PPD®
Part of Thermo Fisher Scientific

ACCELERATE YOUR CLINICAL TRIAL

with PPD's Expertise in Rare Ophthalmic Diseases

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.

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, PPD 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.

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. PPD's **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 PPD's 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.

PPD'S RARE OPHTHALMOLOGY TEAM HAS SUPPORTED:

26+	Rare disease studies
8,695+	Patients
1,854+	Global sites
300+	Ophthalmological professionals

We have managed and delivered on multiple indications, including:

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

PPD has experience in products with various routes of administration such as:

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

PPD has 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 PPD-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 PPD management, site support, and patient identification
- **Beat enrollment timelines by a month** on an age-related macular degeneration (AMD) treatment-naïve trial by employing proactive enrollment strategies
- **Facilitated registration approval** for products to treat AMD, branch retinal vein occlusion (BRVO), central retinal vein occlusion (CRVO), uveitis, diabetic macular edema (DME), and open-angle glaucoma/ocular hypertension

END-TO-END OPERATIONAL EXPERTISE FOR RARE OPHTHALMIC DISEASES

PPD's 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 more than 7 ophthalmology image reading centers

EASING ENROLLMENT & INCREASING RETENTION WITH PATIENT-CENTRIC SERVICES

We recognize how burdensome clinical trials in rare disease can be for patients and their caregivers. PPD provides a host of supportive concierge services to reduce the burden for both sites and patients and **make it easier for patients to participate 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, PPD has implemented ophthalmologic-specific 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**
- **Leber's congenital amaurosis**
- **Lens opacification/ post-cataract surgical inflammation**
- **Retinopathy of prematurity**
- **Retinitis pigmentosa**
- **Usher syndrome**
- **Uveitis**
- **Myopia & Fuchs dystrophy**
- **Dry Eyes**
- **Retinal Vein Occlusion**
- **Geographic Atrophy**
- **Stargardt Disease**

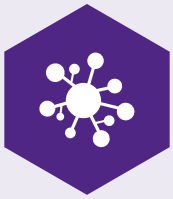
CROSS-FUNCTIONAL RARE DISORDER & PEDIATRIC RESEARCH IN OUR CENTER OF EXCELLENCE

Our Rare Disease and Pediatric Center of Excellence (COE) offers **access to more than 20 rare disease experts, including former FDA officials,** who provide strategic insights across all therapeutic areas to inform and optimize clinical and regulatory strategy development.

We recognize that there are special challenges in pediatric ophthalmic studies, so we provide patient and caregiver services informed by our long-term partnerships with patient advocacy groups, as well as a compassionate, open discourse with individual participants and researchers.

OVER 7 CENTRAL READING VENDORS IN:

Angiography



Endothelial Cell Counts

Visual Field

Fundus Autofluorescence Imaging

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

Corneal Haze

Iris Color Photos

Find out more.
Contact us at **PPD.com/therapeutic-expertise/ophthalmology/**

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