials, with early data showing potential for drug-free remission in diseases like lupus. The steady success of CGTs has led to growing confidence in the sector by investors with an uptick in 2024 investment surpassing 2023, a major bounce back since the post-COVID downturn.



Graphic credits Joanna Sadowska

Despite the many successes, CGT production costs continue to be high and manufacturing remains the sector's Achilles heel due to a lack of standardization, scalability issues, and overcapacity in CDMOs. These challenges make purpose-built automation, decentralized manufacturing, real-time analytics, and AI/ML-based approaches a focal point for 2025 and critical to addressing inefficiencies and enabling scalability. AI and machine learning have revolutionized genomic data analysis and now drive advancements in drug discovery and development, though their full potential in clinical validation and manufacturing remains untapped. Innovations such as stable cell lines for AAV manufacturing increase consistency, scalability and cost-effectiveness. The CGT sector is also facing a looming patent cliff making agile innovation necessary to remain competitive. Despite continued success, the CGT sector remains at a "critical juncture," requiring collaboration, innovation, and investment to overcome manufacturing and scalability challenges and unlock the potential for widespread patient access.

Top Headlines:

Worlds-first direct-to-brain gene therapy gets FDA nod

The FDA has granted accelerated approval to PTC Therapeutics' gene therapy Kebilidi, the first therapy delivered directly to the brain, for treating ultrarare aromatic L-amino

acid decarboxylase (AADC) deficiency. This severe genetic disease causes developmental delays, disability, and early death. Kebilidi uses AAV-based gene therapy to deliver a functional DDC gene to the brain. This approval marks a significant advancement in gene therapy, particularly for neurologic diseases requiring direct brain delivery.

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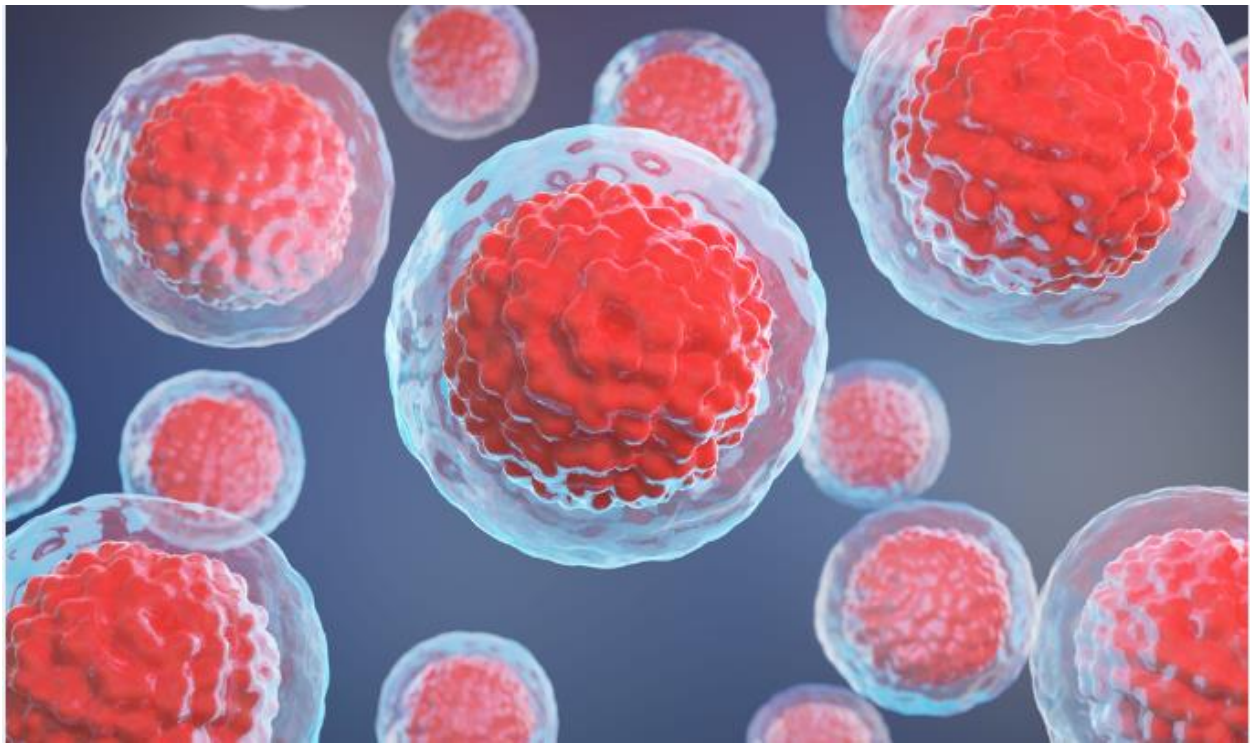

First-in-call Mesenchymal Stromal Cell Therapy wins FDA approval

Ryoncil (remestemcel-L) is the first mesenchymal stromal cell therapy approved by the FDA in the U.S. for steroid-refractory acute graft-versus-host disease (GVHD). Derived from unrelated donor bone marrow, Ryoncil reduces inflammation, achieving a 70% response rate in pediatric Phase III trials, with 30% showing complete response. This therapy offers a vital new option for children and families with no alternative treatments for this severe condition.

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Gene therapy enables hearing in five deaf children

A gene therapy trial treated six children with a mutation in the OTOF gene, crucial for hearing, resulting in five showing improved speech recognition. The therapy overcame the challenge of the gene's large size by splitting it into two parts and using separate viral vectors to insert them into cochlear cells, enabling the production of the missing protein. This innovative approach, which may extend to other genetic deafness treatments, demonstrated significant success in restoring hearing. Published in *The Lancet*, the study's success could lead to regulatory approval in 3 to 5 years, offering hope for children with congenital deafness, a condition lacking approved drug treatments.

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News at a Glance

- Novartis acquires Kate Therapeutics for $1.1 billion expanding gene therapy pipeline, focusing on neuromuscular disorders and enhancing gene delivery capabilities.

- Canadian CDMO OmniaBio debuts CGT manufacturing facility with an initial focus on cellular immunotherapies and iPSC-based therapies.

- CGT sector braces for looming patent cliff including top-selling drugs like Merck's Keytruda. Navigating this coming cliff will require a combination of innovation and strategy.

- 2024 sees breakthroughs and investment in CGT but manufacturing hurdles persist.

- Recent J.P. Morgan conference reveals major pipeline updates- Pfizer's obesity focus, Sarepta's success with Elevidys, BioNTech's mRNA-based cancer immunotherapy push and Moderna's vaccine outlook shift.


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