

20/20 Onsite Helps Biotechnology Company Safely and Successfully Forge Ahead with Clinical Trial Assessments and Pre-Screenings



Background

As a clinical-stage biotechnology company, Applied Genetic Technologies Corporation (AGTC) relies on the results of gene therapy clinical trials to develop treatments that can transform the lives of patients suffering from rare and debilitating ophthalmic, otologic and central nervous system diseases. Two ophthalmic diseases AGTC has been conducting clinical trials for are X-linked Retinitis Pigmentosa (XLRP), an inherited condition that causes progressive vision loss in boys and young men, and Achromatopsia (ACHM), an inherited retinal disease that results in markedly reduced visual acuity, extreme light sensitivity causing day blindness, and complete loss of color discrimination.

20/20
onsite



THE AGTC
CASE STUDY

Background

PANDEMIC-RELATED CHALLENGE

When the COVID-19 pandemic caused a nationwide shutdown in March 2020, it disrupted many clinical trials that were potentially bringing new therapeutics to market that could benefit patients in need. AGTC's trials were no exception. Most of the XLRP and ACHM clinical trials' 63 participants were unable to appear for their scheduled follow-up assessments. Some clinical sites were closed or had limited staffing. And with only a few sites located throughout the country, getting to them often required air travel, which was restricted and no longer an option for participants who were located throughout 24 states. Without accurate and timely data derived from these ocular assessments, the trials would be significantly delayed.

PANDEMIC-RELATED SOLUTION

AGTC's leadership began discussing transitioning to a decentralized model, and was aware that 20/20 Onsite had been delivering high-quality, convenient vision care to companies and patients via its state-of-the-art mobile vision clinics (MVCs). Initially, AGTC's regulatory and quality assurance groups had concerns about critical essentials such as maintaining compliance and equipment calibration within a decentralized or hybrid model. The clinical operations and patient advocacy teams, however, knew they had to challenge the status quo in order to move forward. Confident that this patient-centered model would allow them to continue to assess the safety and efficacy of their treatments in a flexible, secure, and convenient way, AGTC partnered with 20/20 Onsite to perform direct-to-home key follow-up assessments that would enable them to move forward with the XLRP and ACHM trials.

20/20
onsite



THE AGTC
CASE STUDY

Background

SKYLINE ENROLLMENT CHALLENGE

In early 2021, AGTC began enrollment for its SKYLINE trial of AGTC-501, a recombinant AAV vector-based gene therapy developed for the treatment of XLRP. Due to a combination of complex Inherited Retinal Disease protocols and an inability to pre-screen patients in a timely manner, enrollment was lagging. The company's goal was to enroll 12 participants in the trial, but two months into the enrollment process, they had only been able to qualify a small fraction of the patients needed to move forward with the trial. Challenged by screen failures, leadership knew that if changes weren't made quickly, there was a high likelihood that the trial would fail to meet its enrollment target on time.

SKYLINE ENROLLMENT SOLUTION

AGTC began addressing its SKYLINE enrollment challenge by fine-tuning its protocols in order to open the study to a wider group of participants. Next, they turned to 20/20 Onsite's MVCs as a means to alleviate the burden on the sites and access prospective participants more quickly so that they could be pre-screened for the trial. In addition to the MVC that had already been used for the pandemic-related rescue mission, a second MVC had to quickly be reconfigured and deployed to pre-screen patients expressing an interest in taking part in the trial and re-screen patients who had previously failed to meet criteria.



The Onboarding Process

The clinical team at 20/20 Onsite worked closely with AGTC to understand the quality control protocols, reconfigure their mobile clinics to accommodate the specialized requirements of the research, properly train its staff, and obtain the necessary certifications.

Dedicated MVCs were deployed with trained and knowledgeable ophthalmic technicians. The mobile clinics were outfitted with clean and organized data systems as well as the same high-tech vision testing equipment utilized at AGTC's traditional brick and mortar sites, including:

- Ocular Photosensitivity Analyzer (OPA) to quantify the visual photosensitivity thresholds (VPT) in healthy and light sensitive subjects.

- Macular Integrity Assessment (MAIA) to capture microperimetry retinal images.
- Advanced Vision and Optometric Testing (AVOT) to measure the quality of spatial vision, scattered light in the eye and the ability to see rapid flicker and motion perception.
- Optical Coherence Tomography (OCT) to see each of the retina's distinctive layers and allow the ophthalmologist to map and measure their thickness.
- Color Brightness (CoBri) test to assess the percept of achromatopsia patients that originates from a color stimulus and whether this percept changes after gene therapy.



