ACCELSIORS

# ORPHAN DISORDERS

**SPARK**


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## Accelerant™ for Orphan Disorders

Several constraints are challenging the rare disease clinical trials' planning and conduct. It requires deep expertise to find the proper trial design augmented with appropriately selected endpoints and sufficiently powered sample size, control group(s) selection, the most sensitive biomarkers, and the most comfortable trial assessments acceptable to patients involved, all this considering within highly proper ethical frames and financial constraints.

By leveraging our 20+ years of global expertise, networks, and resources, Accelsiors can significantly contribute to the success of rare disease trial planning and conduct. We can help overcome the unique challenges associated with these trials, accelerate research efforts, and ultimately facilitate the development of effective treatments for individuals living with rare diseases.

Accelsiors brings more than 20 years' experience in planning and conducting rare clinical trials on a global scale. We built our organization to succeed in these challenging indications.

We have proudly introduced the Accelerant Program™ to improve the successful outcome of our Orphan disease clinical trials. Now, we expanded this approach to most of our clinical research studies irrespective of indications. With the Accelerant Program™ we are embedding knowledge and compliance, quality, patients' perspectives, speed, agility, state-of-the-art technologies, and cost-effectiveness in our clinical trials.

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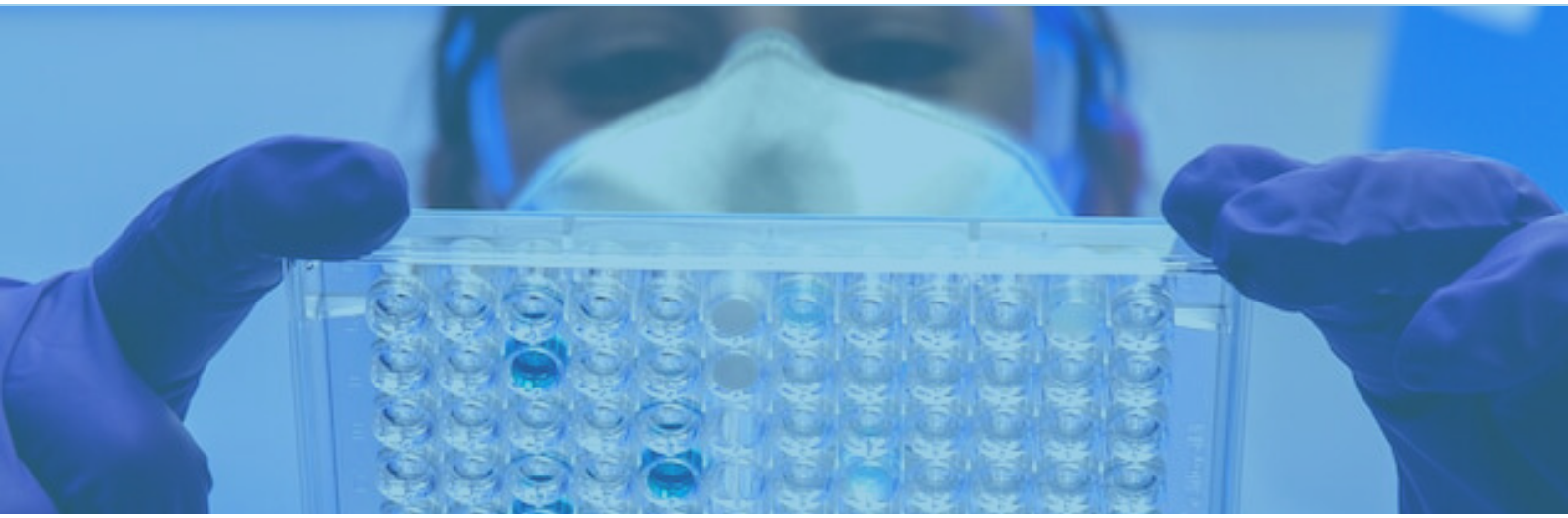

## StrongCORE Scientific™

Our dedicated and competent **StrongCORE Scientific™** team will assist our clients to find the optimal study design, relevant study endpoints, reasonable eligibility criteria, sensitive and meaningful study measurements, patients' perspectives and adequate sample size, ensuring conclusive study outcome, all of these within strongest compliance to often very rigid and demanding regulatory frames and within highest ethical standards.

Regulatory agencies play a critical role in approving and overseeing orphan disorder clinical trials. Developing regulatory frameworks that address the unique challenges of rare diseases, such as flexible trial designs, adaptive approaches, and alternative trial endpoints, can support the timely and efficient conduct of clinical trials.

