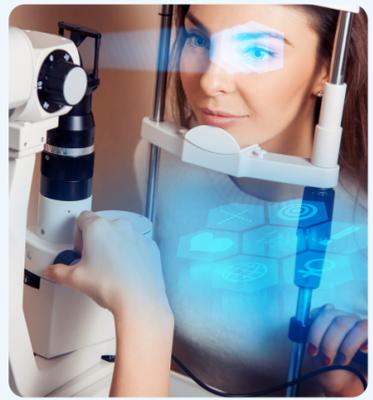


Challenges in Rare Ophthalmology Trials

Introduction

Rare disease clinical trials face a unique set of challenges due to factors like small patient populations, complex disease presentations, and limited research data. Within the realm of ophthalmology, these challenges are further compounded by the intricacies of eye conditions, specialized treatments, and the need for precise diagnostic tools.

This infographic dives into five hurdles specific to rare ophthalmic clinical trials, shedding light on what makes this field distinct.



01. Patient Recruitment and Inclusion Criteria



Scarcity of Patients

Rare diseases naturally limit the pool of eligible participants, making it challenging to recruit sufficient numbers that fit the study's criteria.



Pediatric and Family Considerations

Many rare ophthalmic diseases are congenital or hereditary, requiring trials to engage pediatric patients and their families. This involves navigating ethical complexities and addressing unique logistical needs for children.



Disease Stages and Natural History

Lack of comprehensive Natural History data complicates the identification of participants at appropriate disease stages for inclusion.

02. Defining and Aligning Outcome Measures

Non-Standard Metrics

Traditional ophthalmology measures (e.g., visual acuity) may not suit slowly progressing or degenerative diseases.

Novel Outcome Measures

Developing and validating new tools, such as low-luminance maze tests requires regulatory approval and consensus among investigators.

Pediatric Adaptations

Outcome measures must account for children's abilities to cooperate, particularly with complex imaging techniques.

03. FDA and Regulatory Engagement

Early Collaboration

Involvement of the FDA or equivalent bodies from the study's inception ensures that outcome measures and designs meet regulatory standards.



Personalized Trial Designs

Trials tailored to individual progression trajectories (e.g., ABCA4 retinopathy) are increasingly accepted, but require robust baseline data.

04. Imaging and Diagnostic Tools

Balancing Feasibility and Quality

Pediatric populations may struggle with extensive imaging protocols, necessitating simplified and less intrusive techniques.

Standardization Across Sites

Ensuring consistent image quality and data collection across multiple sites through centralized reading centers and standardized training is critical.



Use of Imaging Biomarkers

Increasing reliance on imaging biomarkers (e.g., OCT layers, geographic atrophy) demands rigorous protocol adherence and quality assurance.

05. Trial Duration and Disease Progression

Slow Progression



Many rare diseases evolve over long periods, requiring trials to span years to capture meaningful changes.

Outcome Measure Validation



Collecting sufficient Natural History data to validate imaging or other biomarkers as proxies for disease progression.



By addressing these challenges with tailored solutions—such as consensus-building, innovative designs, and meticulous coordination—rare ophthalmology trials can overcome obstacles and improve outcomes for these patient populations.



Discover how partnering with Emmes on your next rare ophthalmology trial can help you achieve your goals. Reach out to us at info@emmes.com to learn more