

Project Outcomes Report

Project name: **LSDU portable technologies**

Project partners: Royal Free London NHS Foundation Trust and Amicus Therapeutics UK Ltd

- Duration: February 2020 – December 2024 (The project was significantly delayed due to COVID-19)

Project overview:

Service improvement: Implementation of a Fabry App to empower Fabry patients to self-monitor signs and symptoms of their disease.

Expected Patient Benefits

- Empowerment: continuing to self-capture real time data on symptoms, relating to their day-to-day activities, on how Fabry affects their lives.
- Better engagement with Royal Free LSDU (Lysosomal Storage Disorder Unit) as their data will be viewed weekly and feedback provided.

Expected NHS Benefits

- Better understanding of Fabry disease, increased frequency of contact with patients, not just relying on information collected at clinic visits.
- Supports the National operating framework for rare diseases: empowering patients.
- Better engagement with patients and symptom management – leading to better outcomes.

Expected Amicus Benefits

- Insights into adherence and patient engagement with the App.
- Commercial insight into Fabry disease.
- Partnership working with the Royal Free NHS trust.

Key Deliverables

- Increase in Fabry App patient downloads & regular user interactions
- Patient reported outcomes better managed via threshold monitoring & reporting
- Development of resource to educate Royal Free LSDU staff & engage Fabry patients on benefits of the App
- Evaluation of the project that will better inform future modifications to the App

Project outcomes:

App Development and implementation outcomes

- The Fabry app was developed and launched – which supported patients to self-monitor signs and symptoms of their Fabry disease.
- Nurse support was needed for data download and analysis.
- Manuscript report on the app was written up and published on Orphanet.

Data outcomes

- 139 symptomatic Fabry disease patients who had a smartphone were invited to download the app.

- 67 patients downloaded and tracked their symptoms on the Fabry app at least once.
- Between May 2018 and December 2022, the median monthly use of the Fabry app was 2 entries and the median time between entries was 16 days.
- Data showed that pain in the hands and abdominal pain were significantly higher in patients with classic phenotype compared to patients with non-classic phenotypes.

Patient Benefits

- Patients found the app easy to use and accurate for tracking their symptoms.
- Patients reported a better sense of disease status.
- Patients reported improved communication with the Royal Free LSDU.

NHS Benefits

- Clinicians felt the app aided better monitoring of symptoms and medication use, including those prescribed by another doctor.
- Clinicians received more accurate and frequent reporting of patient symptoms.
- As a result of monitoring symptoms, patients were contacted and medications were reviewed accordingly.

Amicus Benefits

- Insights into adherence and patient engagement with the app.
- The publications provided insight and shared learnings into the symptoms and management of Fabry disease.

Conclusions and learnings:

- For those patients who used the app, the project demonstrated positive patient response and improved communication by using a smartphone app to facilitate the remote assessment and monitoring of Fabry disease symptoms.
- Patients who were more likely to use the app had a greater disease burden.
- With greater engagement, this intervention has the potential to assess disease progression and early therapeutic intervention.
- Use of the app was not possible for all. Accessibility, engagement and interaction with the app was lower than expected.

References:

- UK Rare Disease Framework
 - <https://www.gov.uk/government/publications/uk-rare-diseases-framework>
- Fabry App: the value of a portable technology in recording day-to-day patient monitored information in patients with Fabry disease
 - <https://ojrd.biomedcentral.com/articles/10.1186/s13023-023-02999-6>
- Digital app for patient monitoring found helpful in small study
 - <https://fabrydiseasenews.com/news/smartphone-app-monitoring-fabry-disease-found-helpful-study/>

***To be approved by both parties & hosted on Amicus / NHS websites**