

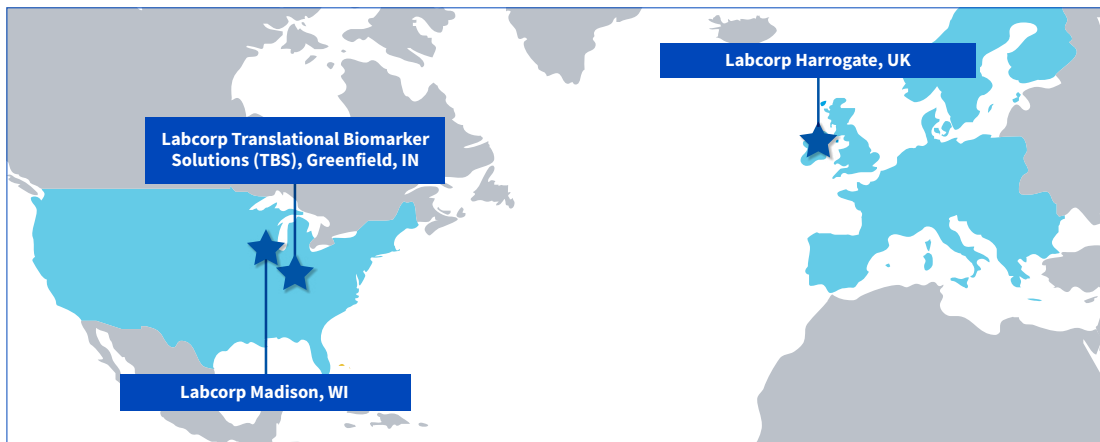
CASE STUDY

Coordinating global capabilities to support a gene therapy targeting a rare neurodegenerative disorder

An emerging biotech company was searching for an experienced partner to help their early-phase efforts to advance a gene therapy that targeted a rare neurodegenerative disease. Learn how multiple teams across Labcorp collaborated to support the biotech's successful IND with GLP toxicology and biodistribution study and CMC services for the sponsor's unique program.

Delivering a suite of solutions and uniting expertise

As a small company that was rapidly expanding, the biotech looked to Labcorp to provide scientific and regulatory guidance for their initial gene therapy. Working across multiple Labcorp sites, each with specific services and expertise, Labcorp helped them advance their gene therapy from preclinical work to the clinic.



Labcorp Madison, WI

The Labcorp site in Madison, WI, first performed an IND-enabling toxicology and biodistribution study for the sponsor's viral gene therapy, which involved intracisternal administration, a challenging dose route. Along with the Labcorp Early Phase Development Solutions (EPDS) group providing scientific and regulatory strategy support, the study team provided support with study design and data interpretation, and helped provide support to advance the sponsor's program and function as an extension of the sponsor's team. The study team hosted the sponsor at the Labcorp Madison site to review the toxicology operations in-person, a visit that helped the sponsor gain an understanding of the level of effort needed to support a complex study.

Labcorp Harrogate, UK

The sponsor also visited with the BioCMC team at the Labcorp Harrogate site, which performed the qPCR biodistribution analysis of the study performed in Madison. The Harrogate team supported the biodistribution analysis through their expertise in qPCR assay development and validation. The site's Microbiology team provided method development and validation to support viral shedding analysis and the Virology team provided potency testing of the test article batches.

Labcorp Translational Biomarker Solutions (TBS), Greenfield, IN

To better understand transgene expression and enzyme activity, tissue expression analysis was performed by the Translational Biomarker Solution team at the Labcorp site in Greenfield, IN. As an exploratory lab, they follow robust quality principles, allowing for rapid results that inform preclinical decision making, while thinking ahead to prepare for transfer to a CAP/CLIA or GMP environment, if needed.

Working with multiple tissue types from the study, the TBS team worked with the sponsor to create a method that was accurate and qualified for each tissue type. The team then processed samples from the study performed in Madison and measured enzyme activity. Based on this data, the sponsor was able to make key decisions to advance the program.

To complete their IND submission, the sponsor needed a rapid turnaround to fulfill an FDA data request. Even though the holiday season in December was approaching, the project management team coordinated cross-functional efforts to support an on-time data submission.

Recognizing the results of a strong partnership

With the support of Labcorp from multiple teams across the U.S. and UK, the sponsor received Fast Track Designation for their therapy, which is currently progressing in the clinic. The client continues to rely on Labcorp for nonclinical programs to support their rapidly growing product pipeline. Labcorp now has templates and processes in place to support the client and has proven to be a trusted partner for the sponsor.

Based on the success of their first gene therapy, the sponsor has continued to partner with Labcorp to help with additional gene therapies to support neurodegenerative diseases, which received orphan drug designation and a rare pediatric disease designation. Labcorp hopes the sponsors' pipeline of novel gene therapies will help bring new hope to patients where no disease-modifying therapy is currently available.

Learn more at biopharma.labcorp.com/cgt