AliraHealth

health storylines™

Partnering with Patients to Streamline Trial Operations: From Protocol Design to FDA Submission in 60 days

Case Study:

Evaluating the usability of a novel eye dropper bottle for a rare disease population

Introduction

As the healthcare sector continues its shift towards demonstrable patient-centricity, FDA has issued a series of guidelines for the industry regarding topics including diversity and inclusion, patient experience data, and addressing rare patient populations. As these changes are implemented across the product development lifecycle, data requirements to demonstrate evidence of efficacy and safety for regulatory approval are also shifting.

In order to establish whether newly developed medical products fulfill the diverse needs of patients, usability studies employ a unique framework to collect the required quantitative or qualitative data for regulatory approval. Based on the FDA guidance document "Applying Human Factors and Usability Engineering to Medical Devices," conducting usability assessments on new medical devices or products is necessary to maximize safety and effectiveness for their intended users, uses, and use environments.

The following is an example in which experts from Self Care Catalysts, an Alira Health company (SCC), helped a biopharmaceutical company generate patient-reported real-world evidence using our Health Storylines Decentralized Clinical Trial (DCT) platform to successfully complete their IND application in a record timeline.

Who, What, and Why

As a result of product shortage, this biopharmaceutical company had recently changed manufacturers for an eyedropper bottle used to treat a rare but serious disease, with a lifelong impact on many parts of the body, particularly the eyes. Due to the rarity of this disease, only one other product was on the market for patients to use, and there was a strong demand to get this biopharmaceutical company's improved product back on the market. This urgency led to FDA requesting a usability study of the eyedropper bottle with an incredibly short deadline. However, the patient population of interest was extremely rare and not centrally located, introducing a significant barrier to completing this usability study in the accelerated timeline required by FDA.

This is where SCC's existing partnerships with patients and patient advocacy groups, and expertise in driving digitally supported research, were required in order to address some of the barriers to completing this type of study in a record timeline.

Partnering with Patients to Meet the Regulatory Challenge

To gather the data required by FDA within the specified timeline, and avoid barriers associated with a rare and geographically distributed patient population, a decentralized clinical trial (DCT) design that leveraged remote data collection was employed. SCC conducted a single-arm, open-label, virtually- conducted study that leveraged the virtual visit function of the Health Storylines platform to remotely assess participants' ability to successfully perform critical tasks related to the usability of the new bottle. SCC designed the protocol with the unique needs of the patient population in mind, in a way that would meet the regulatory requirement, streamline operations, and ease the burden on the patient. SCC also led patient recruitment, designed instructional materials to guide the patients through the process, and managed the Institutional Review Board (IRB) submission to support the execution of the study. This DCT design was particularly successful as it 1) leveraged virtual tools allowing patients to stay home, 2) supported the ability to remotely obtain an ophthalmologist's review of videos and gauge the "success" of the eye drop application, 3) offered the ability to archive study videos for the review of primary data for inter-rater reliability or audit purposes if requested. Meeting these objectives using a traditional site-based or in-person assessment model would have taken exponentially longer than the allotted time.

Outcomes - FDA Approval

By working within an accelerated timeline, SCC, in partnership with this biopharmaceutical company, collected the data required to successfully demonstrate the usability of the new bottle for this rare disease population and ultimately received FDA approval.

Role of the patient: helping to accelerate outcomes for all your needs

Beyond the ability to quickly initiate and complete decentralized studies, our partnerships with patient communities and, in particular, the relevant rare disease patient advocacy group for this study helped instill patients' trust in us and the platform. By adding value to patients' lives regardless of the affiliation to a particular research configuration, the Health Storylines platform promotes sustained engagement and high-quality data that accurately reflects the patient experience.

This integrated partnership with patients can aid in the generation of unique patient-focused insights and accelerate research and patient-facing activities across a product portfolio and throughout the product lifecycle.

How Can Health Storylines Help You

SCC has previous experience designing and executing research among patients, health care professionals, and caregivers in a multitude of therapeutic areas and global research settings. Our approach to studying design, patient recruitment, engagement, and the complete life cycle of research operations powered by the Health Storylines digital platform is informed by deep insight into the patient journey. This patient focus results in a streamlined process of engagement with technology that yields timely, high-quality results

The Health Storylines (HS) product suite is a patient-centered digital operating system offering an enterprise solution that supports DCT, Real World Evidence (RWE)/Patient Journey Data (PJD), Patient Engagement, and Patient Monitoring that enable the entire organization to support multi-functional activities that support the whole product lifecycle management. Our goal is to help patients be better and participate in research while sharing self-accountability and responsibility for their care; enabling a high-touch, high-tech, high-science approach to healthcare.