



# CLINICAL OPERATIONS AND INSPECTION READINESS CONSIDERATIONS IN COMPLEX GENE THERAPY AND RARE DISEASE TRIALS

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# TOPICS

- Role of clinical operations study lead in managing highly complex logistics of gene therapy and rare disease trials



- Key considerations in understanding site and patient expectations



- Best practices and tools for regulatory agency inspections

# THE ROLE OF CLINICAL OPERATIONS STUDY LEAD IN GENE THERAPY AND RARE DISEASE TRIALS

Clinical operations study lead plays a pivotal role in managing highly complex logistics within gene therapy and rare disease trials.

Gene therapy and rare disease trials require meticulous planning and execution due to unique patient populations, specialized treatments, and stringent regulatory requirements.

The clinical operations study lead acts as a linchpin, coordinating various aspects of the trial to ensure smooth execution and compliance with regulatory standards.



# KEY RESPONSIBILITIES OF CLINICAL OPERATIONS STUDY LEAD



**Strategic Planning:** Develop comprehensive study plans, timelines, and budgets tailored to the unique needs of gene therapy and rare disease trials.



**Logistics Management:** Coordinate patient recruitment, site selection, and drug distribution to ensure seamless operation across multiple sites and regions.



**Stakeholder Collaboration:** Facilitate communication and collaboration between cross-functional teams, including KOLs/investigators, sponsors, CROs, and regulatory authorities.



**Risk Management:** Identify potential risks and develop mitigation strategies to address challenges related to patient safety, data integrity, and regulatory compliance.

# THE IMPACT OF EFFECTIVE CLINICAL OPERATIONS STUDY LEAD



**Adherence to Trial Timelines:** Efficient clinical trial management clinical operations study lead can expedite trial timelines, bringing potentially life-saving treatments to patients that otherwise may not have any treatment options.



**Enhanced Patient Experience:** Strong clinical operations study leads keep patient experience on the forefront to ensure a positive patient experience, fostering trust and compliance throughout the trial. Example: Prescreening protocol, division of assessment on long visit days, partnering with a patient concierge service provider etc.



**Regulatory Compliance:** The expertise of clinical operations study leads ensures compliance with regulations, safeguarding the integrity of trial data and patient safety.- Begin with the end in mind

# UNDERSTANDING SITE AND PATIENT EXPECTATIONS IN GENE THERAPY AND RARE DISEASE TRIALS

Patient recruitment is often tough due to vigorous inclusion/exclusion criteria, rare nature of the disease indication and sometimes apprehension from the patients to enroll in a gene therapy study.

For the study lead, it is important to keep transparent and meaningful channels of open communication with the PI and site personnel from the very beginning for successful trial enrollment, retention, and overall trial execution.

# KEY CONSIDERATIONS IN ADDRESSING SITE EXPECTATIONS

**Specialized Expertise:** Sites participating in gene therapy and rare disease trials often require specialized expertise and resources to effectively manage patient care and treatment administration.

**Infrastructure Support:** Providing sites with adequate infrastructure support, including training, technology, and logistical assistance can streamline trial operations and ensure compliance with protocol requirements.

**Communication Channels:** Establishing clear and efficient communication channels between sponsors, CROs, and sites fosters collaboration and facilitates rapid resolution of issues or concerns.



# UNDERSTANDING PATIENT EXPECTATIONS IN COMPLEX TRIALS

**Treatment Access:** Patients participating in gene therapy and rare disease trials often have high expectations regarding access to innovative treatments and personalized care.

**Supportive Care:** Addressing patients' holistic needs, including psychological support, financial assistance, and logistical guidance, enhances their experience and promotes retention in the trial.

**Transparency and Education:** Providing transparent information about the trial protocol, potential risks, and benefits empowers patients to make informed decisions and fosters trust in the clinical research process.



# BEST PRACTICES AND TOOLS FOR REGULATORY AGENCY INSPECTIONS IN GENE THERAPY AND RARE DISEASE TRIALS



REGULATORY AGENCY INSPECTIONS  
ARE CRITICAL CHECKPOINTS IN GENE  
THERAPY AND RARE DISEASE TRIALS  
TO ENSURE COMPLIANCE WITH  
REGULATORY STANDARDS AND DATA  
INTEGRITY.



