PCT CELL THERAPY SERVICES

CASE STUDY

TITLE: Targeted chemistry, manufacturing and controls (CMC) assistance to accelerate clinical trial initiation

CLIENT: A biotechnology company developing an *ex vivo* cell expansion system to manufacture hematopoietic stem/progenitor cells for transplantation to facilitate blood cell recovery following high dose chemotherapy for a variety of disorders.

OPPORTUNITY:

Client had developed an *ex vivo* expansion strategy requiring the use of fetal bovine serum (FBS) and a multi-cytokine cocktail to optimize expansion of the target cells. In a pre-IND meeting, the FDA advised Client that a final wash procedure with demonstrated clearance of residual cytokines and FBS was required to allow release of final product for administration to patients.

Client did not have the internal expertise or equipment to define, develop and qualify suitable assay test methods. Furthermore, the set up and execution of such a program internally were prohibitive, since:

- Initiation of a clinical trial was required in a short time-frame to generate data to assist Client with a planned finance round.
- The time to recruit staff and execute the program was estimated to be inconsistent with Client's timeline.
- The financial investment associated with the purchase of the required equipment and the employment of additional staff of suitable experience was significant for the finite program required for the IND.

SOLUTION:

Client engaged PCT to identify, develop and qualify appropriate target readout assay methods of suitable sensitivity to demonstrate that Client's final wash step adequately cleared residual cytokines and FBS for manufacture of final cell products. PCT utilized its significant experience in the development, validation and execution of GMP manufacturing processes, assay test methods and controls along with the presentation of such to the FDA.

In a period of less than two months, PCT:

- Identified four critical molecules representative of the residuals for which FDA had requested clearance data, along with suitably sensitive test methods for their detection.
- Compiled and executed protocols for the development and qualification of the assay test methods.
- Compiled and executed a protocol to demonstrate the effective clearance of residual cytokines and FBS by the final wash step.
- Provided Client with final reports detailing the above development, qualification and clearance studies that were submitted directly to FDA as part of their CMC package.
- Enabled Client to initiate the clinical trial on schedule.

CONCLUSION:

Using an integrated solutions approach, PCT leveraged its significant experience in the development, validation and execution of GMP manufacturing processes, assay test methods and controls, along with the presentation of such to the FDA, to enable Client to cost-effectively maintain their timeline, initiate their clinical trial and meet their subsequent financing goal.

PCT is a Client-focused contract development manufacturing organization (CDMO) supporting the development and commercialization of cell therapy and regenerative medicine products through specialized services tailored to address the unique scientific, technical, manufacturing, Quality, regulatory and financial challenges of our diverse Client base. Our services include cGMP-compliant manufacturing, storage and distribution of clinical products, cell and tissue collection, processing and storage, process and product development, engineering and innovation, regulatory consulting, facility design, validation, and due diligence evaluations.

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