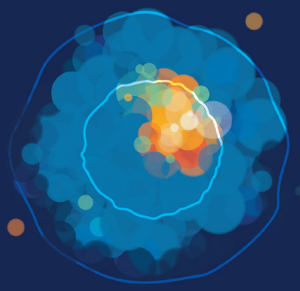
Case Study:

GENE THERAPY TRIAL FOR PATIENTS WITH BCG UNRESPONSIVE NON-MUSCLE INVASIVE BLADDER CANCER



A Phase III, Open Label Study to Evaluate the Safety and Efficacy of an Adenovirus-Based Gene Transfer Vector for Treatment of Non-Muscle Invasive Bladder Cancer

157 PATIENTS

33 SITES

1 COUNTRY

SERVICES PROVIDED

- Project Management
- Biostatistics
- Site Activation and Maintenance
- Clinical Monitoring
- Interactive Response Technology (IRT)
- Clinical Safety
- Data Management
- Medical Writing

CHALLENGES

- Many sites lacked experience in gene therapy trials and required operational support to ensure adequate equipment and processes, in addition to preparing and submitting to Institutional Biosafety Committees (IBC)
- Site staff and patients expressed concerns regarding the safety implications a viral vector for gene therapy
- As the gene therapy trial required longer survival follow-up, the FDA BLA submission was created from an interim data cut

SUCCESSES

- Medpace activated all sites and completed patient enrollment within the projected timeline
- The interim data cut and Clinical Study Report were successfully submitted to the FDA
- The FDA inspected 4 investigational sites with no findings and the Investigational Product was approved

CRITICAL MILESTONES

- Site Selection
- Site Startup
- Enrollment
- Interim Data Cut
- Clinical Study Report

THE SOLUTIONS

- Extensive site qualification was completed to assess site and investigator experience, infrastructure, SOPs, and capabilities
    - For sites inexperienced with gene therapy trials, Medpace completed documents and responded to questions from the IBC, including consulting on Investigational Product and biosafety SOP development required for IBC approval
    - Medpace worked with the sponsor to supply biosafety cabinets and additional equipment to sites
- Medpace collaborated with the sponsor to develop educational materials for the site staff and patients regarding the handling and safety of the investigational product, which included preparation guidelines and proper sanitation procedures, providing reassurance over the safety concerns of the Investigation Product