IMPLEMENTING BEST PRACTICES AND  
UTILIZING APPROPRIATE TOOLS CAN  
FACILITATE SUCCESSFUL INSPECTIONS  
AND MITIGATE POTENTIAL  
COMPLIANCE RISKS.



PROACTIVE PREPARATION AND  
COLLABORATION BETWEEN  
SPONSORS, CROS, AND SITES ARE  
ESSENTIAL FOR NAVIGATING  
INSPECTIONS EFFECTIVELY.

# KEY BEST PRACTICES FOR REGULATORY AGENCY INSPECTIONS



**Pre-Inspection Readiness:** Conduct mock inspections and internal audits to identify potential compliance gaps and address them proactively before regulatory agency inspections.



**Document Management:** Implement robust document management systems to organize and maintain trial documentation, including protocols, informed consent forms, and regulatory submissions, in a centralized and accessible manner.



**Training and Education:** Provide comprehensive training to trial personnel on regulatory requirements, standard operating procedures (SOPs), and data management practices to ensure consistency and compliance across the trial.

# UTILIZING TOOLS FOR REGULATORY AGENCY INSPECTIONS

## Implement

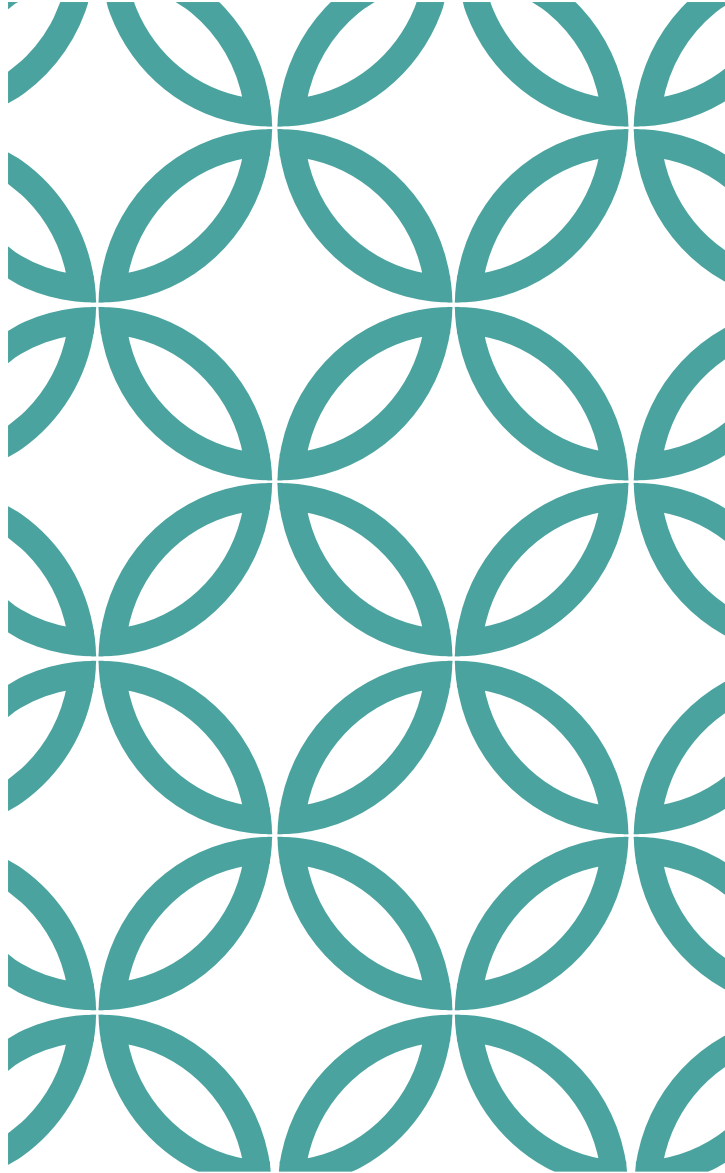
Electronic Data Capture (EDC) Systems: Implement EDC systems to streamline data collection, entry, and verification processes, ensuring data accuracy and traceability during inspections.

## Utilize

Regulatory Compliance Software: Utilize regulatory compliance software to track and manage regulatory requirements, milestones, and submissions, facilitating real-time monitoring and compliance oversight.

## Deploy

Inspection Readiness Platforms: Deploy inspection readiness platforms that offer customizable checklists, templates, and training modules to prepare trial teams and sites for regulatory agency inspections efficiently.



**THANK YOU!**

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