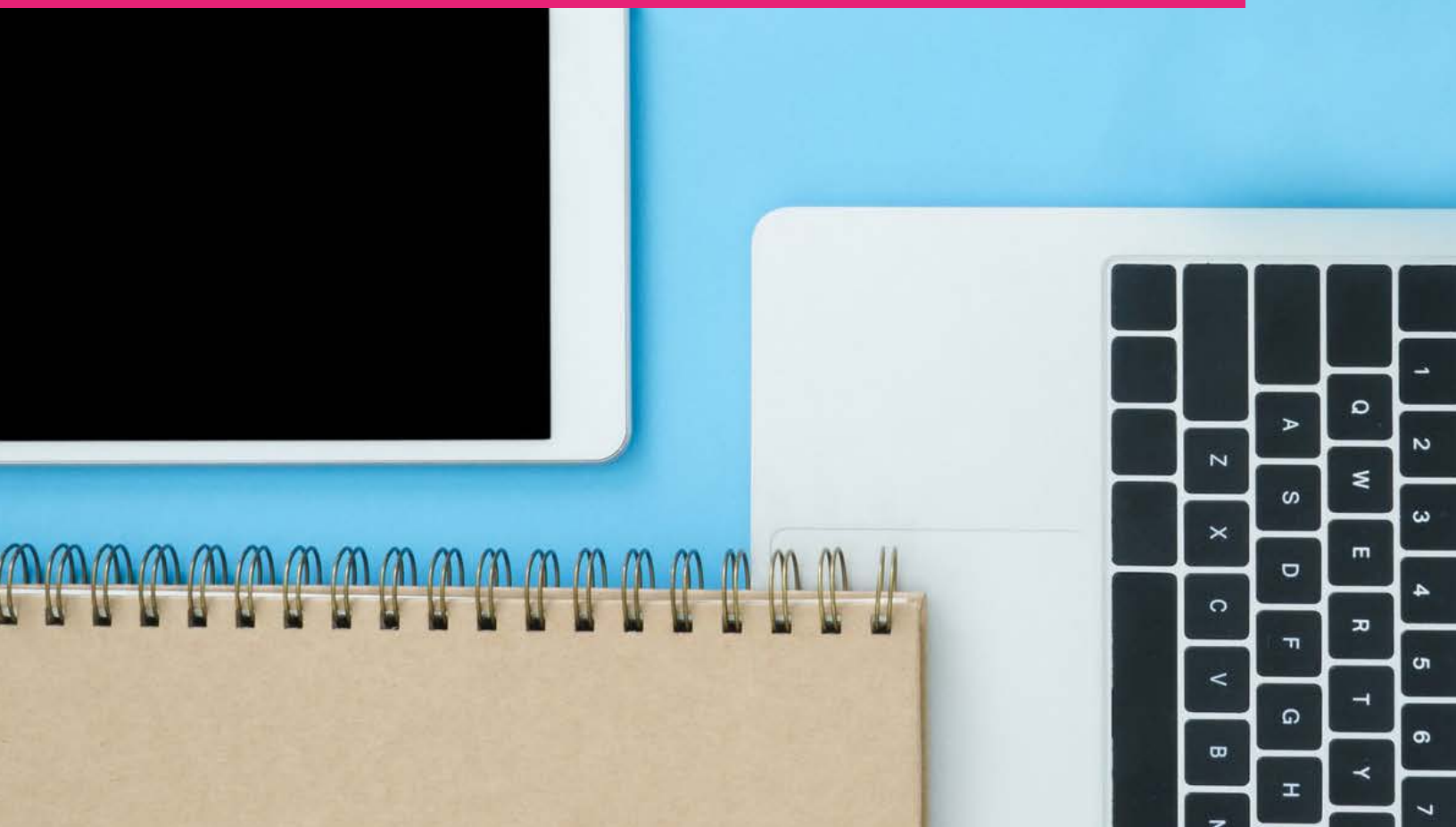
PRA Health Sciences

CASE STUDY

# Multi-stakeholder Collaboration Achieves Rapid Study Start-up for an Advanced Therapeutic



## STUDY DESCRIPTION

PRA worked with an emerging biotech firm to implement a first-in-human clinical trial using a live biotherapeutic product for a rare disease indication.

