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Pediatric rare disease enrollment case study in Latin America

Statement of the Problem: The paucity and scattered rare disease patient population across the globe and with more than 50% of rare diseases affecting the pediatric population are the major factors that impede successful and faster enrollment into pediatric rare disease trials, therefore slowing down innovative treatment options. Particularly in Latin America, many rare disease patients have not been in a clinical trial before and many go misdiagnosed or not diagnosed at all. In 2016, there were only 25 ongoing pediatric rare disease clinical trials in Latin America in 7 countries, and yet Latin America as a whole represents 626.7 million people, or about twice the population of the United States and 8.63% of the entire world. With a growing middle class in Latin America and the US Latino population representing 17% of that population, pediatric rare disease trials in Latin America are worth their weight to be studied.

Methodology: This case study explores the unique study enrollment challenges in Latin America and shares three solutions within a global Phase 3 pediatric rare disease trial for a major pharmaceutical company.

Findings: Even with the most seasoned CROs and strong site feasibility reports, more than 80%; 7 of all clinical trials do not meet critical milestones and more than 50% of sites do not enroll a single subject, increasing costs of the study and delaying data capture. This project demanded innovative thinking and creative problem solving from not only Sponsors, Investigators, CROs, but also from the referring physicians, families and most critically, patient advocacy groups.

Conclusion & Significance: By applying detective skills, relationship building, data based motivation tools and having a deep understanding of the cultural landscape, communication and innovative management practices were integrated for successful enrollment, which can be duplicated in future trials.

Biography

Sara G Tylosky has over 20 years of experience in strategic management and global marketing in the pharmaceutical and biotech industries. Working with large pharmas to start- up biotechs, her enrollment and training consultancy in Phase 2-4 trials through the boutique CRO Farmacon has allowed companies to reach enrollment early and reduce study costs. By bringing real-time feedback and uncovering issues, her team helps insure targets stay on track. She holds a Master's in Business from Florida Atlantic University and an undergraduate degree from Whitman College.

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