GLG

EBOOK

GENE THERAPY: THREE EXPERT PERSPECTIVES

With Contributions from:
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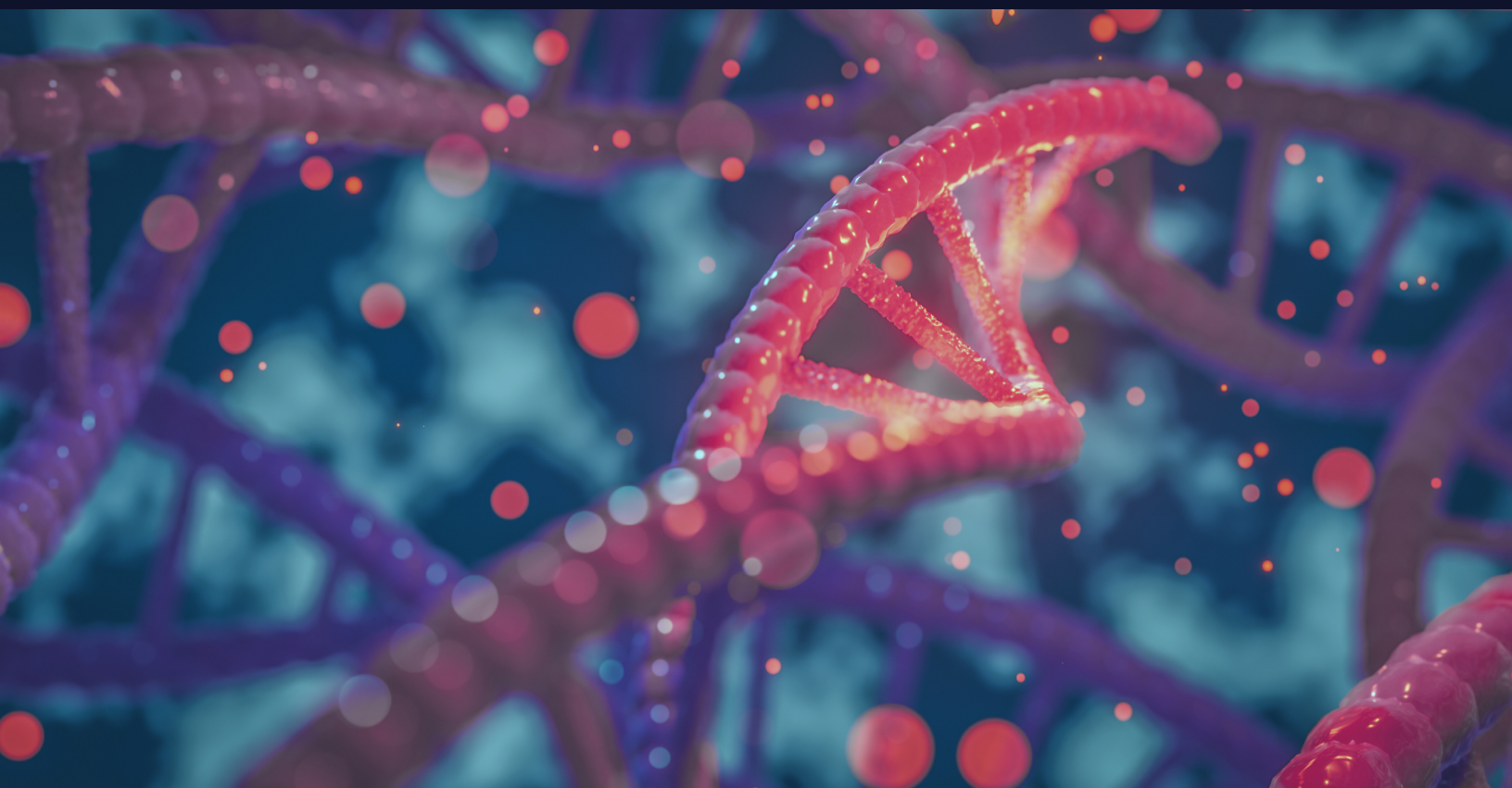

