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Focus

Roland Berger

Pharma's big opportunity to ride the next wave | How to enter the cell and gene therapy market

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Pharma's big opportunity to ride the next wave / *How to enter the cell and gene therapy market*

Cell and gene therapies (CGT) are a new breed of therapies that have the potential to cure serious diseases rather than treat symptoms. Gene therapies (gene augmentation and editing) replace or repair DNA to help the body regain functions or prevent dysfunctions. Current targets include hemophilia, neurodegenerative diseases, muscular dystrophy, metabolic disorders and eye diseases. Cell therapies, on the other hand, use specially grown or adapted human cells to replace depleted tissue or perform therapeutic functions. Targets include burns, certain cancers and degenerative and inflammatory diseases.

Sales of CGT products are forecast to reach EUR 1.8 billion in 2021 and EUR 27.9 billion in 2026, with the US Food and Drug Administration expecting to license 10 CGT products a year from 2025. Five current therapies are projected to achieve annual sales in excess of USD 1 billion. This potential has seen several new CGT companies, including Fate Therapeutics and CRISPR Therapeutics, already achieve valuations in excess of EUR 7 billion.

Acquisition activity in the market is intense, competitive and involves huge sums. For example, Gilead bought Kite Pharma for USD 12 billion in 2017. The reasons are threefold: The segment exhibits substantial growth potential; CGT presents an opportunity to expand into new markets; and the potential of cures is very commercially attractive.

But market entry is complicated, with several considerations to bear in mind. We make recommendations in five priority areas:

Research and development: Secure full control and continuous development of the underlying technology platform

Business development and licensing: Establish a team to rapidly evaluate potential acquisitions and make informed decisions

Manufacturing: Ensure control, strive to own the entire end-to-end manufacturing process

Market access, marketing and sales: Interact early with payors and regulators to enable fast rollout, and be alert to competition

Organization, governance and culture: Strong commitment and play to win attitude required for success

Overall, we believe three considerations are key. CGT entrants should (1) aim for end-to-end value chain coverage, (2) target newly emerging technologies and (3) be cognizant of the many new therapeutic areas that are on the rise.

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Introduction

For centuries, pharmaceutical companies have treated diseases in the only way they knew how: give a patient the right medication, and it would help them overcome symptoms, make life more bearable and even keep them alive. But it rarely provided a cure.

The emerging field of cell and gene therapies changes that. Instead of drugs, CGT products harness or enhance the body's own living building blocks to treat and potentially cure diseases. From cancer to hemophilia and diabetes, CGT products could be designed to attack tumors, repair genetic defects and regenerate degenerated tissue.

Although still in its infancy, the promise of cell and gene therapies is immense – not just for patients, but also the pharmaceuticals industry. Global CGT sales are expected to skyrocket from EUR 1.1 billion in 2020 to EUR 27.9 billion in 2026. From 2025 onwards, the US Food and Drug Administration estimates that it will be approving 10 cell and gene therapies a year.

The potential of this new breed of therapies has fueled a spate of multi-billion-dollar acquisitions in the sector recently, with several large pharma companies entering the market. The number of deals continues to increase. Yet due to the differences between classical pharmacotherapies and CGT, market entry is not straightforward.

In this report, we look at cell and gene therapies in detail, outlining how they work and which diseases they can potentially treat. We also assess the CGT market, the factors that make it attractive to pharmaceutical companies and why such big figures are in play. Finally, we detail key considerations and recommendations for companies looking to move into the CGT space. We conclude that it is the next big wave in pharma, following the biologicals wave.

1 / A giant leap forward

BY VIRTUE OF THEIR MECHANISM
OF ACTION, CELL AND GENE
THERAPIES PROMISE TRULY CURATIVE
TREATMENTS

The treatment of serious diseases has long been based around the use of rather indiscriminate drugs and therapies, or invasive surgery. These are often effective at treating symptoms, yet few cure the underlying condition. Organs may never fully regain their ability to perform a vital function. Diabetes is a classic example: insulin injections can replace the function of damaged pancreatic islet cells and offer patients a relatively "normal" life, but are hardly a short-term, low-impact or side-effect-free solution. And diabetics are the lucky ones – many other diseases have no or only rudimentary symptomatic treatments.

A better solution to conventional pharmacotherapy is to replace the damaged tissue itself. Organ transplantation may be an option here, albeit imperfect. But there's another option – if every human being developed from one single cell, then why not manufacture the required tissue instead, either in situ or outside the body for implantation? This is exactly the approach cell and gene therapies are pursuing – to restore functionality of tissue, thereby reversing severe disease and potentially improving the life of millions. → [A](#)

GENE THERAPIES

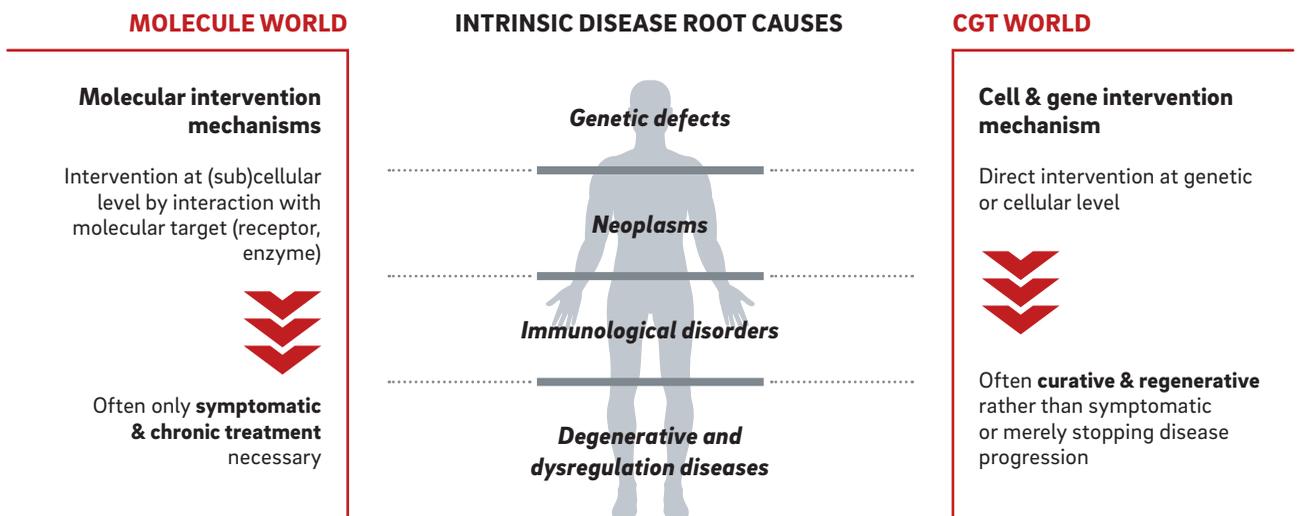
Many diseases are caused by genetic defects, one of the four intrinsic root causes of disease. They occur when the genetic code (DNA) of the affected person lacks the information to produce a certain protein, or codes for a dysfunctional version of it. If a defect on one specific gene causes the disease, it is called a monogenetic disease. While conventional therapies treat symptoms, gene therapies target the defective gene itself. There are two main techniques: gene augmentation and gene editing.

