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901.HEALTH SERVICES AND QUALITY IMPROVEMENT - NON-MALIGNANT CONDITIONS

Monitoring Patient Reported Outcomes in PNH: Interim Results of a Market Research with florio ® PNHLouise M Arnold, BSc (hons)¹, Holger Brondke, PhD², Katharina Steinitz, PhD², Richard J. Kelly, BSc, MD³¹ Department of Haematology, St. James's University Hospital, Leeds, United Kingdom² Florio GmbH, Munich, Germany³ Department of Haematology, St. James's University Hospital, Leeds, United Kingdom

Background: Patients with paroxysmal nocturnal hemoglobinuria (PNH), an ultra-rare, acquired disorder, report various cardiovascular, gastrointestinal, neurological or hematological symptoms that can severely impact their physical functioning and health-related quality of life. Patients may require long term treatment with anti-complement therapies to alleviate PNH symptoms or complications. Whilst blood biomarkers can aid the monitoring of disease progression, patient reported outcomes (PRO) and related symptoms are also of value in assessing response to therapy, but they are not always continuously tracked by patients. Hence florio PNH, a software medical device (smartphone app), was designed to allow easy monitoring of PRO and tracking of medication by patients. It allows documentation of therapy and common symptoms as well as hemoglobin and LDH levels. florio PNH can send treatment reminders and compiles the entered data into a patient report. Patients were invited to participate in market research of florio PNH to better understand monitoring habits and expectations towards an app in this area.

Methods: Twenty patients were invited to participate in market research sponsored by Florio GmbH. The participants came from the UK PNH service centre in Leeds and were selected by the coordinating clinical team at routine clinic visits. The research was submitted for ethics approval and written informed consent was provided. The market research consisted of a one hour entry interview assessing current behavior and expectations towards monitoring of outcomes, followed by an eight-week period of field-testing florio PNH app. The subsequent one hour exit interview explored the user experience with the app, and which app features were seen of value, of less value or were missing. A total of 20 patients are to be enrolled in the research, of which 17 had completed the initial interview at the time of this report.

Results: The majority of participants were 50 years of age or older (59%) and described themselves as "not tech savvy". Six participants rated the impact of PNH on their life as significant, 6 as somewhat and 5 as little to none. Fatigue was named by 10 patients unprompted as the prominent symptom affecting their social and work life. Before the research, three participants neither tracked symptoms nor reported medication, five tracked both, seven only tracked their anti-complement infusions and two only major events when they happened. Forty seven percent of participants (8 of 17) did not prepare information at all for clinical consultations in advance, as they either experienced few events or relied on their memory. All users managed to install the app, set up their account and log events without problem. The app was described as easy to use, straightforward and participants appreciated that all data was in one location and possible to summarize into a report. Sixteen (94%) patients said that they believe the app will help them track events and that they would be enthused to use it in the future.

Conclusions: Monitoring applications are employed in several rare bleeding disorders and have been shown to be valuable assets for collecting patient reported outcomes, especially in the outpatient setting. Participants of this research had no experience with using disease specific apps for collecting outcomes, and usually rely on the calendar functions provided by their smartphones or paper diaries. 94% concluded that the app will help them organize their PNH data, share data with their treating clinical team, better plan their future activities and be reminded when the next treatments are due.

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