

Powering the pipeline: The technologies and deals rewriting biotech's playbook

Artificial intelligence, innovation in cell and gene therapies, and evolving investment models. Never has the biotechnology landscape experienced so many breakthroughs that have such an influential stake in shaping the future of medicine and patient care.
These key themes are sure to dominate future discussions, none more so than at this year's Bio USA convention in Boston, MA , where the stage is set for industry leaders, researchers, investors, and policymakers to unveil groundbreaking research converge, forge partnerships, and collectively shape the future trajectory of biotechnology.

AI's role in biotech R&D

The conversations begin with Artificial Intelligence (AI), a technology with transformative capabilities across industries.
From target identification to clinical trial optimization, AI is arguably playing its most influential role in biotech research and development, accelerating timelines, reducing costs, and significantly increasing the probability of success.

AI's power is evident in identifying novel drug targets, traditionally a painstaking process. The technology's ability to mine vast amounts of biological data to uncover potential targets that might otherwise go unnoticed is revolutionising this aspect of drug discovery.
Analysis appears to back this up. US based consulting company, Coherent Solutions **highlights** that, *"Such an approach allows researchers to zero in on the most promising opportunities faster and accelerate the drug development process."*
Besides identification, AI is rewriting the process of evaluating drug target interactions and analysing disease mechanisms with unparalleled precision. Coherent Solutions adds that this is leading to *"more effective drugs, ensuring that compounds are not only innovative but also highly specific to the target."*

But where else is AI making its presence felt? The time consuming and error prone job of recruiting patients for clinical trials may be the answer. Machine learning models have been developed that can analyse huge numbers of Electronic Health Records (EHRs), identifying eligible participants quickly and with high accuracy. Indeed, AI tools like TrialGPT are already showing promise in matching patients to clinical trials, with early findings suggesting faster recruitment and reduced administrative burden.
AI's capabilities also extend towards ensuring a more dynamic and patient focused clinical trial design. This is achieved by harnessing the AI algorithm to analyse real world data (RWD) and identifying the patient subgroups more likely to respond positively to treatments.
This enables real time adjustments based on patient responses for better outcomes. The financial proposition is compelling. Coherent Solutions predicts that with AI's integration *"pharma companies can expect up to $25 billion (C22.06bn) in savings in clinical development alone."*
Perhaps with one eye on the future, generative AI has caused a few waves among industry observers, who point out its ability to generate entirely new data, such as images, sentences, audios, videos, and novel chemical molecules.
Keen to prove its mettle in drug discovery, Roche recently revealed details of its "lab in a loop" mechanism, where lab and clinical data are used to train AI models and algorithms designed by the pharma company's researchers.
From there, the trained models are used to make predictions on drug targets, therapeutic molecules. These predictions are further investigated in the lab, generating new data to further tweak existing models. This iterative process streamlines the traditional trial and error approach, leading to faster and more effective therapies.

"To take advantage of these new approaches and to apply them rapidly, we need to bring together expertise from different disciplines - by doing so we have a tremendous opportunity to hopefully bring medicines to patients faster than we do today," commented **John Marioni**, Senior Vice President and Head of Computational Sciences at Genentech, a member of the Roche Group.

Advancing cell and gene therapy

As AI advances drug discovery and development, adjacent fields are also benefiting. The rapidly evolving landscape of cell and gene therapy is a prime example, where precision tools and data driven insights are driving breakthroughs once thought impossible.
In the US, Neurotech's Encelto, an intraocular implant of encapsulated engineered human cells, was recently approved for the treatment of macular telangiectasia type 2 (MacTel), as reported by the American Society of Gene & Cell Therapy (ASGCT) in their Q1 2025 report.
Additionally, the approval of Alnylam's siRNA therapy, Qfitlia, for the treatment of haemophilia A and B marked a pivotal moment for RNA interference (RNAi) based therapeutics, highlighting the growing clinical relevance of gene silencing strategies in managing complex, inherited disorders.
In China, Platinum Life's Ruiboshen received approval for the country's first mesenchymal stem cell therapy for steroid refractory acute graft versus host disease.
The American Society of Gene and Cell Therapy (ASGCT) **report** a continuing trend of increased clinical trial numbers for non genetically modified cell therapies in non oncology indications up to 74% in Q1 2025 compared to 58% from the previous quarter.
Conversely, gene therapy trials are shifting towards oncology. Of the 79 trials initiated in Q1, 57% were for oncology indications, the highest oncology trial proportion for the past year.
But approvals aside, the field continues to push boundaries, especially in gene editing, where the CRISPR Cas9, remains a revolutionary tool. The UK MHRA's approval of Vertex Pharmaceuticals and CRISPR Therapeutics' exagamglogene autotemcel (CASGEVY) in November 2023 marked a pivotal moment, being the first marketing authorization granted to a CRISPR gene editing therapy.

