Report Overview:

- Virica's Insights
- Top Headlines
- News at a Glance

Virica's Insights

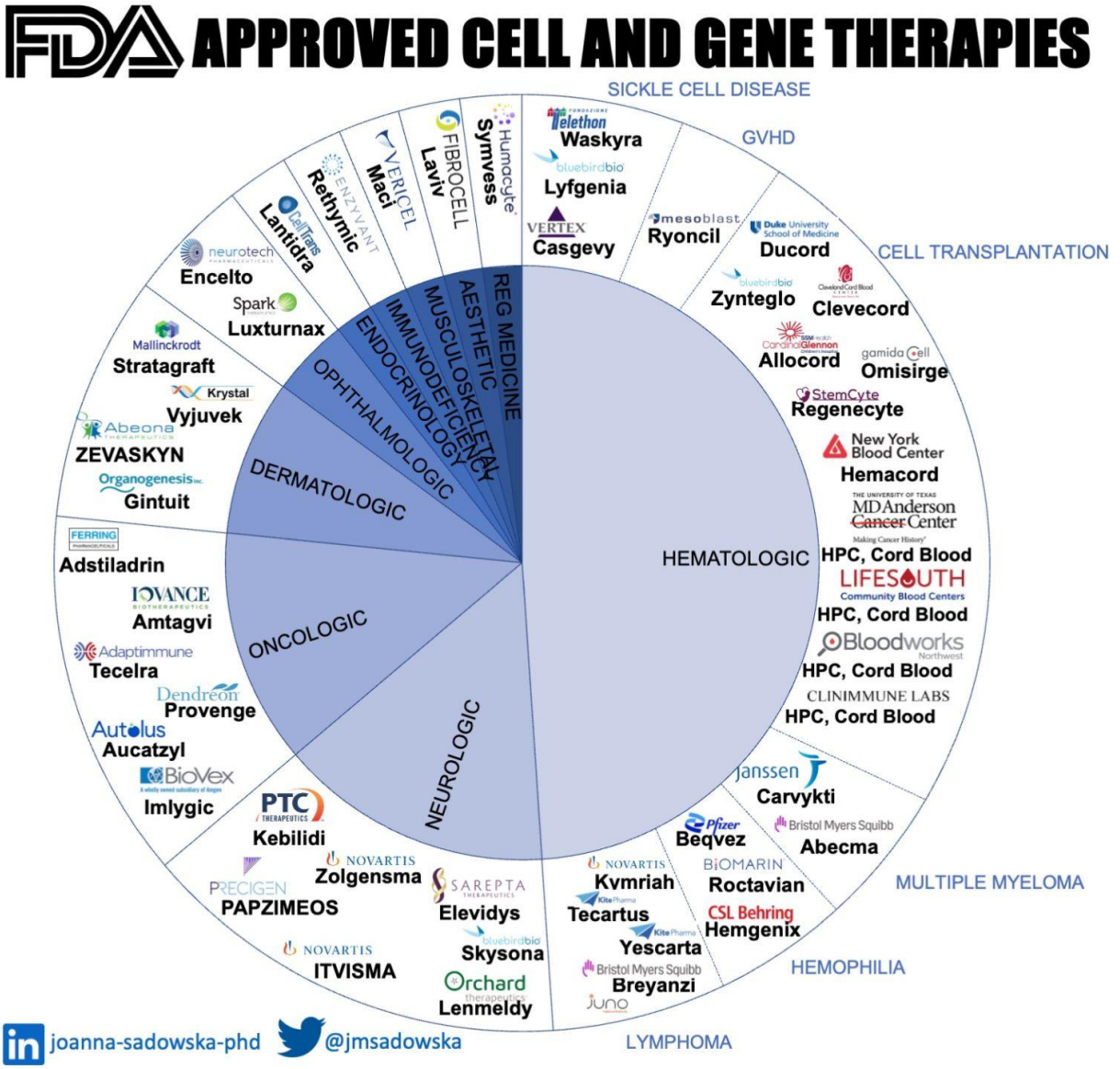
Regulatory Momentum Meets Commercial Reality