GENE AUGMENTATION

In gene augmentation, the missing piece of DNA is introduced into the nucleus of the affected cells. To ensure the new DNA reaches the targeted tissue, a

A: Cause for hope: Cell and gene therapies offer several advantages over conventional (molecular) interventions across the four main intrinsic disease types

Treatment mechanisms



Source: Roland Berger

scalable delivery vehicle is required. Modified adeno-associated viruses (AAVs) have emerged as the vector of choice for this purpose. Normal viruses deliver their DNA into a host's cells. But in gene therapy, instead of carrying their own DNA, AAVs carry the missing piece of DNA to the nucleus of the target cell.

Once delivered, the cells have blueprints for producing the missing functional protein. This "cures" the affected cell, but if it divides, there is a dilutive effect resulting in reduced expression of the protein over time. This effect is less pronounced in tissue with slower cell divisions, such as that in the central nervous system or eye.

The first commercially available gene therapy,

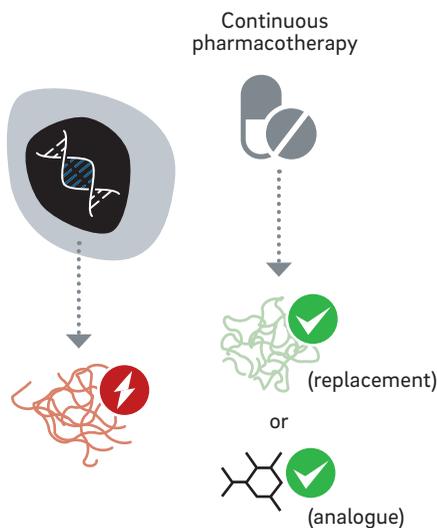
Luxturna (voretigene neparvovec-rzyl) from Spark Therapeutics, specifically targets retinal tissue. It is used in patients with a gene defect that prevents the photoreceptors in the eye from producing a functional protein, causing them to die prematurely. Called retinitis pigmentosa, the disease usually results in blindness. Luxturna halts its progression by delivering new DNA into the photoreceptor cells, enabling them to start producing the functional protein.

It is worth noting that gene therapy cannot restore tissue that has already been lost. This means a disease can only have advanced so far for a gene therapy to be therapeutically beneficial. → **B**

B: Direct approach: Gene augmentation and gene editing target the defective gene, rather than merely helping to mitigate symptoms of disease

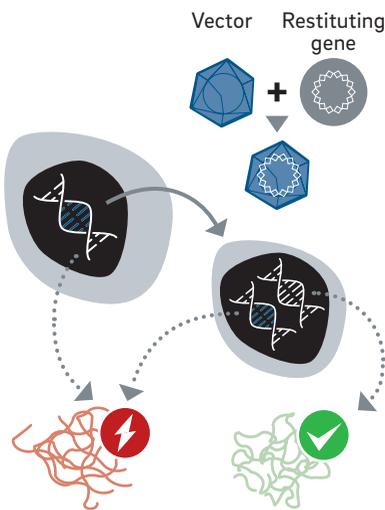
Gene therapy

CONVENTIONAL



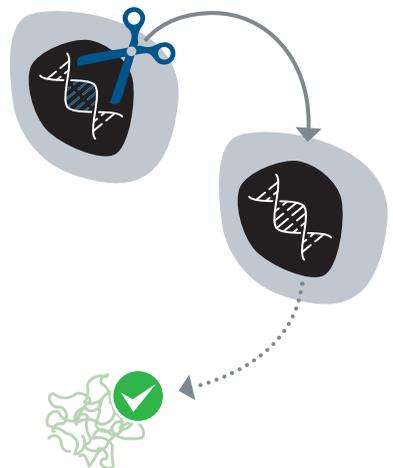
Dysfunctional gene results in the production of a **dysfunctional protein (often no drug available)**

GENE AUGMENTATION



Augmentation of the functional gene results in the **restoration of a functional protein**

GENE EDITING



Gene editing **corrects the dysfunctional gene**

Source: Roland Berger

GENE EDITING

The second gene therapy technique can be used to make changes to existing DNA, through knockouts, deletions, corrections and additions. Gene editing can therefore help to tackle diseases that are outside the current focus of gene augmentation.

