PRODUCT PROGRAM MANAGEMENT

Development & Validation of Bioanalytical Methods for a Viral (AAV) Gene Therapy Product

THE NEED

A preclinical-stage gene therapy company advancing the development of a AAV gene therapy product for a genetic disease affecting neurological and cardiac functions required the development, qualification and/or validation of a full complement of bioanalytical (bioA) test methods to support their non-clinical / toxicology studies and Phase I clinical program.

Their requirements were extensive, necessitating the engagement of numerous development partners and contract research partners (CROs). Primecore was tasked to facilitate the establishment of 'fit-for-purpose' bioanalytical methods to detect and quantify multiple analytes (proteins, nucleic acids and whole cells) in several test matrices (serum/plasma, CSF, tissue lysate, tissue biopsies and peripheral blood mononuclear cells), utilizing conventional and novel analytical platforms (qPCR / ddPCR, in-situ hybridization using RNAscope™, ELISA, ELISpot, SIMOA™, MSD-ECL immunoassay, 'Simple Western' Jess™ by Protein Simple).

THE APPROACH

Primecore drafted a phase-appropriate bioanalytical development and qualification plan that aligned with FDA guidelines for each bioanalytical test method. Primecore also implemented a communication plan to manage technical meetings between the client and the CRO partners, and between the client's own cross-functional teams, comprised of analytical SMEs and managers in pre-clinical development, clinical research operations, and quality.

THE SOLUTION

- Fostered communication and knowledge transfer between client and development partners with weekly review and discussion of assay-specific development plan, raw materials and technical / scientific data generated.
- Implemented cross-functional team updates with a weekly review of the BioA program as a whole, permitting assessment of program progress and identification of risks and escalations.
- Initiated monthly governance meetings enabling executives to track progress, monitor program spend and execute on critical decisions affecting pre-clinical and clinical studies.

THE RESULTS

- ***Primecore's team was able to swiftly take control and steer the client's BioA program, bridging the knowledge and experience gaps, whether technical (assay specific), quality (ICH and FDA guidelines) and/or program management (control of schedules and timelines). This provided definition, direction, and momentum to the BioA program and averted the risk of a significant delay.***
- ***Efficiency of the client's internal BioA program management team was enhanced and output from cross-functional team meetings was increased. Progress was visible on a weekly basis.***
- ***The client needed urgent help in managing the BioA program and Primecore was able to shift SME resources to manage this critical workstream.***

PRIMECORE