**Revealing the Power of the Rare Disease Community With State-of-the-Art Collaborative, Trustworthy, Digital Education Software**

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*Washington, D.C., October 16-17, 2017:* rareLife solutions will be presenting the results of 2 separate case studies, each demonstrating how a collaborative, digital, educational, multistakeholder, online platform built for the Familial Adenomatous Polyposis (FAP) community (www.oneFAPvoice.com), was used to gather community feedback to inform and direct the development of 2 separate FAP trials.

In the first case study, the FDA questioned the validity of a primary endpoint in a fully recruited Phase III FAP trial that compared the efficacy of 3 treatment regimens in delaying the time to the first occurrence of an FAP-related event. The sponsor endeavored to support its position with FAP patient sentiments and expert physician input; however, the time to accrue said community feedback was limited. Patients with FAP were asked to complete a survey through oneFAPvoice about their postsurgical experiences. Forty-nine valid patient responses were submitted in one week. The results showed that the majority of patients were adversely affected - financially, physically, and/or emotionally - by this particular intervention. Inherited GI expert physicians were also surveyed via oneFAPvoice and nearly all respondents reported that patients with high-risk adenomas were at an increased risk for progressive polyposis and/or cancer, warranting surveillance and polypectomies. The information was included in the FDA response package and the FDA acknowledged the sponsor’s position to include surgical removal of high-risk adenomas as a primary endpoint in the FAP trial.

In the second case study, a pharmaceutical company was designing an FAP trial in the pediatric patient population and sought to better understand critical issues relevant to maximizing patient enrollment and retention. The oneFAPvoice platform fielded a patient/caregiver survey to understand the needs related to trial endpoints, entry criteria, and quality of life (QoL) parameters. Eighty-four responses were submitted within one week. The results showed that over 90% of respondents reported that delaying the time to surgery was an important clinical trial endpoint; 88% responded that delaying surgery was important even if it meant taking medication daily to suppress polyp growth and potential cancer development. Fifty-six percent of participants responded that children 12 years of age or younger should be allowed to participate in the proposed trial with parental consent. The 3 QoL activities identified as the most important included 1) the ability to play and exercise without restriction, 2) the ability to do everything other kids can do, and 3) feeling happy most of the time. Importantly, the results informed the development of the trial protocol.

Both case studies show how a digital education software platform such as oneFAPvoice that allows for collaboration among diverse community members within a given rare disease, can be used to inform and advance scientific research and transform the overall community into informed, engaged, and valued contributors.

**Familial Adenomatous Polyposis**

FAP is a rare, hereditary cancer syndrome most notably characterized by the development of hundreds to thousands of colon polyps that, if left untreated, will lead to cancer in nearly 100% of the cases at an average age of 39.

**www.oneFAPvoice.com**

oneFAPvoice is a digital, educational, multistakeholder online community developed by rareLife solutions and dedicated to those impacted by or involved with FAP. The community platform provides trustworthy information from a variety of sources ranging from scientific journals to consumer-oriented healthcare and lifestyle publications as well as disease-specific videos, social and media sites, and news and events. The platform features community managers that facilitate conversations among community members, engage with FAP and rare disease influencers, and raise awareness of the platform. Importantly, the platform is also designed to collect and share data from and with the community using rotating polls, surveys, user metrics, and user generated content assessment. The entire FAP platform is desktop, tablet, and mobile-friendly.

**rareLife solutions**

rareLife solutions collaborates with companies, institutions, and organizations which are seeking better treatments and services for the historically underserved rare disease communities. The company specializes in 3 elements critical to the successful development of treatments for rare diseases: collaboration, science and technology.  rareLife solutions offers scientific communications services and software solutions for advocacy groups, pharmaceutical and device companies, medical and research centers, and professional healthcare associations.

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