Consultancy

Regulatory Strategy

Scientific Input

Rapid Proof-of-Concept for Novel Drug Treatment

NEED

- Emerging US biotech company requiring integrated program management of outsourced IND-enabling preclinical studies for first lead compound
- Assistance with documentation for filing IND
- Testing human safety and potential efficacy of novel small molecule drug
- Financing dependent on expeditious development

APPROACH

- Celerion program director led integrated project team
- Assisted the client with development plan and managed all outsourced studies
- Provided FDA regulatory guidance and prepared IND documentation
- Developed and validated challenging bioanalytical assays
- Biomarker assays optimized, and validated for deployment
- Phase I Multiple Ascending Dose study design incorporated biomarker of target enzyme activity
- Samples collected and processed at multiple clinical sites with biomarker assays performed at centralized bioanalytical lab
- Coordinated complex sample collection and processing logistics
- Provided flexible scheduling of biomarker analyses to support multi-site proof-of-concept Phase II studies

BENEFITS

- Completely outsourcing drug development program enabled the client to focus internal resources on Discovery needs
- Preclinical work completed and IND submitted within challenging investor-driven timelines
- Biomarker results from Phase I MAD study demonstrated proof-of-concept, enabling the client to raise additional financing
- Biomarkers translated from lab bench to clinic with “fit-for-purpose” assay validation; qualified assays available for the client to carry forward to Phase II and III
- Knowledge acquired from program was leveraged by the client to facilitate even faster drug development of follow-on molecules