INTRODUCTION

Introduction written by Hersh Gupta, Vice President, GLG

Cell and gene therapy are the next frontier in medicine. These revolutionary developments unlock the ability to treat and sometimes cure diseases and conditions that until now could only be treated palliatively. Cell and gene therapy can allow physicians to alter a patient's cellular and genetic makeup in order to treat serious medical conditions, such as leukemia and spinal muscular atrophy.

CELL THERAPY

Broadly speaking, a physician performing cell therapy treats a disease by injecting live cells — that have been altered outside the body — into a patient. The cells can be the patient's own cells (autologous cells) or a donor's cells (allogeneic cells).

GENE THERAPY

Gene therapy treats disease by altering a patient's genome. A person's genetic code is changed using a viral vector that introduces genetic material into a patient's cells. This changes how a cell produces a single protein or group of proteins. Non-viral gene therapy is also being intensely studied; it involves the introduction of genetic material into cells using methods such as electroporation and ballistic delivery.

THE GROWTH OF A MARKET

Several companies, such as Novartis, Kite Pharma, and AveXis, already have cell and gene therapies on the market; however, these therapies remain an area of great interest in the biopharma industry. Nearly 300 cell and gene therapies are currently in development[1], establishing these as a valuable and fast-growing market that will be worth billions of dollars over the next decade.

[1] https://catalyst.phrma.org/medicines-in-development-for-cell-and-gene-therapy

WHAT YOU'LL FIND HERE

In this ebook, you'll find the perspectives of three different executives, all of whom are important players in the cell and gene therapy value chain.

- **EDWARD LANPHIER**, the Founder and former CEO of Sangamo Therapeutics, a genomic medicine company, shares an executive's perspective on the cell and gene therapy space and comments on the areas of focus and mergers and acquisitions (M&A) activities in the space.
- **DR. ED PEZALLA**, former Vice President of Pharmaceutical Policy and Strategy at Aetna, shows us how payors view cell and gene therapies and the factors to consider in getting them reimbursed.
- **LANCE WEED**, former Vice President of Operations at uniQure, gives us his perspective on the costs, risks, and challenges of producing cell and gene therapies at scale.

These perspectives help enumerate some of the major challenges and opportunities that exist for cell and gene therapies. They provide insight into the hurdles that will need to be overcome in order to ensure continued growth of this space.

GOING FORWARD

Cell and gene therapy will be an area of focus for many years. Hundreds of ongoing trials for therapies have the potential to treat and even cure dozens of conditions, like rheumatoid arthritis, diabetes, and various cancers.

Adeno-associated virus (AAV) evolution will continue to be a focus area, along with approaches for tissue-specific, non-viral delivery of gene therapies. However, large-scale manufacturing of cell and gene therapies will continue to be a hurdle as more players enter the space.

As the Food and Drug Administration (FDA) approves more cell and gene therapies, which then start hitting the market, payors must continually evaluate these therapies' benefit for patients. Cell and gene therapies are some of the most expensive medications on the market, and payors need to determine how to pay for these treatments, which will lead to a shift in current reimbursement models.

GENE THERAPY OUTLOOK:

An Executive's Perspective

Edward Lanphier, GLG Network Member and former CEO at Sangamo Therapeutics

The gene therapy space is ripe for mergers and acquisitions (M&As). Within the past year, Biogen acquired Nightstar, Roche purchased Spark, and Astellas grabbed Audentes. M&As will not only continue in the space but also accelerate.

Acquisitions, however, present tension to big pharma and gene therapy companies. Should the giants seek extraordinarily generous licensing terms, particularly around later-phase or clinical assets, or acquire entire organizations? On the other side, should biotech, gene therapy, or genome editing companies license agreements around principal, if not significant percentages of their technology value or market caps, as they evolve their business strategies? Should they leave little to nothing for an acquirer to come in and pick up? Do they remove the opportunity for an exit?