Rather than transferring a DNA blueprint for a protein, gene editing makes use of a molecular tool, so-called gene scissors. CRISPR is a well-known example. These

tools very precisely cut the DNA and allow correction of the genetic defect. Gene editing can be performed in vivo using a delivery tool such as AAVs, or ex vivo, where extracted cells are edited in a laboratory before being infused into the patient. → **C**

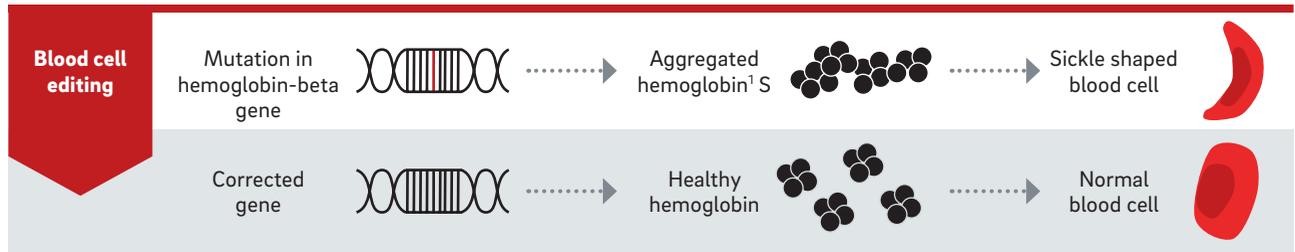
CELL THERAPIES

Cell therapies take a more macro approach to combating disease. Rather than targeting genes, whole human cells,

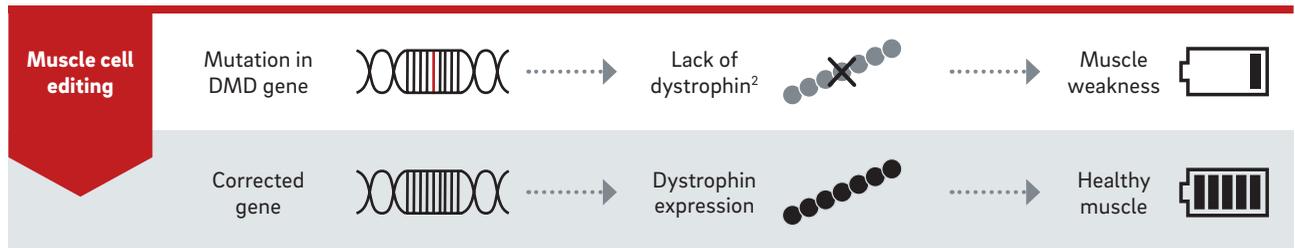
C: Potential targets: Diseases caused by a genetic mutation can potentially be targeted by in vivo or ex vivo gene editing

Therapeutic approach

EX VIVO EXAMPLE – SICKLE CELL DISEASE



IN VIVO EXAMPLE – DUCHENNE MUSCULAR DYSTROPHY



1 Hemoglobin protein in the blood that transports oxygen to the tissues

2 Dystrophin is part of a protein complex that works together to strengthen muscle fibers and protect them from injury as muscles contract and relax

Source: Roland Berger

or even tissues, are used as therapeutic agents. Two main techniques are being pursued: (1) replacing exhausted or degenerated cells and (2) augmenting cells to give them therapeutic functionality.

REPLACING DEGENERATED CELLS

The principle here is relatively straightforward: cells of the depleted tissue type are cultivated in a petri dish in a laboratory (ex vivo production) and then implanted

into the patient. There they take over and restore the function of the diseased or degenerated tissue. Several application areas requiring only one cell type exist, for example chondrocytes to restore cartilage defects in joints such as the knee or hip, or pancreatic islet cells to restore functionality in the pancreas of diabetics.

Tissue engineering goes one step further by attempting to produce an entire piece of tissue or even more complex organ-like structures in a laboratory, for

Cell therapies pursue the vision that diseases can be cured by administering healthy cells to replace diseased cells or by administering engineered cells as therapeutic tools to correct disease.

subsequent reimplantation. Skin to treat burn injuries was an early target as it can be supplied by surrounding vasculature and grown extensively in the laboratory.

Two types of source cells can be used in cell therapies: cells taken from the patient themselves (autologous) and those taken from another donor (allogeneic). Autologous therapies avoid the risk of cells or tissue being rejected by the patient's immune system. But they also have several complications. The process of proliferation is complex and must be performed for each patient individually, pushing up production costs. In addition, many cells cannot be proliferated in the test tube, or the source cells are dysfunctional. Such therapies therefore have limits.

Allogeneic applications are more versatile and can be used to treat multiple patients. They are often based

on undifferentiated cells or even stem cells. These have retained the potential to proliferate, grow into specific cell types and show no or few markers that can trigger an immune response from the host. Such cells are either derived from donor tissue that shows such features by default (e.g., mesenchymal stem cells derived from umbilical cord) or are modified by means of gene editing so that they no longer produce immune markers. The idea is that patients are treated with an allogeneic cell line, which significantly reduces production costs. Cell lines can even be kept in stock.

Allogeneic applications can also be taken a step further. In order to tackle the challenge of cell proliferation and scalability of manufacturing, further cell engineering technologies exist. Induced pluripotent stem cells (iPSCs) are perhaps the most exciting technology. Pluripotent stem cells are undifferentiated cells that can develop into any type of tissue or cell and be easily multiplied. iPSC technology turns mature adult cells into pluripotent cells through genetic reprogramming. This means mature cells are taken from a donor, reprogrammed into iPSCs, edited to be invisible to the immune system, multiplied at an industrial scale and then processed to differentiate into the desired cell type.

In short, stem cell therapies pursue the vision of producing any type of cell for any patient in scalable quantities, addressing many currently incurable diseases at their root cause. → [D](#)

CELLS WITH AUGMENTED FUNCTIONALITY

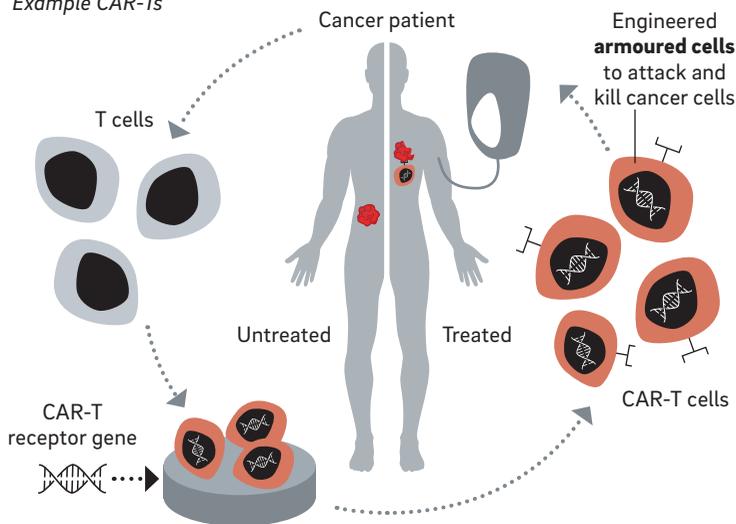
In addition to treatment with like-for-like cells, a second cell therapy technique is gaining traction: genetically modifying cells to add functions and turn them into therapeutic tools. For example, T cells, a type of white blood cell, can be genetically modified in vivo to carry a man-made receptor, called CAR, which enables them to bind to and destroy certain blood cancers. The modified T cells are then infused to a patient to serve