The success of mRNA technology, initially driven by COVID 19, is proving useful beyond infectious diseases by proving its worth in cancer immunotherapy, genetic disorders, and regenerative medicine.

Companies like Moderna and BioNTech, global leaders in mRNA innovation, are expanding their pipelines to include vaccines for RSV, CMV, seasonal influenza, and personalized cancer treatments, reinforced by partnerships.

Navigating biotech's investment landscape

This wave of innovation, from mRNA to gene and cell therapies, is naturally drawing the attention from investors seeking to capitalize on biotech's next breakthroughs. As scientific milestones accumulate, the investment landscape too must evolve.

Investment in biotech remains strong but more selective, with late stage assets (phase 2 and beyond) receiving larger deals. J.P. Morgan and

Pitchbook data both indicate a shift toward fewer, bigger investments, with phase 2 funding rising notably through 2024.

Macroeconomic factors like high interest rates have influenced this trend, affecting venture capital groups' ability to raise new funds. Analysts note increased selectivity from investors, who are currently leaning towards late-stage, derisked firms with defined commercialization paths.

Despite economic challenges, investment continues in key areas. Excedr's into 2025's trends in biotech venture capital funding highlights "oncology, gene therapy, rare diseases, and AI-driven drug development" as key areas securing capital.

VCs are now prioritising the potential for curative treatments in gene therapy and gene editing, particularly for rare and inherited disorders. In a similar vein, the efficiency and risk reduction AI promises in drug discovery represent a highly attractive investment proposition.

Strategic partnerships are reshaping funding, as pharma companies team up with startups to share resources. In 2024, Eli Lilly partnered with HAYA Therapeutics, Genetic Leap, and insitro to target metabolic diseases and accelerate AI-driven genetic medicine. Novartis joined forces with Generate for protein therapeutics and Monte Rosa Therapeutics for molecular glue degraders. These alliances reflect a growing industry trend that unites innovation with the scale and resources of established pharmaceutical players.

Key themes for Bio USA Convention

Bio International Convention

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Bio USA will spotlight trends shaping biotech's future. Key themes include:

AI & Digital Health: Planned sessions include its role in target identification, lead optimization, and predictive modelling for clinical trial success. Discussions will also address the ethical and regulatory challenges associated with AI-generated inventions and the need for robust frameworks to ensure data privacy and intellectual property.

Advancing Cell & Gene Therapies: Discussions include next-gen CAR-T therapies, CRISPR, and the applications of mRNA technology beyond vaccines into oncology and personalized medicine. The convention will also address the challenges and opportunities in scaling manufacturing, improving delivery mechanisms, and ensuring global access to these life-saving treatments.

Investment & Partnership Strategies: Sessions on funding trends, venture capital strategies, and alternative financing models will feature, with an emphasis on late-stage assets and de-risked opportunities. The importance of collaborations between big pharma and biotech, as well as academia will be highlighted as will the partnerships of 2024 that underline this growing trend.

Precision Medicine & Personalized Healthcare: Discussions at Bio USA convention will include tailoring treatments to an individual's genetics, disease profile, and real-time data. The integration of wearables, like glucose-monitoring represents 'Small Data' personalized diagnostics and analytics, will be explored as key enabling technologies

Biomanufacturing Innovation & Supply Chain Resilience: Innovations in biomanufacturing, including the adoption of data science, digital twin technologies, and increased automation in analytical development, will be key discussion points. Ensuring a robust and global supply chain for these complex therapies will also be a critical area of focus.

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