

Project Outcomes Report

Project name: **Fabry Online Education Programme**

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

Duration: February 2022 – March 2024

Project overview:

To support knowledge and understanding of Fabry disease across a wide UK medical audience, through development and launch of a national online accredited Fabry education Programme. Supporting improved identification and earlier diagnosis of Fabry disease. With the aim of improving patient experience and outcomes in line with the national strategy for rare disease.

Patient Benefits

- Reduced length of time to diagnosis of Fabry disease through improved knowledge of health care professionals.
- Better experience of the journey to diagnosis and improved confidence in HCPs seen throughout their journey.
- Improved management of Fabry disease through improved knowledge of HCPs.
- Better outcomes as a result of improved clinician management.
- Better experience of the overall healthcare system.

NHS Benefits

- Upskilled workforce in an area where education is limited supporting implementation of UK national strategy for Rare Disease.
- More efficient use of resource through reducing time to diagnosis of Fabry disease through delivery of HCP education.
- Better holistic care for Fabry patients.
- Improved experience of patients with Fabry disease through improved HCP education.
- Better patient outcomes as a result of improved clinician management.
- A positive impact on the broader area of Rare Disease in supporting the diagnostic odyssey.
- Support for succession planning into the area of Lysosomal storage disorders.

Amicus Benefits

- Better holistic care and in turn outcomes for Fabry patients supporting our mission to improve the lives of patients with rare disease.
- Potential expansion of the relevant and eligible Fabry patient population as a result of this project in aiming to improve identification of Fabry disease and reducing time to diagnosis.
- Ensure the appropriate use of medicines for Fabry in line with local or national guidance.

Project outcomes

- The project has delivered the desired outcome – in that a Fabry education curriculum has now been designed, created and the following modules have been launched on the MPS society website:
 - **Module 1:** Fabry disease overview
 - **Module 2:** When to suspect Fabry disease
 - **Module 3:** Testing for Fabry disease
 - **Module 4:** Genotype-phenotype relationship
 - **Module 5:** Treating Fabry disease
- Early monitoring has shown good use of the curriculum which supports the national strategy for rare disease to educate healthcare professionals.
- While the downstream project impact is difficult to measure, as it is too early to evaluate the use of the curriculum, we are confident the benefits to patients and the NHS should be met over time.
- The project has supported the Amicus mission to improve the lives of patients with rare diseases.
- The project has involved considerable and equal contributions from both parties – in line with the original proposal.
- Both parties have benefitted from the project and there has been a strong and trusting relationship throughout the collaboration.
- The pilot in Fabry disease has met the criteria to scale into other rare diseases where healthcare professionals need education.

Conclusions and learnings

- The project had clear and measurable outputs – as the learning modules have been created and launched.
- The project took longer than initially expected due to the COVID pandemic. Time constraints and the amount of content that needed to be created, edited and finalised also impacted on the timelines.
- Measurement of use of the curriculum by healthcare professionals will be assessed after the conclusion of the project. Questionnaires for future educational projects will get some data on downstream impacts.
- The success and future learnings of the project will continue to be measured through the MPS society where the curriculum is hosted.

References:

- UK Rare Disease Framework
 - <https://www.gov.uk/government/publications/uk-rare-diseases-framework>
- MPS Society Fabry Online Education tool
 - <https://mpssociety.org.uk/professionals/fabry-education-tool>

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