

The GRIT Study: Getting Global Rare Disease Insights Through Technology Study

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Abstract : Background: Disease management of metabolic, genetic disorders is long-term and can be cumbersome to patients and caregivers. Patient-Reported Outcome Measures (PROMs) have been a useful tool in capturing patient perspectives to help enhance treatment compliance and engagement with health care providers, reduce utilization of emergency services, and increase satisfaction with their treatment choices. Currently, however, PROMs are collected during infrequent and decontextualized clinic visits, which makes translation of patient experiences challenging over time. The GRIT study aims to evaluate a digital health journal application called Zamplo that provides a personalized health diary to record self-reported health outcomes accurately and efficiently in patients with metabolic, genetic disorders. Methods: This is a randomized controlled trial (RCT) (1:1) that assesses the efficacy of Zamplo to increase patient activation (primary outcome), improve healthcare satisfaction and confidence to manage medications (secondary outcomes), and reduce costs to the healthcare system (exploratory). Using standardized online surveys, assessments will be collected at baseline, 1 month, 3 months, 6 months, and 12 months. Outcomes will be compared between patients who were given access to the application versus those with no access. Results: Seventy-seven patients were recruited as of November 30, 2021. Recruitment for the study commenced in November 2020 with a target of n=150 patients. The accrual rate was 50% from those eligible and invited for the study, with the majority of patients having Fabry disease (n=48) and the remaining having Pompe disease and mitochondrial disease. Real-time clinical responses, such as pain, are being measured and correlated to disease-modifying therapies, supportive treatments like pain medications, and lifestyle interventions. Engagement with the application, along with compliance metrics of surveys and journal entries, are being analyzed. An interim analysis of the engagement data along with preliminary findings from this pilot RCT, and qualitative patient feedback will be presented. Conclusions: The digital self-care journal provides a unique approach to disease management, allowing patients direct access to their progress and actively participating in their care. Findings from the study can help serve the virtual care needs of patients with metabolic, genetic disorders in North America and the world over.

Keywords : eHealth, mobile health, rare disease, patient outcomes, quality of life (QoL), pain, Fabry disease, Pompe disease

Conference Title : ICMHMPSM 2022 : International Conference on Mobile Health Monitoring and Patient Self-Management

Conference Location : Vancouver, Canada

Conference Dates : May 23-24, 2022