CASE STUDY

EMERALD CLINICAL TRIALS

# Emerald Clinical Helps Biotech Deliver Cell and Gene Therapy Study with Best-in-Class Data

Early risk identification and comprehensive quality management plan ensured best possible outcome.

## SITUATION

Neoantigen vaccines have appeared only recently as emergent immunotherapy and show great promise. Biopharmaceutical companies developing these drugs often have few resources and no experience in conducting the unique and complex clinical trials associated with cell and gene therapies (CGT)—which are very different from those conducted for traditional drug therapies. With limited patient populations, manufacturing issues, novel medical procedures and specialized equipment, these studies offer multiple challenges. Success requires critical efficiency, accuracy and safety measures.

Emerald Clinical was contracted by a Biotech company to conduct a Neoantigen Vaccine for Solid Tumor study in China and provided study strategy, Quality Management System (QMS) setup, clinical operations and vendor oversight. With our experienced CGT team and over 20 years of trial experience in China, we were a good fit to ensure the best possible outcome for this CGT trial.

## CHALLENGES

Protocol standardizations were lacking as well as clearly established standards for conducting apheresis of Peripheral Blood Mononuclear Cell (PBMC), identification techniques of neoantigen, optimum dosing regimen and evaluation of cellular immune response. Critical quality control issues around the cumbersome and time-consuming neoantigen production process (10–12 weeks) required careful attention.

Recruitment difficulties included a high screen failure rate and an insufficient target patient pool due to a very stringent protocol. The study timeline was being negatively affected by these challenges.

## Highlights At A Glance

- Evaluated all potential risks considering primary and secondary study endpoints
- Developed comprehensive Quality Management System before any site initiations
- Prepared backup plans for all key technical procedures and study start-up steps
- Identified additional target patients leading to on-time recruitment

## SOLUTIONS

Initially Emerald Clinical worked with the sponsor to identify risk areas and evaluate the potential impact on the protocol endpoints. A comprehensive QMS plan supported by standardized operational procedures was finalized before initiating any sites. With a step-by-step approach, concerns from both sites and IRB / IEC authorities were addressed, and approval was granted to begin the study.

A comprehensive list of potential vendors providing next generation sequencing (NGS) tests was created and a keen oversight process put in place. On-site audits were conducted for key operational processes including NGS testing, Elispot and aphaeresis procedures.

To expedite and enhance recruitment, Emerald Clinical detected pitfalls in the protocol that could have a negative impact on the overall study timeline and implemented the following actions:

- Expanded the resource of sampling for NGS tests, which included a wider set of patients who were able to provide fresh tissue samples
- Prepared a backup plan for all key technical procedures and important study startup steps, ensuring that the personalized needs of the patients could be met utilizing the best vendors
- Worked collaboratively with individual Principal Investigators to further identify potential target patients and the various tests needed for each, thereby shortening the turnaround between the patients' clinical visits and the manufacturing of their personalized neoantigen

## RESULTS

Emerald Clinical's long-term development plan considering the primary and secondary endpoints made it possible to be nimbler in addressing any issues. With a QMS in place, startup was initiated efficiently and effectively. A broader identification of potential patients led to on-time recruitment. The study was delivered with best-in-class quality data that was critical for submission to regulators. Emerald Clinical is proud to be on the forefront of these new and innovative treatments that can lead to a longer and higher quality of life for patients.

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