Cell and gene therapy reached a critical inflection point in 2025. The FDA delivered on its promise of regulatory momentum, approving first-in-class therapies like Encelto for macular telangiectasia and Waskyra for Wiskott-Aldrich syndrome, while granting breakthrough designation to AMT-130 as the first potential disease-modifying treatment for Huntington's disease. But 2025 also revealed that scientific and regulatory success no longer guarantee commercial viability. Pfizer's withdrawal of Beqvez, an FDA-approved hemophilia B gene therapy priced at $3.5 million, came less than a year after approval, with zero patients treated commercially.

Manufacturing Takes Center Stage

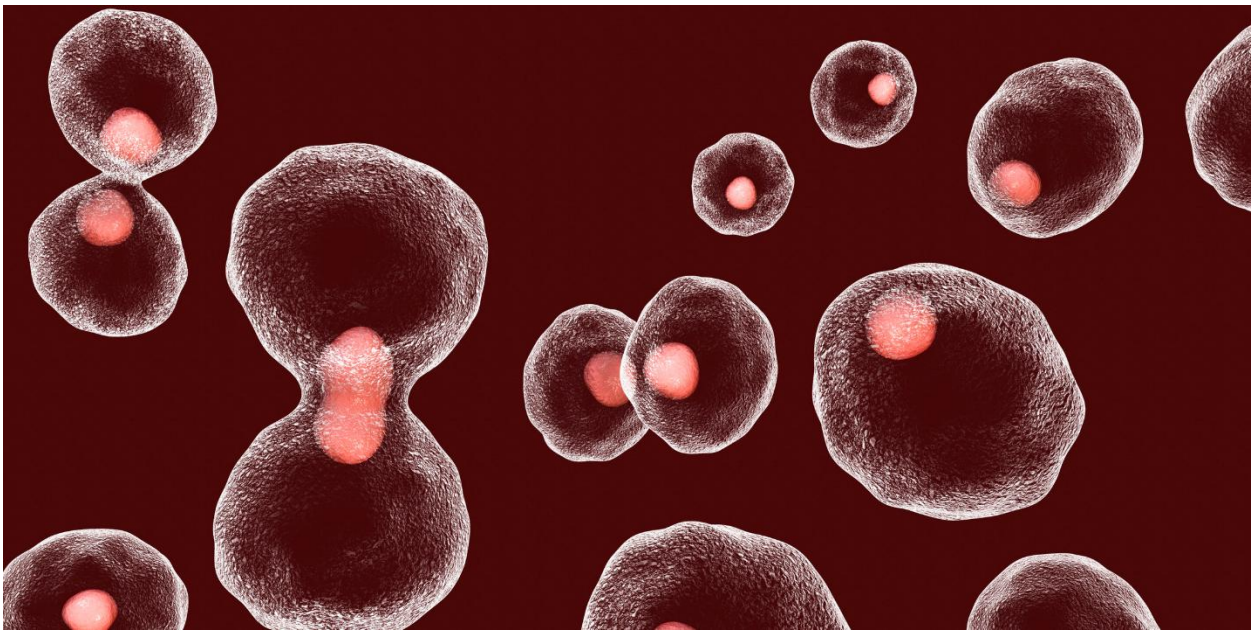
The industry's response signals a fundamental shift. FDA Commissioner Marty Makary introduced "common sense reforms," carving out manufacturing exemptions that recognize the unique demands of individualized, small-batch biologics. This regulatory flexibility arrives at a pivotal moment. CAR-T therapies are moving earlier in treatment timelines and expanding

beyond oncology into autoimmune diseases, a shift that could multiply addressable patient populations exponentially. Yet manual processes still account for 50% of manufacturing costs, an unsustainable bottleneck as demand scales. As 2026 unfolds, the rare disease space is accelerating with gene therapies for Hunter syndrome and Danon disease approaching regulatory decisions, while Kyverna races toward the first autoimmune CAR-T approval in neuroinflammation. The stage is set for a year where manufacturing capability, not just scientific innovation, will determine which therapies reach patients.