Scanning the list of the major pharma players, I'm positive all are making major strategic decisions regarding their approach to this space. They'll tell you that they're looking at companies that have been clinically de-risked — essentially later-stage companies. Whether it's an adeno-associated virus (AAV) platform company, a genome editing company, or even a cell therapy platform company, executives don't mind if the smaller companies out-license some of the more mature programs if the platform has legs. They'll also seek those with enough runway or platforms to which multiple products can be applied.

Beyond single-gene targets, big pharma is beginning to look at pathways and multi-gene targets: the next generation of the science as well as company formations. In other words, big pharma will be looking to skate to where the puck is going.

Areas of Focus

So where's the puck going? AAV evolution is a major development area. Virtually every gene therapy and genome editing company using AAV is investing enormous resources in the development, optimization, and continued evolution of various serotypes in the AAV platform. For examples, see 4D Molecular Therapeutics and Voyager Therapeutics. All the major gene therapy companies have worked hard to find novel or best-in-class serotypes for specific tissues. You can see that playing out in some of the earlier-generation vectors that are now in advanced clinical trials.

On the non-viral side, we will see continued progress in tissue-specific delivery with non-viral vectors or approaches. Those are more transient, but from a genome-editing perspective, where you can get permanent modification, it offers significant opportunities.

In terms of the stem cell space, the bluebird bio approach, which uses modified retrovirus, is the most advanced. That program is in registration in Europe and is moving forward in the treatment of beta thalassemia and sickle cell anemia. But second-generation approaches — including non-viral delivery, genome editing with CRISPR, and zinc finger nucleases — in theory offer significant differential technical advantages over a retrovirus.

Challenges

With many exciting treatments coming down the pike, a key challenge will be meeting manufacturing demand. Contract manufacturing organizations are making significant efforts to increase the kind of capacity needed for phase-three and market launch approaches. Pfizer is putting a stake in the ground in terms of committed viral vector manufacturing, based on both the Spark deal as well as the upcoming hemophilia A program. But that's a drop in the bucket. As investors evaluate commercial launches and therefore market penetration, particularly for some of these high-dose viral vectors, they'll find it's not a trivial undertaking.

Access to capital will, of course, remain important for biotech companies. That gets into the tension between out-licensing lead programs and leaving enough technology value and product value within the company such that shareholders retain a real upside. Finally, personnel is always an issue, and attracting and retaining great people is a tension in any technology industry.

How insurers and patients will pay for these treatments is also a big question mark for the space. The Alliance for Regenerative Medicine (ARM), a group in Washington D.C., and the ARM Foundation published a report on reimbursement that I highly recommend. Given the state of the U.S. private insurance system, payments will be complex. I believe these products will be approved with clear safety and efficacy data, but we won't have long-term outcome data, and a bias will exist toward pay-for-performance kinds of outcomes. With the absence of long-term durability data, pricing therapies at a premium will be difficult.

There's a bright spot among all these challenges: The FDA's Center for Biologics Evaluation and Research and the cell and gene therapy group are well informed and excellent industry partners. That's something companies and investors can feel positive about as they navigate launching these amazing new treatments to the market.

GENE THERAPY OUTLOOK:

The Payor's Perspective

Dr. Ed Pezalla, GLG Network Member and former VP of Pharmaceutical Policy and Strategy at Aetna

Gene therapies can help patients with illnesses that either aren't treatable or require difficult, expensive, or problematic treatments, but also, in many instances, they only need to be given once in a person's lifetime. It's this latter element of gene therapy that begs the question: How will insurance companies and/or patients pay for these treatments when they've only dealt thus far with medicines that require regular administration?

