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Media Contact:

Chris Adams
Vice President, Marketing & Communications
cadams@fightingblindness.org
(410) 423-0585

Foundation Fighting Blindness and InformedDNA Partner to Engage and Screen Patients for ProQR's Pivotal Usher Syndrome and Retinitis Pigmentosa Clinical Trials

Trial recruitment is enabled by the Foundation Fighting Blindness' patient registry, My Retina Tracker® Registry, which includes genetic and vision information for more than 20,000 people with inherited retinal diseases.

COLUMBIA, Maryland – March 8, 2022 – The Foundation Fighting Blindness, the world's leading organization committed to finding treatments and cures for blinding retinal diseases, is partnering with ProQR Therapeutics and InformedDNA® to accelerate patient identification and enrollment in clinical research for people with Usher syndrome or non-syndromic retinitis pigmentosa (RP) due to mutations in exon 13 of the *USH2A* gene.

RP is a degenerative disease of the retina that currently leads to legal blindness in nearly all patients and affects approximately one in 4,000 people throughout the world. Usher syndrome is a rare and debilitating disease that causes RP in combination with hearing loss or deafness. ProQR's Phase 2/3 trials center around its emerging therapy, ultevursen (previously named QR-421a), and are accepting patients with mutations in exon 13 of the *USH2A* gene. The ultevursen program will be looking to recruit individuals with early to moderate and advanced vision loss. The ultevursen program is taking place at multiple sites in the U.S. and EU, following observations of vision improvements in the Phase 1/2 trial for ultevursen (previously named QR-421a).

The Foundation is using its exclusive My Retina Tracker® Registry to identify patients with mutations in *USH2A*-exon 13 to accelerate identification of candidates for the trials. Registry members who are contacted by Foundation staff may make an appointment with an InformedDNA board-certified genetic counselor, who will review the clinical study opportunity and screen the patient for likely study eligibility. There is no cost for individuals to speak with counselors from InformedDNA about the trials.

"To get vision-saving therapies across the finish line, we need to identify participants for current and future clinical trials. That was the impetus for launching the My Retina Tracker Registry," said Todd Durham, PhD, senior vice president of clinical research outcomes at the Foundation Fighting Blindness. "Finding trial participants is



challenging because IRDs are rare. We are excited about this collaboration with InformedDNA because we can showcase the value of the Registry as a resource for both patients with inherited retinal diseases, such as *USH2A*-mediated retinitis pigmentosa, and the researchers developing therapies to save and restore their vision. This collaboration should accelerate the recruitment process for these trials and provide our Registry members valuable access to genetic counselors at InformedDNA, who can discuss whether these trials may be appropriate for them. As with every clinical trial, we encourage our members to consult with their ophthalmologist.”

“The *USH2A* gene is the most common gene associated with both retinitis pigmentosa and Usher syndrome, making it a critical problem to solve for this patient community,” said Karmen Trzupsek, MS, CGC, director of clinical trial services at InformedDNA. “This collaborative recruitment effort, sponsored by ProQR and supported by the Foundation Fighting Blindness My Retina Tracker Registry and InformedDNA’s specialty genomics services, represents an important step in expanding access to clinical trials for patients with these rare diseases. Once potential candidates for the trials are identified through the Registry, our virtual screening process is highly efficient and effective at engaging patients in their home communities and decreasing time to enrollment in the trials.”

“We are pleased to partner with the Foundation Fighting Blindness and InformedDNA to use the My Retina Tracker Registry to help enable genetic diagnosis, improve access to clinical trials, and raise awareness of ongoing clinical research and our ultevursen program for people living with *USH2A*-mediated retinitis pigmentosa,” said Daniel A. de Boer, Founder and Chief Executive Officer of ProQR Therapeutics.

About the Foundation Fighting Blindness

Established in 1971, the Foundation Fighting Blindness is the world’s leading private funding source for retinal degenerative disease research. The Foundation has raised more than \$856 million toward its mission to prevent, treat, and cure blindness caused by retinitis pigmentosa, macular degeneration, Usher syndrome and the entire spectrum of blinding retinal diseases. Visit FightingBlindness.org for more information.

About the Foundation Fighting Blindness My Retina Tracker Registry

Developed and maintained by the Foundation Fighting Blindness, the My Retina Tracker® Registry is a global, free, and secure Registry of more than 20,000 people with inherited retinal diseases. The Registry includes extensive information on the genetics, vision loss, and lifestyle impacts of people with IRDs. Through a program linked to the Registry, the Foundation provides no cost genetic testing and counseling for inherited retinal disease patients through its testing partner Blueprint Genetics and its counseling partner InformedDNA.

Patients and/or family members can register at www.MyRetinaTracker.org.



A My Retina Tracker Registry registrant's personal information is always protected. Researchers and therapy developers cannot access a patient's personal information when querying the Registry. The patient and/or family member is notified when they potentially meet the criteria for a clinical trial and then contacts the clinical trial sponsor or their representatives if they want to learn more about participating in the trial.

About ProQR

ProQR Therapeutics is dedicated to changing lives through the creation of transformative RNA therapies for the treatment of severe genetic rare diseases such as Leber congenital amaurosis 10, Usher syndrome and retinitis pigmentosa. Based on our unique proprietary RNA repair platform technologies we are growing our pipeline with patients and loved ones in mind. Learn more about ProQR at www.proqr.com.

About InformedDNA

InformedDNA is the country's leading applied genomics solutions company, helping harness the full power of precision medicine. With the largest independent staff of board-certified genetics specialists in the U.S., InformedDNA ensures that health organizations have access to the highest quality, most current genomics insights to optimize clinical decisions. Its solutions, which cover evidence-based guideline development, patient experience management, and value management, have helped optimize the health benefits of more than 100 million covered lives and have navigated hundreds of thousands of people to the right treatments or clinical trials. For more information, visit www.InformedDNA.com.

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