Source: https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products

Top Headlines:



FDA Approves Waskyra, Setting a New Benchmark for Ex Vivo Gene Therapies

FDA approved Waskyra, the first gene therapy for Wiskott-Aldrich syndrome (WAS), giving patients a new option when no HLA-matched related stem cell donor is available. The one-time, ex vivo approach uses a patient's own blood stem cells, genetically modified with functional WAS gene copies and reinfused after chemotherapy. In studies and an expanded access program, severe infections fell 93%, and moderate-to-severe bleeding events dropped 60% versus the year before treatment, with most patients not reporting such bleeding four years later.

Why it matters: The FDA signalled more regulatory flexibility, accepting data from multiple sources and allowing some manufacturing/quality (CMC) data to be leveraged from similar approved products. This could streamline future ex vivo CGT approvals and cut duplicative CMC work.

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Itvisma Gains FDA Approval, Bringing One-Time Gene Therapy to Treat SMA

Novartis won FDA approval for Itvisma, a new version of Zolgensma that expands SMA gene therapy to older patients with a confirmed SMN1 mutation. It uses the same AAV-delivered functional SMN gene as Zolgensma but is given intrathecally rather than IV, enabling dosing in larger-weight patients with a more concentrated formulation. Novartis priced Itvisma at

$2.59M and said it is 35%–46% less than chronic treatments over 10 years, with a support program where eligible patients may pay as little as $0.

Why it matters: The intrathecal route demonstrates how delivery innovation can expand patient eligibility for existing AAV platforms, a critical pathway as first-generation IV gene therapies face weight and immune limitations.

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FDA's AMT Designation Signals a New Route to Commercial-Scale CGT Supply

Approval isn't the finish line in cell and gene therapy, many companies then struggle to reliably and profitably manufacture at scale, creating supply delays and longer waitlists. The FDA's Advanced Manufacturing Technologies (AMT) designation, finalized at the end of last year, is pitched as a lifeline by giving companies a venue to align plans and documentation expectations with regulators before post-approval manufacturing changes. Cellares, Cellino, and Oribiotech have already secured the first AMT awards, as the industry grapples with commercial viability, with 8 of 28 EMA-approved CGTs now unavailable and cautionary examples like Bluebird Bio and Dendreon.

Why it matters: AMT provides a clear regulatory path for automation, closed systems, and analytics upgrades, reducing the risk of manufacturing changes that can delay launches and supporting proactive scale-up.

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News at a Glance

Regulatory & Approvals

- Six FDA decisions loom in Q1 2026, including REGENXBIO's Hunter syndrome gene therapy (Feb 8) and Rocket's LAD-I gene therapy (March 28).
- FDA signals greater CMC flexibility for cell & gene therapies, allowing phased validation, flexible specs, and scaled manufacturing changes to speed approvals.

Clinical & Translational Breakthroughs

- Kyverna's CAR-T achieves "unprecedented" stiff person syndrome results with 46% mobility improvement and 100% drug-free at 24 weeks; H1 2026 BLA filing positions first autoimmune neuroinflammation approval.
- Cartesian's mRNA CAR-T wins Nature Medicine spotlight as trial "shaping medicine in 2026" with phase 3 pivotal trial targeting H1 2026 launch.

Market & Business

- ASGCT and Orphan Therapeutics launch CGTxchange, an AI-powered clearinghouse matching "deprioritized" therapies with investors and nonprofits, rescuing promising assets abandoned for commercial reasons.
- Orca Bio secures $250 million to expand East Coast manufacturing capacity and scale its allogeneic T-cell therapy that significantly reduces graft-versus-host disease.