Ora

Creating vision
beyond what we see

Driving Breakthroughs in IRD Treatment: Ora's Expertise in Clinical Trial Management

Combining deep retina therapeutic area expertise with substantive site relationships to deliver high-quality clinical trials.

It is difficult to envision a more devastating ophthalmic condition than Inherited Retinal Diseases (IRDs), which result in progressive and at times rapid loss of vision leading to blindness, often in pediatric patients. Having worked closely with many IRD clinicians and patients, we know how important it is to bring new therapies to market to treat these severe retinal diseases. The unmet need is substantial, so time is of the essence.

Studying IRDs often entails finding patients with rare mutation specific disorders, and matching study designs and endpoints to fit the pathology of the dystrophy. Not all endpoints accepted by regulators are a good fit for inherited retinal diseases that could be characterized by rod degeneration, cone degeneration, or optic nerve degeneration, depending on the underlying genetic flaw. At Ora, we meet the demands of ultra-rare patient enrollment and take a scientific focus that offers the optimum endpoint strategy for your inherited retinal disease program. We share your drive to find the right therapies for these patients and bring them to market, and we are here to offer our expertise and executional excellence to guide you to success.

IRD Highlights

Over 45 retina trials involving 250+ sites globally during the last 5 years, with IRD research across the US, Europe, and LATAM for phase 0, 1, 2, and 3 programs

Experience across IRDs, such as Retinitis Pigmentosa, Achromatopsia, Choroideremia, Retinoschisis, Leber's Hereditary Optic Neuropathy, Stargardt Disease and Leber's Congenital Amaurosis

Significant investment in site networks in LATAM and Eastern Europe to access treatment-naive patients and provide clinical trials as a care option to under-served populations

Close relationships with all major reading centers, customizing your study analyses to best meet endpoint needs

Each study Ora conducts is tailored to best meet the requirements imposed by protocol design, site selections, and target patient populations. Unlike large Clinical Research Organizations (CROs), we are accustomed to the nuances associated with dealing with inherited retinal disease centers of excellence. Most mutation specific IRD trials involve academic groups, many of which Ora contracts with and operates with regularly.

Ora also maintains close relationships with global ophthogeneticist KOLs and regulatory bodies. Many of your known speakers and advisory board members collaborate closely with us as investigators, protocol consultants, and friends. We also meet with the FDA and EMA regularly to understand the latest guidance for, and collaborate on, appropriate endpoints. Our Ora Visual Navigation Course (VNC) for mobility testing was developed through close collaboration with industry partners and US FDA. We pride ourselves both on ophthalmic knowledge and community connections to ensure we are giving you the best guidance we can on your program.

- Ora has conducted 4 turnkey IRD programs in the past 5 years. These programs include mutation specific and mutation agnostic RP, and LCA, with a wide range of product mechanism from gene therapy to oglionucleotide and stem cell based products.
- In addition to providing turnkey CRO services in this area, Ora also developed and validated a multiluminance mobility test that has been installed in over 35 IRD centers globally and utilized in over 19 IRD development programs.

Ora's Posterior Segment Division represents the fastest growing department of Ora, comprised of a >100 person, globally situated, operations team experienced in a myriad of retinal indications. With over 35 active programs, Ora's Posterior Segment division is likely conducting more retina studies than any other singular CRO. The team includes an on-staff retina surgeon, a theraputic area head with 20+ years of ophthalmic drug development experience, and an operations head that has conducted or overseen over 30 retina trials. Ora's Posterior Segment team also maintains relationships with the industry's top retinal imaging centers and develops novel endpoints to better understand and treat diseases of the retina.

Ora vs CRO Analysis

	Ora	Small US-Based CROs	Large Global CROs
Tailored approach to every study to ensure optimal patients, sites, and execution	●	●	○
Study leadership highly experienced within specific ophthalmic therapeutic area of research	●	◐	○
Study site staff and monitors highly trained in ophthalmology	●	◐	○
CRO staff retention year over year +90%	●	○	○
Have ophthalmologists, optometrists, and retina specialists on staff shaping protocols, leading core study components and medical monitoring	●	◐	○
Ability to execute traditional CRO research or implement customized models and endpoints to design novel programs, whatever is needed to best prove out treatment effectiveness	●	○	○
Meticulously tested and documented SOPs and processes for phase 0, 1, 2, 3 and 4 research around the globe	●	◐	●
Priority and exclusive site relationships within the United States, Europe, LATAM, and APAC with top investigators and KOLs	●	○	●

Ora

Ora is a global full-service ophthalmic drug and device development firm with vast capabilities through all steps of clinical research, including preclinical, clinical, CMC & regulatory, and patient and site evaluations. Through Ora's 40+ years of experience, the company has assisted in bringing more than 80 products to market. Ora's team of experts utilizes global regulatory strategies, integrated research operations, and extensive site and patient engagement to accelerate product development in anterior and posterior segment, as well as ophthalmic devices.

80+ Product approvals

1000+ ophthalmic sites across the Europe, US LATAM & Asia

2000+ clinical studies and consulting projects

400+ employees across over 25 countries

25,000+ patients enrolled in the last 7 years