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What to Consider When Conducting a "Make vs Buy" Analysis for a Cell & Gene Therapy Facility

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Research in the cell and gene therapy industry is generating some of the most promising treatments in recent medical history. In just a few decades, this cutting-edge concept has become a practical solution for not just a treatment, but to potentially cure formerly untreatable diseases. These therapies have the potential to treat patients in many ways including delivering a specific gene to the patient's cells or by transplantation of human cells to replace or repair damaged tissue and/or cells. In today's market, cell and gene therapy companies are making significant headlines, however, these companies are facing numerous challenges.

Manufacturing is one of the biggest obstacles for cell and gene therapy companies, as the technology required to scale up from the laboratory must be tailored specifically for each product. Due to the cutting-edge nature of cell and gene therapies, equipment and facilities must be scaled up using custom and innovative solutions. With almost no room for error or delay, how can cell and gene therapy companies create a manufacturing process robust enough to meet demand?

Long before a product has achieved regulatory approval, a solution is needed for scaling up the process from the lab to a GMP facility. This process must be approached strategically in order to deliver life-saving therapies to the patient population in a cost-effective manner. There are two options companies are presented with as they prepare to bring new products to market: contract scale-up and commercial production to a CDMO (Contract and Development Manufacturing Organization) which referred to as "buy"; or invest capital to design and construct an in-house GMP facility, referred to as "make".

As leadership, investors and technical operations advisors begin to plan for clinical and/or commercial production and decide between "Make vs Buy," there are several key questions that must be addressed:

- Patient Population
- Dose per Patient
- Scale-up Timeline and Yield Projections
- Product Pipeline
- Production Throughput (Both at regulatory approval and for product lifecycle)

Should you use a CDMO or build your own GMP Facility?

In order to accurately assess and answer these questions, key stakeholders must collaborate with R&D, regulatory, expert advisors and market research. The first step in completing a Make vs Buy analysis will be to establish a robust GMP process. Research & Development needs to develop a GMP process that can be used to estimate production costs including equipment, facility space, and raw materials. Once the process has been defined, an estimate and timeframe for facility production can be developed, or alternatively, it can be used to determine the cost and timeframe of outsourcing production to CDMOs. The ultimate goal is to compare the cost per dose for each option, this data can then be used to determine the ROI of building a GMP facility versus outsourcing to a CDMO.

Manufacturing In-House Considerations

If a company is using a unique and proprietary manufacturing technology to develop their product, outsourcing may not be feasible and may require the construction of a facility. In-house manufacturing is attractive to investors because it allows for protection of intellectual property, greater flexibility and lowers long-term costs. It may also differentiate your company in the cell and gene therapy industry and increase company valuation. In-house manufacturing gives the company the ability to own the process from start to finish. Owning a set method and product, like specific vectors, proves the ability to execute repeatedly and create a pipeline of products.

It is important to note that time is of the essence when bringing a product to market and significant upfront capital may be needed for "build." In some cases, it makes sense to build a phase appropriate manufacturing facility if a suitable CDMO partner or an ideal production slot is not available.

Outsourcing Manufacturing Considerations

When it comes to outsourcing the development of cell and gene therapy products, concerns often revolve around the process and whether it can be performed correctly and in the required timeframe. However, it is difficult to plan for commercialization in these early stages without the knowledge of what exactly will be required to scale, what launch quantities will look like, or what the market penetration will be.

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>
> –Joe Neroni, Director | Project Farma

In some cases, it makes sense to “buy” if there are concerns about upfront capital, time or resources needed to “build”. “Buying” can be an attractive option to maintain focus on R&D instead of start up and build out of a GMP facility. If outsourcing becomes the sensible option, early interaction with a CDMO is key; the timeline from contract negotiation to product release can take up to twelve months. Early strategic communication is important in order to establish realistic timelines for development and manufacturing. An experienced partner with auditing and quality agreement establishment experience is critical. With the rise of advanced therapies, the few existing and established manufacturers are experiencing a bottleneck and have become backlogged. Thus, setting a realistic timeline and obtaining a production slot should be priority number one. Additionally, continuously monitoring the market for CDMO capacity is highly recommended.

After solidifying a contract and timeline, a detailed and efficient technology transfer plan needs to be put in place to ensure a smooth transition to the manufacturer. It is also important to note that if you outsource in the early development stages, you may face a similar decision later to continue to outsource or bring manufacturing in-house.

Conclusion

With an increased demand for the discovery and expansion of cell and gene therapies, a new set of unique industry challenges are arising. Many companies are finding that the best solution is to use a combination of both in-house and outsourced manufacturing processes to swiftly bring their products to market in the most efficient and cost-effective way possible.

About Project Farma

Project Farma is a patient-focused consulting firm providing business strategy, project management, validation, engineering consulting services with a proven track record in cell and gene therapy. We partner with cell and gene therapy organizations in addition to healthcare, pharmaceutical, bio-pharmaceutical companies to support finding ground-breaking treatments and solutions. Our cell and gene therapy clients include early stage startups, established advanced therapy companies, medical institutions and CMOs/CROs. We are committed to helping advance cutting-edge medicines by leveraging our deep knowledge and broad experience in cell and gene therapy to meet any new challenges.

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Precision ADVANCE, a collection of interconnected services and complementary teams, uniquely focuses on the complexities of clinical, regulatory, manufacturing, and commercial needs to successfully bring cell or gene therapies to market.

Connect with one of our experts. Contact us at **precisionadvance@precisionmedicinegrp.com.**
To learn more about Precision ADVANCE, visit **precisionmedicinegrp.com/advance.**

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