How It Worked

The MVCs traveled throughout the United States providing patients with the accessibility and convenience of a mobile clinical center without sacrificing the quality of care administered. The customized program consisted of:

1. 20/20 Onsite and AGTC worked with the site to schedule the patient to be seen on board the MVC.
2. At the scheduled appointment time, the MVC met the patient and their optional guest at or near their residence to conduct the assessment or prescreening.
3. Friendly onboard staff provided a safe, informed experience. With only one patient onboard at a time, staff was able to work at a pace comfortable for the individual patient.
4. Staff performed secure, compliant, and timely data collection.



How It Worked

PANDEMIC-RELATED RESULT

AGTC met their goal of reporting out ophthalmic data from all three ongoing clinical trials in the fourth quarter of 2020. With the help of Onsite 20/20, AGTC was able to complete 150 assessments over 24 states across four trials as of July 2021. Since the MVCs removed most of the barriers related to travel, compliance, and ongoing participation, the trials met or exceeded retention rate expectations during a pandemic. What began as a “rescue mission” during the pandemic has blossomed into much more, and AGTC and 20/20 Onsite have expanded services beyond pandemic rescue into additional services and studies.

SKYLINE ENROLLMENT RESULT

As a result of the updated protocols and the ability to quickly pre-screen and re-screen patients via the MVCs, AGTC not only met its planned enrollment target on time, but exceeded its original goal of 12 participants. By January 2022, a total of 14 patients were enrolled in the SKYLINE trial. Additionally, key findings on a patient’s baseline characteristics, combined with a novel application of machine learning techniques, are being applied to the SKYLINE and VISTA trials to potentially further enhance the ability to identify likely responders. The SKYLINE MVC pre-screening process helped prequalify many patients for potential enrollment in its upcoming VISTA trial.



How It Worked

HIGH PATIENT SATISFACTION

Some patients preferred the MVC experience to that of the traditional assessment process. In fact, 97% of participants said they would recommend the service, citing the following reasons for their satisfaction:

- MVCs eased the time and burden of travel, providing enhanced convenience and flexibility.
- MVC staff put nervous patients (many of whom are pediatric) at ease by making them feel welcomed and comfortable, demonstrating equipment prior to performing exams and allowing breaks as needed.
- Sanitary environment and safety-first staff made patients feel safe while undergoing assessments in the midst of the COVID-19 pandemic.
- Assessments were comprehensive and efficient, making for a no-hassle experience.

FASTER TIME TO MARKET

According to life sciences analytics firm GlobalData, nearly 200 companies stopped or delayed trials during the first few months of the pandemic.¹ AGTC was able to mitigate pandemic-related delays by utilizing 20/20 Onsite's MVCs. When faced with enrollment challenges in 2021, the MVC process gave AGTC the ability to increase enrollment speed and decrease screen failures as a result of the MVC's ability to pre-screen patients to help qualify them for the next phase of the trial. Ultimately, this helped to push AGTC further along the path to developing treatments that can improve the lives of patients suffering from XLRP and ACHM.

DECREASED COSTS

The use of MVCs allowed AGTC to avoid the high costs associated with trial delays and participant dropout. According to a study conducted by CenterWatch, the financial impact of delayed trials is quite significant, costing between \$600,000 to \$8 million each day the trial is delayed.²



¹ *Cost of Disrupted Clinical Research Due to COVID-19 Equates to \$10+ Billion & Potential Study Delays*, Drug Development & Delivery.

² *Patient-Centric Clinical Trials: The Acceleration Of Decentralized Patient Monitoring*, Forbes, October 20, 2021.

Conclusion

The hybrid clinical trial model is slowly being embraced by many companies worldwide for certain studies that would benefit from the flexibility, convenience, and support they provide. It's important that research stakeholders take action before their study falls behind due to lack of enrollment and patient dropout. Partnering with a company like 20/20 Onsite to supplement site activity with an MVC is a proven way to keep your trial on track.



We recognize the incredible commitment patients make when they enroll in our trials and our goal is to match that commitment and support them in any way possible. By using a creative patient focused approach to testing via 20/20 Onsite's mobile vision testing program, we 'take the test to the patient' to support their needs during this extremely difficult time."



Sue Washer, CEO
AGTC