To be sure, one-time therapies have long existed in medicine, but they're different in character from ongoing treatments. For example, if you remove a patient's appendix, their appendicitis is cured; the same goes for gallbladder disease. Outcomes of other treatments, such as bone marrow and organ transplants, are less certain.

We currently pay for pharmaceuticals for chronic diseases on an ongoing basis. If it's not working, at some point treatment stops — it's not being paid for anymore — and the patient moves on to a different therapy. High blood pressure medications are a good example of this, where a patient gets a new drug when their body no longer responds to the original treatment.

One-time therapies totally change the ballgame, and that's where two big issues need solving in terms of payment models. First, we must consider how we value these treatments and what methodologies should be used. The other issue is making these treatments affordable. This is important for payors with less funds, such as Medicaid programs that can't go into debt or borrow, or a small health plan.

Here are the factors we need to consider:

The Touchpoints for Payment

When determining payments, two factors come into play depending on the illness treated: outcomes or milestones.

A clinical outcome could be something that happens or doesn't happen for the patient individually. Take hemophilia, for example: What's the patient's bleed rate over a certain period? Because patients don't have a lot of bleeds now, doctors are more likely to look at laboratory measures to find the rate. This works with hemophilia because factor levels correlate with bleed rates and complications of the disease.

Milestones can be related to those outcomes. You might measure a laboratory value at six months, 12 months, every year after that, and so on. Payments could be tied to the level of lab value, whether it's above a certain threshold or between certain parameters. But you could have other milestones as well — for example, when the patient doesn't need another therapy. In patients with beta thalassemia, we could consider how many blood transfusions an individual requires in each time frame. With CAR-T, doctors may look at the disease's progression, which could be measured in a variety of ways, depending on the tumor.

Longer Time Frames May Be Needed

Payors could base payments on outcomes or milestones on a single endpoint — for example, at 12 months, is the patient making enough hemophilia factor? But that doesn't really answer the question "Is the treatment is working?" It also doesn't solve the problem of knowing whether or not the therapy is sustainable and durable over time. We're much more likely to see milestones or outcomes measured over a three- to five-year period, depending on the disease state and how long doctors think we need to follow a patient before we know the treatment is working.

This, unsurprisingly, gives rise to what's basically a contract with multiple payments, like when buying houses, property, and businesses. This payment system is like a mortgage for patient care and is based on whether the patient is doing well at certain endpoints.

Harvard Pilgrim Health Care has already implemented this model with the first gene therapy on the market, Luxturna, the Spark Therapeutics treatment for congenital blindness. There are outcomes at 30 and 90 days, then at 30 months. Spark arranges for patients to be measured using a method similar to what was conducted in the clinical trial: The patient navigates a maze, showing that they can distinguish light and dark, to see outlines and shapes in a way that makes it safe for them to maneuver.

Bluebird Bio, meanwhile, offers five-year contracts in Europe for the treatment of beta thalassemia, which is based on patients not needing transfusions or having reduced transfusion requirements, with annual payments tied to those milestones.

The Challenges for Payors

If there were a true sort of mortgage or financial instrument tied to gene therapies, it's possible those could be traded or sold in some way, just as mortgages are done now. That would be an advantage to pharmaceutical firms, and not so much the payors. Since some firms are rather small, they may not have many marketable products, or the products may only be used in a small number of patients, so the potential to sell those financial instruments will help them gain some revenue immediately to fund other activities and items in their pipeline.

Some issues may arise. For example, some patients may be lost to follow-up. That's part of the contractual arrangement: How do you manage the contract if the patient can't be tested? Do the payments just stop? Is it the responsibility of the pharma company or the payor to find that patient?

Another issue is whether payments are attached to the patient and would move from one payor to the next. At this point, they don't. Most of the proposed plans involve a straight-out contract between two entities: the manufacturer and the health plan or the ultimate payor. If the patient moves to another health plan, the payor can still check on that patient's health, have them see their provider, and take measurements. But the contract is still with the plan that made the original payments, so the payor will continue to make payments over time.

