PRODUCT PROGRAM MANAGEMENT

Program Management of New Gene Therapy Pipeline Products

THE NEED

An innovative start-up company had their focus on advancing a rAAV gene therapy for a debilitating genetic disease affecting both neurological and cardiac functions. Efforts required a CMC specialist to lead and manage their new product pipeline programs. The therapeutic candidates, in early concept stage, required a development strategy enabling the evaluation and selection of optimized early phase products for IND-enabling pre-clinical studies.

THE APPROACH

- A development strategy was created delineating the development roadmap and resources required to generate non-GLP rAAV material to advance early-stage candidates through proof-of-principle (POP) and proof-of-concept (POC) studies.
- A development plan and program schedule was prepared outlining the timelines, cross-functional interdependencies, potential bottlenecks, and risks.
- A project management structure was implemented, enabling the program to be governed, managed, and monitored to achieve the key program objective – the selection of 2 promising candidates supported by provisional patent filings, for IND-enabling CMC activities within 12 months.

THE SOLUTION

The presentation of the pre-CMC development roadmap enabled the client to prioritize the order in which the selected candidates should be developed and reach a decision to outsource time-critical activities to a CDMO partner.

- Primecore led the evaluation, selection, and engagement of the CDMO partner; prepared the request for proposal (RFP) and reviewed the proposals generated; seven CDMO candidates were evaluated,
- Primecore navigated contract negotiations and facilitated the tech transfer process, ensuring raw material, plasmids and supporting analytics were in place for the partner to manufacture sufficient quantities of early pre-clinical grade rAAV for POP (2-5x1013 vg) and POC (2-5x1014 vg) studies,
- Primecore managed the project meetings between the client and CDMO partner offering both management and SME expertise.

THE RESULTS

- *A CDMO partner with rAAV experience and scope for the manufacture of GLP and cGMP grade material was identified and engaged within the time frame indicated.*
- *Development of two lead candidates were parallel tracked, enabling delivery of multiple small-scale batches for POP studies, which enabled a decision on candidate selection for POC studies earlier than anticipated.*
- *Primecore quickly added value for the client by rapidly expanding the development portfolio with prioritized candidates.*

PRIMECORE