MANUFACTURING SUPPORT | PROCESS DEV & COMPARABILITY | ANALYTICAL DEVELOPMENT | QUANTITATIVE MODELING | **QUALITY SYSTEMS** | DEVICE DEVELOPMENT

REGULATORY SUPPORT | PRECLINICAL DEVELOPMENT | PROJECT & PROGRAM MGMT | MARKET RESEARCH | INTELLECTUAL PROPERTY | **FINANCING & DILIGENCE**

Financing & Diligence in Cell Therapy

Why DHC?

- EXPAND CLIENT BANDWIDTH
- **PROVIDE ADDITIONAL TECHNICAL EXPERTISE**
- SOLVE EXISTING PROBLEM (REMEDIATE)

The Ask

CMC is frequently on the critical path for cell and gene therapy product development timelines. A venture capital firm requested a rapid evaluation of CMC Phase 3 readiness for a cell therapy asset in which they were considering investing.

DHC's Approach

In this case, the review of the asset had eight components. The review took into consideration Phase 3 readiness as well as next steps that would be necessary for suitability for BLA (Biologics License Application). Over the course of a fast-tracked three week review, DHC performed:

1. A process-trending and reproducibility analysis of the company's manufacturing process based on historical records of previous batches in Phase 1 and Phase 2.
2. A raw material suitability analysis, considering the materials' fitness for both late stage and commercial manufacturing.
3. A review of the facility's QMS (quality management system), in addition to projecting forward to identify future gaps in commercial readiness.

In many cases, Quality Systems support is not a stand-alone project, but instead an element that appears to varying degrees throughout another project. In the case of this example, we see a QMS-readiness review making an appearance: both conceptually, and as part of a follow-up on-site audit (#3 & 7).

4. An analysis of the facility's qualification status and validation master plan.
5. A review of the development status of critical in-process and release assays used to characterize the product.
6. A review of all recent FDA correspondence with a particular eye to any potential issues that could impact Phase 3 initiation and/or BLA approval.
7. An on-site quality and technical audit to determine Phase 3 readiness and PAI (pre-approval inspection) readiness of both the manufacturing facility and quality management systems.
8. The writing/delivery of a final report summarizing all identified risks to Phase 3 readiness. Within the report, each element was given a risk rating (critical - significant - moderate - minor) which takes into account anticipated time and a rough cost estimate for remediation if necessary. Recommended timing and approach for remediation were provided for each item. Additional recommendations were provided regarding next steps for items with little risk for Phase 3 readiness, but that would require remediation prior to BLA.

The Impact

Within three weeks of start, the client had in hand a report enabling them to make an informed investment decision and providing a post-investment roadmap to Phase 3 and commercial CMC readiness.

Next/Concurrent Steps

- Quality Systems (QMS gap filing)
- Regulatory Support