

**TARGETED CHEMISTRY, MANUFACTURING AND CONTROLS (CMC) ASSISTANCE
TO ACCELERATE CLINICAL TRIAL INITIATION: A Case Study**

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, FDA advised the 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.

The 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 the client with a planned finance round and the cycle time to recruit staff and execute the program was estimated to be inconsistent with its 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: Progenitor Cell Therapy (PCT) has 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. Therefore, client engaged PCT to identify, develop and qualify appropriate target readout assay methods of suitable sensitivity to demonstrate that the clients final wash step adequately cleared residual cytokines and FBS for manufacture of final cell products.

In a period of less than 2 months after contract signing, PCT:

- Identified four (4) 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 the client to initiate the clinical trial on schedule

RESULT: Using an integrated solutions approach, PCT leveraged its significant experience to enable the client to cost-effectively maintain their timeline, initiate their clinical trial and meet their subsequent financing goal.



Progenitor Cell Therapy is a client-based cell therapy services company that supports the development of cellular therapies by providing cGMP-compliant cell manufacturing and consulting services that address regulatory, financial, technical, process, and quality system strategies. PCT provides a full spectrum of support including process and product development, consulting, validation, due diligence evaluations, tissue collection, processing, and storage, product manufacturing, distribution and transportation.