

# The Changing Landscape for Cell and Gene Therapy

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Even with the massive disruption of the health care sector caused by COVID-19, the pace of activity in cell and gene therapies has not slowed—if anything, it has accelerated in the past year. Our analysis shows that, in the decade leading up to 2020, six major pharma companies alone made more than 50 cell and gene therapy alliances and licensing deals worth over \$16 billion and spent approximately \$38 billion on acquisitions. In 2020, total financing in regenerative medicine doubled to almost \$20 billion from just under \$10 billion the year before, according to the Alliance for Regenerative Medicine.

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Cell and gene therapies have made impressive clinical progress but continue to face big hurdles.

These novel therapies have clearly seized the attention of Big Pharma. But even as the agents make impressive clinical progress—the alliance counts 1,220 clinical trials underway last year—cell and gene therapies continue to face big scientific, **technological**, and **business-related** hurdles. Our recent client work and conversations with leaders in the field indicate that pharma companies looking to make or expand their bets should consider five factors as they move forward: innovations from COVID-19, maturing standards and processes, the challenges of small treatment markets, consolidation and diversification in the industry, and pricing and regulatory pressure.

## **INNOVATIONS FROM COVID-19**

On the plus side, while the pandemic disrupted pharma R&D, it accelerated trends in three key areas: clinical trials, cold-chain capabilities, and manufacturing capacity. In clinical trials, COVID-19 sped the adoption of digitally enabled processes and innovative trial design. Cell and gene therapy companies can leverage new practices such as remote monitoring for long-term follow-up and develop master protocols to accelerate trials. In addition, the mRNA vaccines developed by Pfizer and Moderna have led to improved cold-chain manufacturing, distribution, and storage capabilities throughout the sector, providing a much stronger foundation for cell and gene therapy products that have similar requirements.

## **MATURING STANDARDS AND PROCESSES**

Regulatory standards have tightened. In 2020, six cell and gene therapy programs were forced to delay their development timelines after the FDA asked for more information about their production processes. Requests for additional data delayed one cell therapy company's program by four to six months.

In 2021, it will be essential to comprehensively characterize new products' critical quality attributes and show that the manufacturer can scale up production without compromising quality. Companies will need to invest in scalable platforms and reduce variability and testing timelines. This will increase demand for talent with practical expertise.

As the number of treatments grows, safety concerns rise. Over the past year, several trials were put on hold for safety reasons. Companies must advance the exploration of cell and gene therapies cautiously, evaluating the potential of higher doses and efficacy against the adverse effects while also investigating newer delivery vehicles.

## **THE CHALLENGES OF PROVING POTENTIAL**

Cell and gene therapies have proved their potential only in rare diseases with well-defined genomic targets, high unmet need, and small numbers of patients. While these treatments are critical to people with serious conditions, the potential of cell and gene therapies must be expanded to apply to diseases in larger indications (such as neurological disorders, diabetes, and cardiovascular illness) in order to maintain funding, resources, and attention. Success will depend on demonstrating effectiveness against traditional modalities and standards of care, lowering costs, developing superior delivery methods, and scaling up production.

It's also a fact that the FDA has shown a clear intent to reduce regulatory barriers in order to give companies an incentive for investing in treatments that serve only a handful of patients (such as those with Batten disease or ataxia-telangiectasia). The agency is working with the National Center for Advancing Translational Sciences and the Foundation for the NIH to create a revised regulatory framework. The new framework would enable companies pursuing such rare-disease treatments to

leverage existing product characterizations if the platform remains the same (akin to changing a lightbulb while keeping the lamp).

## **CONSOLIDATION AND DIVERSIFICATION**

On top of the record level of investment in 2020 (one analysis in late 2020 found that 16 of the 20 largest biopharma manufacturers have added cell and gene therapy products to their portfolios), a recent survey by BDO determined that 57% of life sciences companies plan to invest in cell therapies in 2021 and 47% plan to invest in gene therapies. We expect this level of interest will lead to consolidation of the value chain and diversification of the bets for major players.

As large pharma companies become more active, they are looking to identify which parts of the value chain are missing from their platform, since they want to ensure that they have a hand in multiple technologies. A few key areas are identifying targets (coding and noncoding sequences, cell types), editing systems (DNA, RNA, protein), manufacturing cell lines, and developing delivery systems (nonviral, tissue targeting, cell type targeting, overcoming immunogenicity, and leveraging artificial intelligence and machine learning). Pharma companies are placing bets on multiple different platforms and technologies (such as cell and vector types) because no one knows where the winning therapies will come from.

## **PRICING AND REGULATORY PRESSURE**

Cell and gene therapies scored some big wins in the past year. But even with the recent approvals and innovations in access, 2020 was a year of significant regulatory challenges—particularly in chemistry, manufacturing, and controls—and a continued focus on functional outcomes and durability evidence. It's clear that the expectations regarding durability, consistency, and comparability are changing; this highlights a critical need for an early focus on clinical, medical, and data strategies that prioritize functional outcomes and durability measurements. (Durability becomes increasingly important as the therapeutic applications extend beyond rare-disease populations to indications with more competing products or an effective

standard of care.) These factors also emphasize the importance of a clear value proposition to payers and providers.

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In addition, with the Democrats in control of the Senate, we can expect political calls in the US for significant changes to pricing. At the same time, while the EU has unveiled a pharmaceutical-strategy document that recognizes cell and gene therapies as “major milestones” of progress in health care, it has also stressed the need to improve the affordability and cost-effectiveness of medicines, as well as health systems’ sustainability. The practical challenges are evident in Germany, where Bluebird Bio is withdrawing its beta thalassemia treatment, Zynteglo, because of an inability to agree on pricing.

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The field of cell and gene therapy is advancing quickly, and 2021 promises further progress. But with progress comes new challenges. Biopharma companies large and small should approach the year ahead with a clear view of changing expectations and market conditions.

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