D: Strength in numbers: Unlike autologous therapies, allogeneic cell lines can be used to treat multiple patients, making them the more scalable therapeutic option

Cell therapy

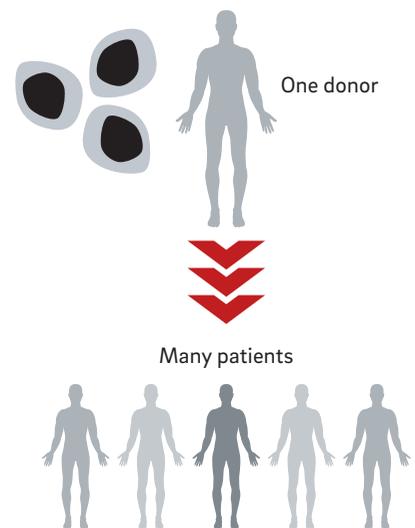
AUTOLOGOUS

Example CAR-Ts



The patient gets **re-administered his own, processed cells**

ALLOGENEIC



One immune-evasive **allogeneic cell line** can be used **for many patients**

Source: Roland Berger

as a highly personalized and powerful tool against these hematologic malignancies.

As well as blood cancers, research is opening up the possibility of treating solid tumors with augmented cells. Another recent development is the use of cell types such as natural killer (NK) cells. These are part of the innate immune system and can be applied in

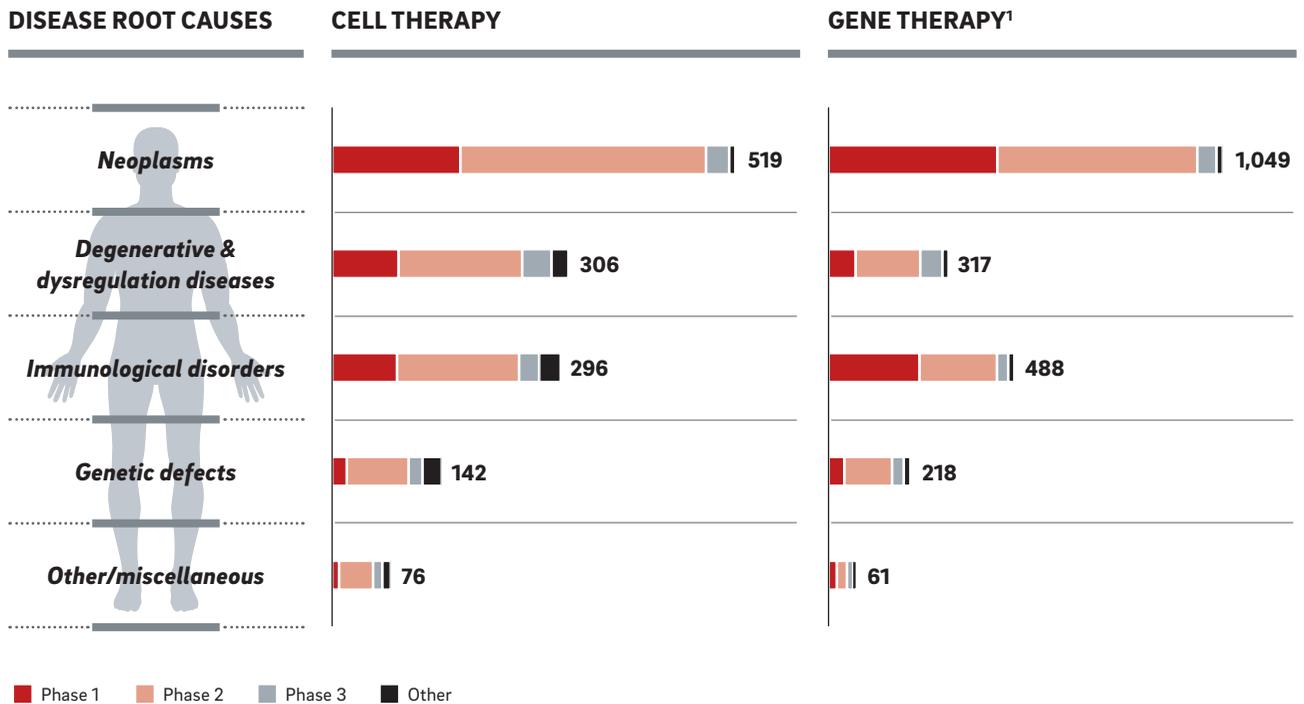
different patients without the need for allo editing. Both cell types, as well as having different constructs, have advantages and disadvantages. Therefore, a clear strategy is required during the design of a specific product, focusing on all aspects of safety, cell activity, tumor infiltration, persistence and the application of further enhancements.

As with regenerative approaches, the cell source for cancer applications can be autologous or allogeneic. Current approaches in hematologic indications are focused on autologous cell therapies, but a shift of focus to allogeneic cells is ongoing. This will allow a higher scalability of manufacturing paired with off-the-shelf applicability. In the long run, iPSC-derived cell therapies may emerge as the endgame because of their cell homogeneity and scalability.