**Primary Endpoint**
Product safety

**PRA Services**
Full Service

**Drug Class**
Live Biotherapeutic

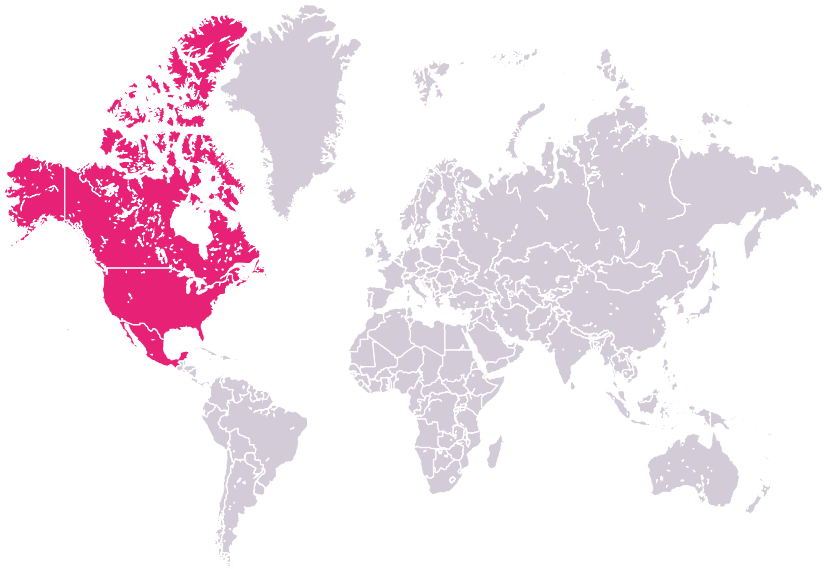
**Study Phase**
Phase I/II

**Study Duration**
12 months

**No. of Clinical Sites**
6

**Patient Population**
Healthy volunteers and adult patients diagnosed with a rare disease

**Regions**
North America



## Situation

PRA identified a lack of clinical research experience at the site level with this specific type of product categorization. Sites were confused as to the appropriate regulatory, ethics, and safety review processes required to initiate clinical study activities in which the product is a live biotherapeutic product.

## Challenges

At the time of study start-up, it was unclear at the site level about whether this live biotherapeutic product would be categorized as gene therapy for the purposes of safety, regulatory and ethics processes (e.g., requiring Recombinant DNA Advisory Committee and Institutional Biosafety Committee review, registration with the National Institute of Health). Available FDA guidance documents only address regulatory processes, and sponsors, clinical study sites, and institutions provided minimal guidance to support these processes at the site level prior to study initiation.

Improper filing and review of safety, regulatory, and ethics applications often causes back-and-forth discussions with multiple rounds of questions. This can significantly delay study start-up and prevent patients from receiving timely treatment.

## Solutions

PRA worked with the National Institute of Health (NIH) to confirm appropriate categorization of a study investigating a live biotherapeutic product. To ensure PRA was successful in initiating sites without additional delay, PRA obtained written confirmation from the NIH that stated live biotherapeutic products do not need to follow the same processes as Gene Therapy products, but still require IBC and ethics review by the site. PRA distributed the NIH confirmation to sites and worked with each site to complete applications for these committees and answer questions during internal reviews.

## Results

By proactively collaborating with all relevant stakeholders, including the NIH, PRA ensured smooth study start-up and enrollment with minimal impact to the study timeline. Experts within PRA's Center for Rare Diseases coordinated this process and supported the relationship between the biotech sponsor, clinical study sites, and Key Opinion Leaders.