For payments to transfer to another health plan, there likely needs to be federal legislation. There's no reason, otherwise, that an entity would want to take on such payments, unless it's joined a network of payors, perhaps created by a third party or a manufacturer, where they've all agreed to take each other's patients and accept responsibility for the payments. Right now, that's not happening, so if the patient moves payors, it doesn't change the original arrangement.

Other Factors to Consider

There are interesting components to the differences when an adequate therapy is available versus where there is none. It's difficult for health plans not to cover a gene therapy or other therapy for a serious disease that has no other treatment. It's a space with very little competition, so most of those treatments get covered. It puts less pressure on the manufacturer to accept a lower price in that setting, except that they'd want to maintain goodwill.

But what about gene therapies that treat illnesses where there's already a treatment? This situation changes the economics because now there's a benchmark. We know how much a hemophilia patient's treatment costs, and so we can make assumptions. One is that although the prices of hemophilia products seem high, we pay them, so willingness to pay has been established. There's also a direct-cost offset — if you're no longer obliged to give the patient the original treatment, you can save money. These elements affect product price accordingly.

You also can end up in a situation where the patient gets both: They undergo gene therapy and therefore become a less severe hemophilia patient. You may still have to augment their treatment. In that case, you must anticipate that in the contract and perhaps have a sliding scale of payments or rebates to consider.

The interesting ideas here are not only economic but also clinical: Whether a hemophilia patient is better off staying on factor is a clinical decision. So the gene therapy wouldn't necessarily be for everyone because some patients are being successfully treated otherwise.

A Real-World Test

All those factors will soon play out, as BioMarin recently announced it will price its gene therapy for hemophilia, Valrox, from $2 million to $3 million. The company argues that because it's a one-shot therapy, it's cheaper than a lifetime supply of treatment. That may be the case for an adolescent patient entering adulthood whose treatment could cost up to $200,000 a year. They would reach $2 million to $3 million in 10-15 years, so the price seems reasonable in terms of cost offsets.

But that's why payment over time makes a difference — you're not paying all those other costs all at once. Also, we don't know if patients will continue to respond to gene therapies for many years. The price looks good only if you think it's going to work well.

GENE THERAPY OUTLOOK:

The Manufacturer's Perspective

By Lance Weed, GLG Network Member and former Vice President of Operations at uniQure

The gene therapy field has grown tremendously since 2017, when the FDA greenlit the first directly administered gene therapy in the U.S., Luxturna. In 2018, $9.6 billion was invested globally into the field.

These first-of-their-kind therapies work by delivering modified genes directly into a patient using a vector — in many instances, the naturally occurring adeno-associated virus (AAV) — to replace missing or defective genes or add news ones to treat illness. In the case of Luxturna, the treatment replaces abnormal genes to restore vision loss.

Close to 20 companies are conducting clinical trials for AAV-based gene therapies, with three programs in phase 3 trials. What this means, however, is that facilities are needed to develop treatments at scale.

Here's what you need to know about gene therapy manufacturing.

The Costs of Manufacturing Gene Therapies

The first considerations are how the virus packages up the DNA of interest, the intended use of the product, and how much virus is needed to get the effective dose.

When thinking about viral production capability, manufacturers should consider the viral particles per liter — basically, the current quoted yield from a bioreactor. When treatments go through purification, there's approximately a 30% yield, so you can calculate how much you would get per batch. You can then determine how many doses that translates into for a specific application.

Some products have manufacturing costs in the hundreds of thousands of dollars for a large-dose indication; others are down to $1,000 for a low-dose indication. It depends on the type of treatment being considered: An eye indication would be on the low end, then a brain indication, a major organ would be pricier, and, finally, the whole body. Depending on a patient's age and weight, that can vary at the disease's onset.

Then there are facility costs. Effectively, how much time will it take to manufacture that product?