Cells can also be engineered to continuously secrete therapeutically active substances such as anti-inflammatory factors, actively contributing to the regeneration of surrounding tissue. Gene editing could even enable implanted cells to measure the degree of inflammation in the surrounding tissue and adjust the production of anti-inflammatory substances accordingly. Or production of the substance could be switched on or off by administering a transmitter substance. Again, the potential is huge. → **E**

E: Leading research: Neoplasm treatments make up 50% of all CGT development programs

Clinical trials in cell & gene therapy by stage [Q3 2019]



¹ Some clinical trials are based on genetically modified cells, which are counted inconsistently in databases as gene or cell therapy

Source: Alliance for Regenerative Medicine (ARM), Roland Berger

2 / Untapped potential

THE CGT MARKET IS SET TO BOOM, AND BIG PHARMA COMPANIES ARE SPENDING LARGE SUMS TO SECURE A SHARE

Cell and gene therapies don't just have huge potential to change lives – their economic potential is correspondingly large.

THE CGT MARKET

Total CGT sales are expected to hit EUR 1.8 billion in 2021. But the figure is projected to jump to EUR 27.9 billion in 2026.

Forecasts from the US Food and Drug Administration (FDA) suggest that from 2025, it will approve up to 10 CGT products per year. Of the programs currently in clinical development or already on the market, several are expected to be blockbusters, that is, pharma products with annual sales in excess of USD 1 billion. These include Zolgensma, a gene therapy for muscular atrophy that is forecast to have sales of EUR 1.6 billion (USD 1.9 billion) in 2026. → **F**

With the sizeable potential of the CGT market in mind, it is hardly surprising that large pharmaceutical players have already bet big on cell and gene therapies.

Two distinct areas of focus have emerged. The first is the development of cell and gene therapies to cure a specific disease. The second is the development of platform technologies, or products that can be applied to several different therapeutic programs and serve as enabling technologies or technological building blocks. Both streams have already yielded companies that have achieved valuations of several billion dollars – most before first revenues have even been generated.

BILLION-DOLLAR PLAYERS

One example is the immune-oncology specialist Fate Therapeutics. Founded in Germany but now based in California, it specializes in the production and reprogramming of iPSC-derived natural killer (CAR-NK) cells and T cells (CAR-T). Fate recently formed a first partnership with US pharma giant Johnson & Johnson, and although its programs are still in early clinical

Forecasts from the US Food and Drug Administration (FDA) suggest that from 2025 onwards, it will approve in the order of 10 CGT products per year.

development, market capitalization has already reached several billion dollars. As an acquisition target it will attract a significant premium, however, as was seen in recent deals involving the purchase of Juno Therapeutics and Kite Pharma. → **G**

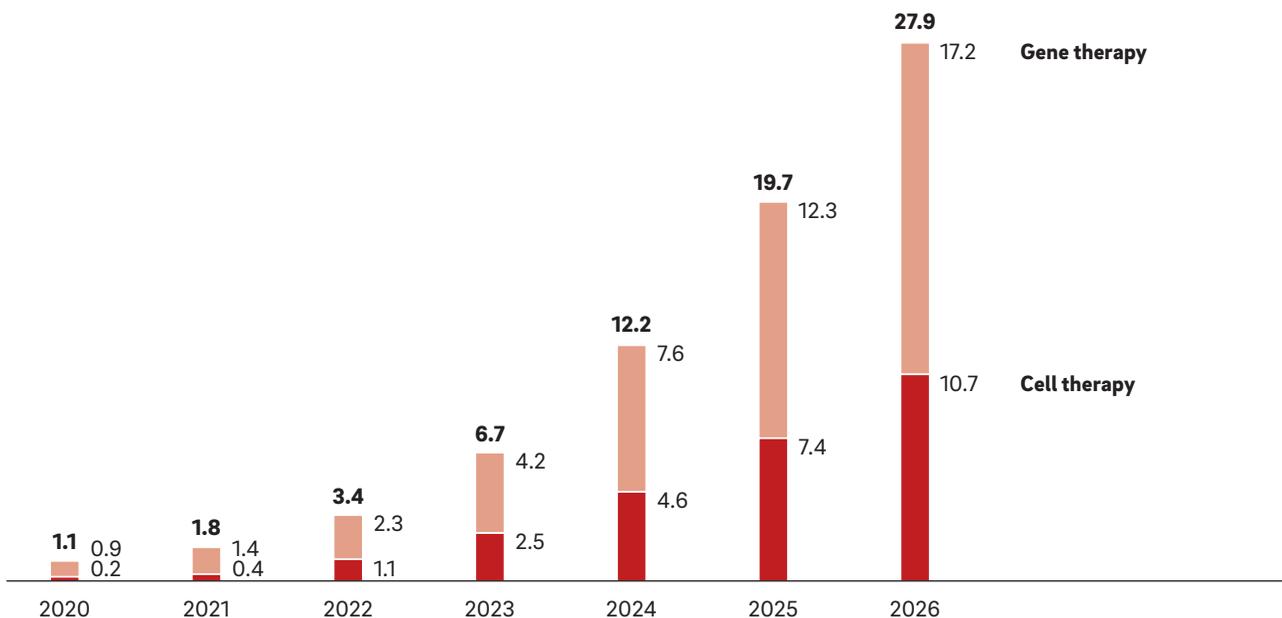
Another company with a valuation of several billions is CRISPR Therapeutics, a Swiss-based firm co-founded by one of the inventors of the CRISPR gene-editing technique. It specializes in the application of gene editing for ex vivo and in vivo applications and licenses the foundational CRISPR/Cas intellectual property to other cell and gene companies.