*Let's consult with Accelsiors expert team members about the right regulatory pathways!*

**Knowledge**

**Experience**

**Compliance**

Accelsiors is well-versed in navigating the complex regulatory landscape governing clinical trials. We can help ensure compliance with relevant regulations, guidelines, and ethical considerations specific to rare diseases. Accelsiors can assist in preparing regulatory submissions, interacting with regulatory authorities, and addressing any unique challenges that may arise during the regulatory process.

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## QualityDrive™

Our **QualityDrive™** team will integrate and coordinate the efforts in defining, risk assessing, and controlling the critical to quality factors required to establish the right focuses of the entire study teams, building quality into the study`s design from planning stages throughout the whole lifecycle.

Quality is a primary consideration in the design, planning, conduct, analysis, and reporting of clinical studies and a necessary component of clinical development programs. The likelihood that a clinical study will answer the research questions while preventing important errors can be dramatically improved through prospective attention to the design of all components of the study protocol, procedures, associated operational plans and training.

*GENERAL CONSIDERATIONS FOR CLINICAL STUDIES E8(R1), Adopted on 6 October 2021*

Close monitoring of safety and adverse events is crucial in orphan disorder clinical trials. Our Quality Drive QbD in clinical trials solution enables focused monitoring strategy maximizing beneficial effect unlike wise traditional approach to managing monitoring. Robust safety monitoring plans, risk mitigation strategies, and clear protocols for managing potential safety issues should be in place to ensure participant well-being.

*Let's share our experience in a 1-hour complimentary telephone consultation. about most common critical to quality factors in rare disease clinical trials.*

## Building quality by Design

## AcceleROUTE™

On the accelerated route of study delivery

Patient recruitment and retention is one of the biggest challenges in executing rare disease trials. Accelsiors dedicated **AcceleROUTE™** team is trained how to find and support enrolling enough eligible patients. We employ our extensive networks and relationships with investigators, patient advocacy groups, and patient registries to facilitate patient recruitment. In the past 20+ years we have developed proven strategies to improve patient recruitment and retention and engagement throughout the trial within reasonable budgets. Accelsiors excel in project management, and we can oversee various aspects of a rare disease trial, including timelines, budgets, and resources. We gain robust experience to effectively coordinate between different stakeholders, including investigators, patient advocacy groups, regulatory authorities, and sponsors, ensuring seamless collaboration and communication throughout the trial.

Accelsiors can identify and engage clinical sites with expertise in the specific rare disease under investigation. We can assess site capabilities, assist in site initiation, provide training, and ensure proper site management throughout the trial. We monitor the trial's progress, supporting sites in ensuring adherence to protocols, data quality, and regulatory requirements.

## WideSCOPE Intelligence™

Data analytics

Machine learning

We are utilizing state of the art clinical research technologies to support our clinical trials through our **WideSCOPE Intelligence™** system. With access to advanced technologies, databases, and infrastructure we significantly enhance the efficiency and quality of rare disease trials. This includes tools for electronic data capture, remote monitoring, centralized and risk-based data management systems, CTMSs, different sophisticated communication platforms. Our communication platforms are much more than an adjusted SharePoint portal. Our communication platforms are specially designed and fully validated for communication and document exchange purposes. These systems are enhanced with multilevel access control, voting features, safe, GDPR compliant record transfer, just to mention some of the most important features. These communication platforms are utilized to support the work of different Diagnostic Confirmation Panels, Endpoint Adjudication Committees, DSMBs and others.

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## ProPATIENT™

Patient engagement in orphan disorders clinical trials is crucial for ensuring the success and relevance of the trials. Our **ProPATIENT™** SOLUTIONS are built to facilitate this process. Involving patients as active partners in the research process helps to incorporate their unique perspectives, needs, and preferences, ultimately leading to more patient-centered and impactful trials. Engaging patients early in the clinical trial process can help shape research priorities, study design, and outcome measures. Involving patients from the initial stages ensures that their perspectives are considered, and the trial addresses their most pressing concerns. Patient input can contribute to the selection of relevant endpoints, outcome measures, and patient-reported outcomes (PROs).

### Patients' Perspective

### Patients' Focus

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## We know. We care. We support.

**Unleash your global potential and accelerate success with Accelsiors — the partner who understands your world and is dedicated to making it thrive.**

To learn more about how Accelsiors can support your expansion. **Schedule a one-hour free consultation with us now.**

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