**Frequently Asked Questions**

1. What clinical studies are in progress at CAROT?

The clinical core is carrying out:

* Natural history studies for a variety of inherited retinal degenerations (IRDs)
* Long-term follow-up for individuals enrolled in a Spark Therapeutics-sponsored Phase ½ gene therapy trial for choroideremia
* Long-term follow-up for individuals enrolled in Phase 1 and Phase 3 gene therapy trials sponsored by Spark Therapeutics for *RPE65* deficiency (Leber congenital amaurosis, retinitis pigmentosa)
* A Phase I gene therapy study sponsored by REGENX to evaluate RGX-314 in individuals with neovascular age-related macular degeneration
* Research studies aiming to develop and fine-tune novel outcome measures for clinical trials
* Research studies aiming to identify genes and mutations that cause IRDs
* Structural and functional imaging studies of the retina
* Structural and functional imaging studies of the brain

2. What is in your pipeline for clinical studies at CAROT?

* A Phase I gene therapy clinical trial for Leber congenital amaurosis caused by *LCA5* mutations (sponsored by Limelight Bio)
* A Phase I gene therapy clinical trial for Leber congenital amaurosis and early onset retinitis pigmentosa caused by *RDH12* mutations (sponsored by Limelight Bio)
* Multiple additional retinal degeneration targets are in the pipeline.

3. What can a patient with retinal degeneration do to help investigators develop new treatments?

* See a retinal degeneration specialist so that the disease can be characterized and so that any anomalies that can be treated are indeed treated. The specialist may arrange genetic testing.
* Network with patient advocacy groups relevant to your condition, such as Foundation Fighting Blindness, Choroideremia Research Foundation,  Curing Retinal Blindness; Sophia Sees Hope, etc.
* Assist in fundraising efforts to support the research and clinical trials.
* Consider enrolling in studies relevant to your disease. Clinical studies are listed at clinicaltrials.gov. Note: Please check with the FDA to be sure that any interventional studies have an associated IND (investigational new drug) number.

 4. Is there any cost for participating in a clinical trial?

* Clinical studies are listed at There are no charges for participating in our clinical trials. Travel, lodging and food costs should be covered, including those of an accompanying person. There are no charges for the clinical testing or intervention.

 5. Are your clinical trial subjects only from Pennsylvania?

* No. While it can be easier on the subject/family to live in the vicinity since there may be signiﬁcant travel obligations, we do not have limitations based on citizenship or homeland.

6. Who can I contact to ﬁnd out more about your clinical studies?

* Assist in fundraising efforts to support the research and clinical trials. If you are interested in participating in non-invasive imaging studies, please contact Dr. Jessica Morgan (jwmorgan@pennmedicine.upenn.edu) or Manzar Ashtari (ashtari@pennmedicine.upenn.edu). If you are interested in participating in our clinical trials, please contact the coordinator listed on the relevant www.clinicaltrials.gov website.

7. How can I send clinical information to your team members?

* Please send information by fax (215-573-7155) or mail: Dr. Jean Bennett,  University of Pennsylvania, 310 Stellar-Chance Labs; 422 Curie Blvd,  Philadelphia, PA 19104 or Dr. Tomas Aleman or Dr. Katherine Uyhazi or Dr. Albert Maguire; Ophthalmology; 3400 Civic Center Blvd, Philadelphia, PA 19104.

8. Where is CAROT?

* CAROT is located in several buildings on the University of Pennsylvania campus.
  + Clinic: Perelman Center for Advanced Medicine (PCAM); 3400 Civic Center Blvd; 3rd floor, Ophthalmology; Philadelphia, PA 19104}. Patient Parking (including valet parking) is available at PCAM
  + Translational Laboratories: 422 Curie Blvd, Philadelphia, PA 19104 and John Morgan Building. Contact us for the exact room numbers. Parking is challenging! Public parking can be found at 119 S. 38th Street, Philadelphia, PA 19104 (Walnut and 38th St); 3335 Chestnut St, Philadelphia, PA 19104 (Chestnut and 34th St)
  + Outcome Measure Development: PCAM (see above) and 3517 Market St, Philadelphia, PA 19104 (arrange visit in advance)

 9. Can children be seen at CAROT?

* Yes, depending on their age and the type of evaluation. Children can also be seen by CAROT investigators (Drs. Aleman, LeRoy, Uyhazi) at The Children’s Hospital of Philadelphia (CHOP), Buerger Center for Advanced Pediatric Care, Dept. Ophthalmology, 3500 Civic Center Blvd, Philadelphia, PA 19104.

10. How can one get to CAROT (clinic) using public transportation?

* Train (also Megabus, Bolt Bus): Arrive at Amtrak 30th Street Station, Philadelphia, PA (Walking; 1.3 miles); Walk west (away from Center City) on Market St; Turn left on 33rd St (0.4 mi); Continue onto Civic Center Blvd (0.2 mi); 3400 Civic Center Blvd is on the left.
* From Septa University City Rail Station (Walking; 0.4 miles); Head west on Spruce St; Turn left onto S 34th St (0.1 mi); Slight left onto Civic Center Blvd (0.2 mi). 3400 Civic Center Blvd is on the left.