F: Boom times: The CGT market is set to soar in the coming years, driven by several new potential blockbuster therapies

Cell and gene therapy sales [2020-2026E; EUR bn]

CELL AND GENE THERAPY POTENTIAL BLOCKBUSTERS

	Company	Platform	Indication	Phase	Sales 2026e
Zolgensma	Novartis	AAV	SMA	Marketed	EUR 1.6 bn
MultiStem	Athersys	Stem cell	Various	Phase III	EUR 1.3 bn
Zynteglo	bluebird bio	LV	Beta thalassemia	Marketed	EUR 1.2 bn
LN-144	lovance	TILs	Melanoma	Phase II	EUR 1.1 bn
CTX001	CRISPR Tx	CRISPR	Beta thalassemia	Phase II	EUR 1.1 bn
LN-145	lovance	TILs	Solid tumors	Phase II	EUR 0.8 bn

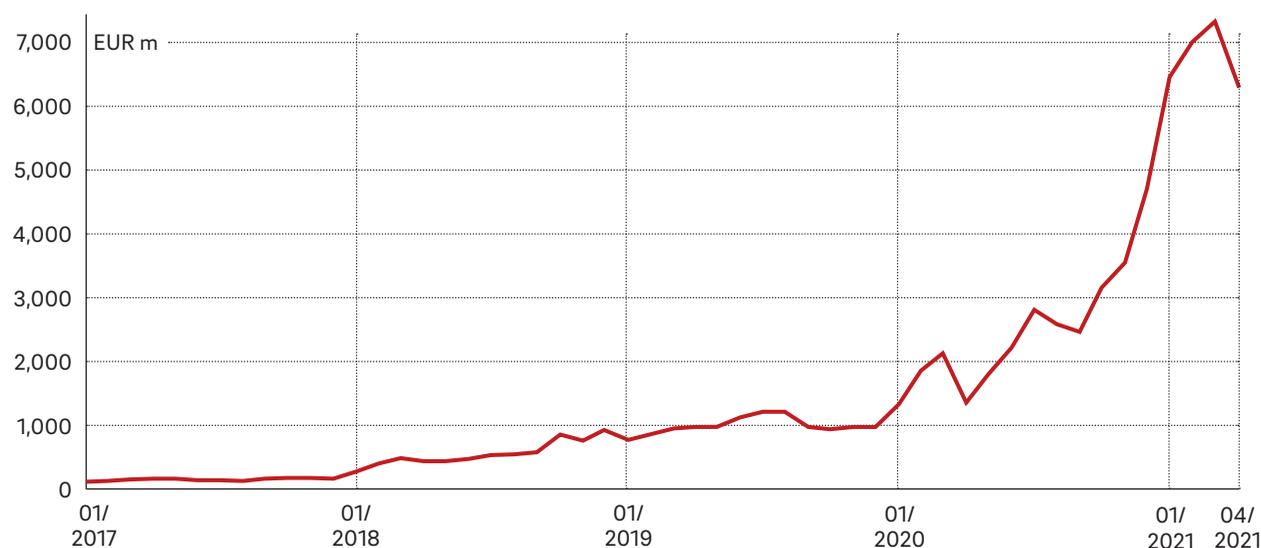


1 Including combination ATMPs

Source: Evaluate Pharma

G: Destined for success: Fate Therapeutics has seen its market capitalization soar since its inception in 2017
Evaluation concept – Example of FATE Therapeutics

MARKET CAPITALIZATION OF FATE THERAPEUTICS



Source: Fate Therapeutics website (accessed April 20, 2021)

ACCESSING THE MARKET

Fate and CRISPR Therapeutics are good examples of the platform technology stream model. Both are mastering a platform technology and offering partners very specific and limited access to their platform. In the case of CRISPR Therapeutics, for example, customers can purchase a license for a certain modification to a certain cell type for a specific disease. Even if they could use the purchased reagents in other areas of work, licensing agreements would severely restrict their use.

Such restrictions mean the only way to get full access to a technology or platform is to buy the

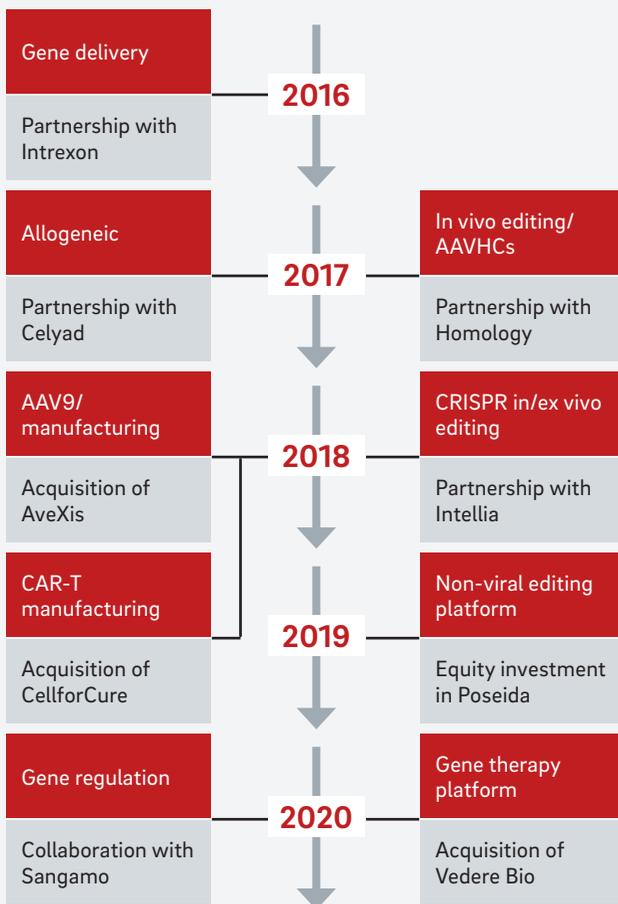
company behind it. The US-based biotech giant Gilead did exactly this in 2017 when it bought Kite Pharma, a leader in cell therapies, for a record sum of USD 11.9 billion. At the time, Kite's most advanced prospect was Axicabtagene ciloleucel (axi-cel; Yescarta), a CAR-T cell therapy that had been submitted for a regulatory review in the US. It became the first therapy to be approved and launched as a treatment for refractory aggressive non-Hodgkin's lymphoma. The high price Gilead paid was therefore not based on the potential of axi-cel alone, but rather on the value of Kite's underlying platform. → [H](#)

H: String of deals: Early movers Novartis, Gilead and Pfizer have been highly active in CGT partnerships and acquisitions over the past few years

