

Leveraging Investigator-Initiated Trials in Rare Disease Drug Development

Investigators interested in rare disease treatment development have the opportunity to approach drug and biologic developers to obtain investigational drug supply for trials in which the investigators, typically at academic institutions, act as sponsor-investigators. Similarly, companies open to extending their product development pipelines can look to investigator-initiated trials as a mechanism to better understand the overall safety profile for their product candidates while exploring the potential therapeutic utility of their product candidates in diseases where unmet medical needs remain. So often, those needs exist in rare diseases where populations are small and investment returns are difficult to project. Drug developers deciding whether to supply investigational products to sponsor-investigators looking to explore therapeutic potential in areas of their research interests should evaluate what level of involvement to exercise over the investigator-initiated trial. We highlight some of these considerations below.

Company Considerations for Level of Involvement in Investigator-Initiated Trials

- **Availability of resources to support the trial**
 - Amount of investigational product
 - Funding for conduct of trial
 - Other trial support (e.g., administrative, monitoring plan, data management, regulatory submission assistance, training, recruitment, etc.)
- **Relationship-building between Company and Investigator and Investigator's Institution**
 - Establish a relationship that may lead to future collaboration opportunities for Company-sponsored trials
- **Opportunity to utilize trial data to support additional Company INDs, to evaluate potential for expanding product indications (in the case of approved products), etc.**
- **Desire to have:**
 - Input on proposed trial design and later amendments thereto
 - Access, where possible, to emerging data
 - Ability to publish data from the trial
 - Ownership rights in the trial data
 - Inventorship and other intellectual property rights that may arise from the trial
 - Termination rights



Ultimately, drug developers hold the decision-making power over whether to allow investigator-initiated research for their product candidates. Thereafter, the contracting process around the setup of an investigator-initiated trial and clinical supply agreement provides drug developers the opportunity to negotiate their level of involvement in the research of their candidates. In negotiating the setup of investigator-initiated research supply, drug developers often balance their support of research into what are often unmet needs with limited company resources, limited supply that may be available and any potential risks that may flow from trial learnings in the proposed disease space. As an upside, investigator-initiated trials afford developers the opportunity to extend their research reach and product development pipelines, so any interest by investigators to conduct research with industry candidates warrants consideration.