Depending on the scale of manufacturing (whether it's designed just for clinical production at a small scale), there will be an expenditure per week. Costs could be anywhere from hundreds of thousands to millions of dollars per batch at a decent-sized facility.

The Importance of Staffing

Facilities need people who understand the mechanisms of scaling up. Often, those on the lab side don't necessarily have the experience or tools to do this. The right people will know to control all the important factors, such as maintaining molecular scale conditions as purification processes are boosted along with the bioreactor process. If staff members aren't experienced in manufacturing, they can make incorrect assumptions, and this can present challenges or failures in that scale-up process.

The Timeline from Building a Facility to Rolling Out Product

Typically for these facilities, design can take six months, while construction could take nine months. You then should establish all the systems to operate in a good manufacturing practice (GMP) environment, which could take anywhere from one to two years. You may run into issues in small-scale manufacturing that require further development to support the large-scale processes occurring — maybe a technology isn't available, or you need to use a different configuration for clarifying your materials. Most often, three years elapse between deciding to build a facility and manufacturing a product that meets all the requirements.

Smaller-scale systems set up for clinical use could be built faster, like when you have existing infrastructure for a GMP operation. If you leverage other companies' capabilities to validate the equipment before the facility is constructed or hire people to build all the business process systems needed to operate your GMP facility, these can also speed things up. In general, a good estimate is three years in advance of the product launch.

The Typical Process of Manufacturing a Product

Usually, it takes a few weeks to expand the cells to get them to where they need to be. Virus expansion may be needed as well to produce enough to support a large-scale batch. Viruses must be applied to the main body of cells in a bioreactor to produce the virus containing the gene of interest. Potentially, the cells may need to be broken open to release the virus, then the addition of an endonuclease to break down the host cell DNA. You may need a depth filter to remove cellular debris. Then, one or two chromatography steps are needed to purify the viral particles adequately. Finally, there's a tangential flow filtration to exchange the buffer to a final formulation and a concentration adjustment. The final step is a 0.2 micron filtration to create your bulk drug substance.

Typically, your bioreactor time is in the order of a week. Purification is two to four days, depending upon the complexity of the process and how you spread it out. You'll need to organize the facility, maximize the turn rate, and support that production as needed for your process.

The Risks Associated with Non-Optimal Purification

Without good purification, immunogenic responses to impurities may occur in the process. But these are vaccine-type processes. There have been many instances of administering vaccines with cellular impurities without causing any severe adverse events to generate the vaccination effect. Good practices will keep microorganisms, endotoxins, and other contaminants out, but it's unclear whether impurities from the process have a significant impact on the patient. No company wants to risk that. That said, companies want to isolate the viral particle and minimize the cellular impurities as best they can.

The Most Common Reasons for Batch Failure

In addition to the manufacturing process, there are also processes to prepare the systems for production. Leaks in single-use disposable systems or failures in the preparation or sanitization could cause batch failures. There are some points of variability in the manufacturing where if you changed the seed stock, the operating conditions you used in the past may no longer be optimal due to the variability in the assay that's used to measure viral concentration. So you may need to have additional controls in place — which may not be apparent when you're working on a small scale — to allow you to process robustly and adapt for changes in the viral seed stocks in your process.

Generally, good visibility (real-time viral concentration data of the process) doesn't occur during processing, so you must eliminate variability as much as possible when you're applying the virus to the main bulk of cells. This ensures consistency in production.

How Many Runs Can a Facility Handle in a Year?

It depends on the turn rate of your longest process, which is typically the bioreactor. If the process is seven days, you can run a batch every week. If it's longer than that, and you can staff the bioreactor to support a seven-day-a-week operation, batches can be run in eight or nine days. An eight-day cycle without weekend shifts could produce product every two weeks. Basically, the bioreactor sets the turn rate of the facility itself.