Selected Big Pharma case studies

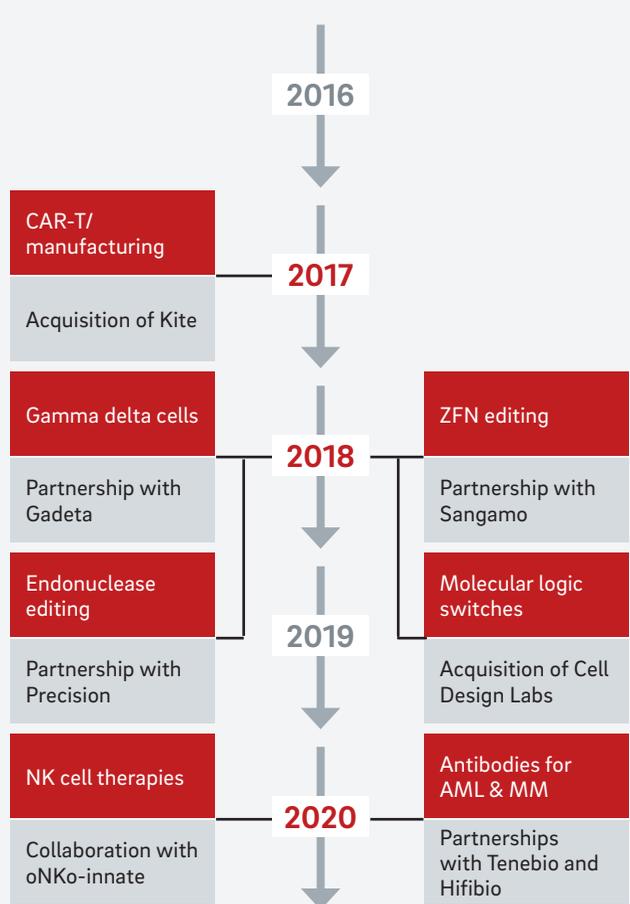
NOVARTIS

Has established cell and gene therapies capabilities through the acquisition of AveXis and development of Kymriah, and has expanded its toolbox in gene editing, allogeneic technology, etc.



GILEAD

Has invested extensively in editing technologies and manufacturing to build upon its growing cell therapy franchise Kite

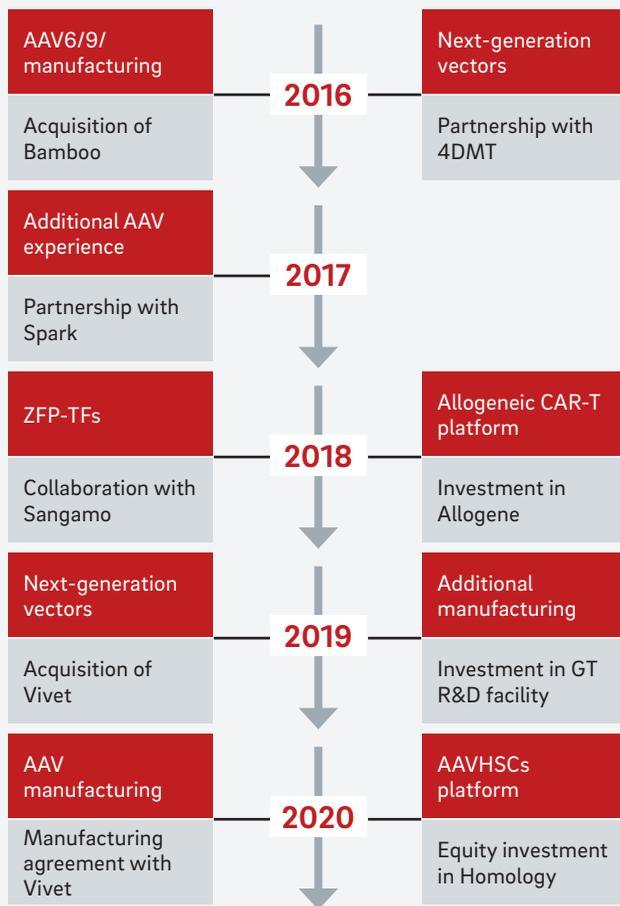


■ Capabilities ■ Activities

Source: Company websites, Roland Berger

PFIZER

Early entrant into the gene therapy space and has since bolstered the platform through investment in editing and next-generation vectors; CAR-T cell therapies have been transferred to Allogene Therapeutics



A closer look at the activities of Big Pharma in the CGT arena reveals that they share certain characteristics – a pattern is developing. On the one hand, their CGT operations are built around a strong nucleus, to which complementary technological and therapeutic programs can be added to realize their full potential. The focus is predominantly on the technology platform. On the other hand, technological capabilities are in-licensed, a move that makes sense given that the current high valuations of licensors mean their acquisition is often out of reach.

JUSTIFYING THE COST

While the rationale for Big Pharma's interest in CGT is clear, one key question remains – why are pharmaceutical companies willing to pay such huge amounts for CGT acquisitions, especially bearing in mind the significant remaining investment costs involved? We see three main reasons:

- 1) While the FDA assumes that 10 cell or gene therapies will receive market approval per year from 2025 onwards, only very few CGT products have been approved to date. Compared to small molecules and biologics, growth rates are substantial and highly attractive.
- 2) CGT represents a completely novel field of technology, in which existing knowledge is largely irrelevant. For many established pharma companies this represents an opportunity to expand into novel areas with a new and potentially superior technology. In many ways, these opportunities may reshuffle the cards in therapeutic areas currently addressed by classical pharmacotherapy. As a result, failure to seize the opportunities of CGT may become a risk.
- 3) Science- and innovation-driven biotech companies have spent several years developing technology platforms and domain expertise protected by broad IP. For established players to build these up internally will take specific capabilities and create a significant time disadvantage.

3 / Recommendations

NEW MARKET ENTRANTS NEED TO CONSIDER FIVE KEY PRIORITY AREAS BEFORE TAKING THE PLUNGE INTO CGT

It's clear that cell and gene therapies hold considerable promise and present significant opportunities for pharmaceutical players. But how can they realize these? In this final chapter, we outline the key considerations and recommendations for companies looking to move into the CGT space, broken down into five priority areas.

