

Accelerate your clinical trial with our expertise in rare ophthalmic diseases

Advances in gene therapy, new biologics, and devices are changing outcomes for patients living with rare ophthalmic diseases.

Each rare ophthalmic disease on its own impacts a small percentage of the population, but the collective volume has a large impact on patients, their families, caregivers, and the healthcare system. This momentum impacts the lives of patients and creates a sense of urgency for drug developers and CROs alike.

At a time when acceleration is essential, the PPD™ clinical research business of Thermo Fisher Scientific is the right partner to advance your therapeutic development. We apply our hands-on global knowledge of medical and operational needs to quickly put your trial on the path to success.

Through access to a wide network of experienced ophthalmic research sites and professionals, our experts navigate complex global ocular trials while offering patient services that ease recruitment and increase retention.



## Dedicated rare ophthalmic capabilities in an era of increasing clinical need

Rare ophthalmic diseases - often due to genetic components - frequently have early onset impacting children and adolescents, including visual impairment and blindness.

Our strong global expertise in rare diseases, pediatrics, and gene/cell

**therapy** has collectively supported strategic offerings, solutions, and recent successes for ophthalmology rare disease trials. We can tailor unique solutions to each protocol and proactively assess challenges that may arise.

Our experts combine their ophthalmic experience with widespread global capabilities to help our clients achieve success in their clinical trials, while our patient-centric support services speed recruitment and increase patient retention by reducing site, patient, and caregiver burden.

# Our rare ophthalmology team has supported:

20+	Rare disease studies
1,793+	Patients
656+	Global sites

### We have managed and delivered on multiple indications, including:

- Fuchs dystrophy
- Optic neuritis
- Neuromyelitis Optica (NMO)
- Stargardt disease
- Sjogren
- Retinitis Pigmentosa (RD)
- X-Linked Retinitis Pigmentosa (XLRP)
- Leber's Congenital Amaurosis (LCA)
- Retinopathy of prematurity (ROP)
- Uveitis (including Uveitic Macular Edema)
- TED

#### We have experience in products with various routes of administration such as:

- Ocular Implants
- Sub-retinal surgical delivery
- Intravitreal injections
- Suprachoroidal injections
- Oral
- Topical eye drops

We have also managed multiple pediatric and gene therapy studies.

### Acceleration success stories

- Reduced global site activation timelines by over 30% through the use of innovative site feasibility and activation strategies
- Accelerated site startup by 30 days using our selected sites and leveraging preferred site relationships
- Successfully completed enrollment 12 months ahead of client's predicted deliverable for XLRP gene therapy rescue trial through our management, site support, and patient identification

## End-to-end operational expertise for rare ophthalmic diseases

Our team of dedicated ophthalmic professionals uses a full suite of innovative end-to-end drug development, study design and strategic guidance capabilities to rapidly determine the market potential for your rare ocular disorder treatment or device.



A global network of >2,000 experienced principal investigators, with indication-specific expertise



Characterization, endpoint determination, and patient recruitment expertise



Optimized, data-driven country and trial site selection



Site activation, enrollment, and retention strategy development



Deep understanding of key protocol components impacting enrollment



Strategic development consulting expertise in core disciplines (CMC, toxicology, pharmacology)



Clinical and regulatory strategy consulting



Data management and ophthalmology expertise in biostatistics, quality, and regulatory expertise



Relationships with key opinion leaders by ophthalmic specialty



Support services for optimizing access and minimizing patient burden



Global strategic partnerships with top performing ophthalmology sites



Global experience with ophthalmology image reading centers

## Easing enrollment and increasing retention with patient-centric services

We recognize how burdensome clinical trials in rare disease can be for patients and their caregivers. Our host of supportive concierge services reduces the burden for both sites and patients and makes it easier for patients to participate in trials by offering:



Telemedicine & **Home Healthcare** Services



**Digital & Decentralized Protocols** 



**Transportation** Coordination & Verification



eCOA/ePRO



**Flexible** Reimbursement **Options** 



Wearable & Mobile Pagers

Through strategic preferred partnerships and expanded vendor capabilities, we have implemented ophthalmologicspecific offerings such as:

- Patient Identification: Identifying patients through registries and/or directly through the site's own EMR data, reducing site workload and improving the speed of enrollment
- Ophthalmology Mobile Sites: Bringing the site to the patient by going to the patients' homes, setting up outside the site office, or alternate locations to assist with visit procedures, including ophthalmic exams and IP management

These services help produce timely and high-quality data for our clients while saving patients time and cost. Our patient-centric approach has led to over 90% patient retention over five years for a recent long-term follow-up trial.

## **Broad expertise** in ocular disorder research

We assign every ophthalmic project **key functional leaders** with significant ocular research experience across early development, PROs, Phase I-IV trials, observational research, and other aspects of research into a variety of eye disorders, including:

- Acute optic neuritis
- Macular degeneration
- Diabetic macular edema/ retinopathy
- Glaucoma
- Thyroid Eye Disease
- Lens opacification/ post-cataract surgical inflammation
- Retinopathy of prematurity
- Retinitis pigmentosa
- Uveitis
- Myopia
- Fuchs dystrophy
- Dry Eyes
- Retinal Vein Occlusion
- Geographic Atrophy
- Stargardt Disease

## Cross-functional rare disorder & pediatric research in our Center of Excellence

Our Rare Disease and Pediatric Center of Excellence (COE) offers access to more than 20 rare disease experts who

services informed by our long-term partnerships with patient advocacy groups, as well as a compassionate, open discourse with individual participants and researchers.

## Experience with central image reading vendors in:



Fluorescein **Angiography** 



**Endothelial Cell Counts** 



Microperimetry



Fundus **Autofluorescence Imaging** 



OCT (corneal angles, retinal thickness, NFL, & ganglion cell layer)



**Corneal Haze** 



**Iris Color Photos** 



Are you ready to learn more about accelerating your clinical trial with our expertise in rare ophthalmic diseases? Visit <a href="mailto:ppd.com/raredisease">ppd.com/raredisease</a> and <a href="mailto:ppd.com/ophthalmology">ppd.com/ophthalmology</a>