A small-scale, 50-liter bioreactor may create enough product for years of supply for a particular indication with a small dose. For indications where you're treating larger organs or the entire body, you could get into a situation where it's batches per patient, not patients per batch. It comes down to the sizing of the operation according to production needs.

Challenges Facing Gene Therapy Manufacturing

Currently, the industry lacks experienced people, and companies are learning manufacturing alongside the companies they're providing the service to. It's not like immunoglobulins, where there's a 30- to 40-year experience base. For these companies, it comes down to the specific talents they have, and they must pay attention to the details — not doing so shows up in higher failure rates and more operator mistakes when manufacturing products.

The Pros and Cons of Manufacturing In-House versus Outsourcing to a Contract Manufacturing Organization (CMO)

Manufacturing in-house is ideal, but it doesn't make sense in some cases. For example, if you have one batch producing many units of product and you can support your entire clinical testing with a few batches, that's better suited to a CMO. If building your own facility in-house, you need the right people to set it up and operate it in a GMP fashion. That gives you control of the schedule, and if you have indications that need many batches to support your clinical trials, doing it yourself may be a cost advantage. Many companies start off with a CMO, then switch to building their own facilities to support phase 3 and commercial production.

Some companies don't want to build their own facilities and instead hire a contract manufacturing organization (CMO) to build a dedicated line while still maintaining control of the manufacturing schedule. This way, they don't necessarily have all the overhead of manufacturing facilities. A thorough financial analysis can help you determine which avenue you can support that can also meet your timeline. Many companies now tell potential customers they're scheduling production in 18 months, but you'll need to strategize regarding the path you'll take and how you'll manage the risks to your timeline.

Can the Industry Handle All the New Launches?

To meet demand, lots of expansion is needed, both in internal manufacturing and at the CMOs. Timelines can possibly be shortened to get a manufacturing capability established for a reduced cost. For example, instead of building your own water system, buy buffers that are manufactured elsewhere. Because they can drive up item costs the could delay your facility from becoming operational. If you push them off, you can get up and operational quicker. Companies can also save time by leveraging CMOs where it makes sense, such as in-filling lines where there may not be a constraint in capacity.

The Biggest Challenges that Need Resolving in the Next Five Years

The largest challenge is establishing robust processes to manufacture products, but there's a lack of in-process measurements that provides a good picture of what's going on. While monitoring a manufacturing process, I look at all the data available from the process. Is it telling me the same story or not in terms of that process? Are you monitoring the media you're using to ensure you've got the right nutrient levels? Your media may be producing lots of cells, but maybe those cells are consuming a key item in your viral production. There's a lot of complexities. If we can put in the necessary controls for that robust production so it's repeatable, that will solve the biggest challenge in the consistent manufacturing of products.

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ABOUT THE AUTHORS

EDWARD LANPHIER

Edward Lanphier was the Founder, President and CEO (1995-2016), and Chairman of the Board (2016-2017) of Sangamo Therapeutics. Previously, he was the Executive Vice President of Commercial Development at Somatix Therapy Corporation, a first-generation gene and cell therapy company. He is also currently a Board Member of the Buck Institute for Research on Aging since 2011.

ED PEZALLA, PHD

Dr. Edmund Pezalla currently works as an independent consultant. He is a leading payor expert consulting for pharmaceutical and device developers and manufacturers on public policy, health technology assessment, and value frameworks. He is also active on several policy working groups. Prior to this, he was the Vice President for Pharmaceutical Policy and Strategy in the Office of the Chief Medical Officer at Aetna from 2007 to 2016.

LANCE WEED

Lance Weed was most recently Vice President of Operations for uniQure, where he designed, built, and established operations for the largest gene therapy manufacturing facility using 100% single-use disposable processing systems. Prior to his role at uniQure, Lance worked for BioVex as Vice President of Operations, where he designed, built, and established operations for an oncolytic virus manufacturing facility, which was purchased by Amgen in 2011 for $1 billion.