RESEARCH & DEVELOPMENT

In the CGT field, platform technologies play a much more prominent role in R&D activities than in the traditional molecule-based pharmaceutical field, where the underlying platforms have matured over decades. The example of gene augmentation therapy demonstrates why. It requires the production of a missing DNA fragment, the encapsulation of the DNA in a viral vector and the delivery of the vector to the target tissue. But once this process has been mastered, it can be applied to other disease therapies, with only the production of a different DNA fragment required. Such leveraging of technologies is akin to a galenic formulation technology in the molecule world. At the same time, with every clinical trial, the understanding of the therapy improves, increasing the probability of success. In principle, the platform is indication-agnostic, and can be rolled out in multiple therapeutic areas.

Full control and continuous development of the underlying technology platform is therefore of utmost strategic importance to CGT players. A good example to underline this are augmented cells for therapies that include local payload delivery, functional on and off switches and advanced synthetic biology functions such as gene circuits, all of which are at an advanced research stage.

Another key differentiating feature compared to classical pharmacotherapy is the manufacturing process. Cell and gene therapies are based on living organisms and not molecules. The quality and control of the manufacturing process therefore determines the

efficacy and safety of the final product. Accordingly, the early development of robust and scalable production processes is of particular importance.

In addition, the delivery of the product into target tissue is often a much more complex undertaking than in classical pharmacotherapy. Delivery mechanisms for areas such as tissue tropism or cell homing/loading and infiltration have to be developed from scratch, and the ADME toolbox of pharmacotherapeutics is often not transferable to the CGT world. This means a build-up of new expertise and capabilities in delivery methods and the entire chemistry, manufacturing and controls spectrum is required.

BUSINESS DEVELOPMENT & LICENSING

Currently, most large pharmaceutical companies have very few CGT development programs in the pipeline. This helps to explain the flurry of activity in acquisitions, and why they are likely to continue.

To address this, we recommend establishing a team that can rapidly (weeks not months) evaluate potential deals in this fast-paced environment. It should include members carefully chosen for their M&A experience and CGT knowledge, and be given powers to drive such deals. Lean BD&L processes with an emphasis on science rather than evidence to allow fast execution without too many functional checkpoints and stage gates will be key to achieving this.

A typical challenge in determining the valuation of such acquisition targets lies in the fact that their development programs (assets) are at an early stage and have very low NPVs after risk adjustment, while their true company value is determined mainly by their platforms. However, compared to assets, the valuation of platforms is more difficult and requires a deeper understanding of the target company, technology and competition. Pure application of comparator deals is very often meaningless as platforms differ significantly

and the sample size is still relatively low. The team must therefore develop ways to adequately grasp the value of a platform, and be given the tools to do this. Only then will the acquirer be able to meet the financial expectations of the target.

MANUFACTURING

New players entering the CGT sector must pay special attention to the production process from the get-go. This means building the entire manufacturing and supply chain from scratch, and maintaining tight control over it from the start. Without this, pharma companies will incur accretion costs and could endanger therapeutic programs, as we saw happening in the early days of CAR-T cell therapies.

To be able to actively exert control, we recommend striving to own the entire end-to-end manufacturing process. In addition, developing the supply chain anew provides many opportunities to optimize it, enabling patient-specific shipments or effective connections with point-of-use locations. Bear in mind that manufacturing platforms for CGT products have not yet been fully established, presenting opportunities to become a market leader.

If players are active in the CDMO space, it's important to take into account that this is a suppliers' market with high prices that prevent cost leadership. Additionally, the CDMO space offers opportunities for near-term monetization of CGT platforms and thereby contributes to the overall company value. Acquisition prices paid by established CDMOs to strengthen their CGT capabilities are high, underlining the high expectations in this new field.

MARKET ACCESS, MARKETING AND SALES

CGT is a new sector, and must be treated as such. This means making changes to the development process, business models and sales strategies.

During clinical development, new products need to show significant patient benefit because of their high costs and complex application compared to conventional treatments. Incremental achievements over the standard of care alone won't justify their use, making the focus on indications with no or low standards of care a priority. Particularly in rare diseases that are incidence driven, new players should ensure a packed pipeline of products and frequent launches. This is because revenue peaks will occur a few years after launch, driven by the prevalence of a hitherto untreated condition, and then fall away. Early interaction with regulators and payors will enable the fast rollout of new therapies.

There are several considerations around marketing CGT products. First, market access will require new payment models in order to secure the backing of payors for what are expensive treatments. Second, being first to market in rare diseases is of utmost importance as the curative nature of CGT quickly reduces the patient pool, leaving followers with small incidence pools. Lastly, new commercial models are required with small and expert sales teams to target the small number of specialized treatment centers.

CGT players must also remain alert to the threat of competition. Although generic or biosimilar-like loss of exclusivity will be less of an issue, the threat from new, improved technologies will be very real. Abbreviated development times (for example, breakthrough status) require holistic and continuous competitor screening across all technologies to take fast, informed decisions for portfolio assets.

ORGANIZATION, GOVERNANCE AND CULTURE

As outlined in the previous chapters, successfully and sustainably entering CGT requires a fully vertically integrated approach, embracing the underlying technology platforms along the entire value chain from science to patient. Furthermore, CGT is not confined to

Conclusion

any specific indication but spans virtually all therapeutic areas within pharma. As a result, it is a significant challenge to properly set up CGT within established pharma organizations, both on the functional side with established R&D, manufacturing and supply chain organizations centered around small and large molecules, and on the business unit or franchise side designed around therapeutic areas. To address these challenges, a cohesive operating model and governance framework needs to be devised in a tailored fashion that is sensitive to the cultural aspects of the existing organizations.

There is little doubt that cell and gene therapies are a promising new segment within pharma. We believe that they represent the next big wave in pharmaceutical innovation, and will rapidly grow to become an important – and lucrative – part of the industry. Due to the enormous differences between classical pharmacotherapies and CGT, market entry is not straightforward. But success is achievable with the right preparations.

We believe there are three key considerations. First, pharma companies must enter the CGT world with the aim of quickly establishing end-to-end capabilities. Second, with many key technologies from the first wave of CGT development already sold out, they should target the even bigger potential of emerging technologies. Third, they should take into account that many new therapeutic areas are on the rise, and place their bets accordingly.

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05.2021

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