

# Maximising engagement through feedback: insights from shared decision-making toolkit for Fabry disease patients

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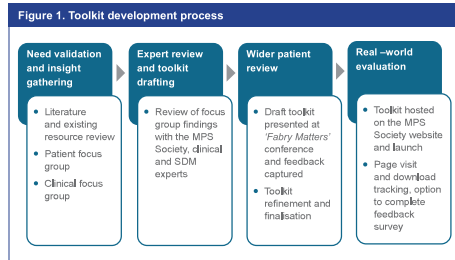
## Background

- Fabry disease (FD) is a rare X-linked lysosomal disorder, characterised by a deficiency of α-galactosidase enzyme, caused by mutations in the galactosidase-alpha (GLA) gene.<sup>1</sup>
- An accumulation of glycosphingolipids (Gb3 and lyso-Gb3) leads to multisystemic disease, reduced quality of life (QoL), and limited life expectancy.<sup>1,2</sup>
- Patients are managed with targeted therapies (enzyme replacement therapy and chaperone therapy) to prevent/delay disease progression; and supportive care for the management of associated symptoms.<sup>1</sup>
- With an increasing number of treatment options for FD it is essential that patients and healthcare professionals (HCPs) collaborate to reach decisions on care, to optimise QoL and health outcomes.
- This collaboration, referred to as **Shared Decision-Making (SDM)**, is based not only on available evidence, but the patient's individual preferences, circumstances, values, and beliefs.<sup>3</sup>
- It has been established that people want to be more involved in their care decisions, and those with long-term health conditions are least likely to feel involved.<sup>4</sup> SDM recognises a patient's right to make decisions about their management and care and provides a means to ensure they are fully informed about their options.
- Here we outline the co-creation of an SDM toolkit for patients with FD and present our findings on engagement with the SDM toolkit in the 4-months following its launch.

## Toolkit development

The toolkit development process is shown in Figure 1.

- At each stage of the development, the toolkit was co-created by patients with FD and FD specialists, with involvement of other key stakeholders including a patient organisation (the Society for Mucopolysaccharide Diseases UK – the MPS Society), and clinical and SDM experts.



### Need validation and insight gathering

- A literature search was conducted to identify FD SDM support materials or patient decision aids and any previous research in FD on:
  - Experience, priorities and unmet needs in decision making
  - Development of SDM support materials
  - Application and evaluation of SDM
- Insights were gathered during two virtual focus groups, one involving:
  - Eight individuals with FD (recruited by the MPS Society), for which written consent was obtained from all participants, which took place on 28 September 2023
  - Four FD specialist consultants and one nurse specialist, from four UK specialist metabolic disease centres, which was held on 29 January 2024

### Expert review and toolkit drafting

- Findings from the two focus groups were presented to the MPS Society and clinical and SDM experts to ensure the insights represented the wider patient and clinical experience and to define the focus and content of the toolkit.

### Wider patient review

- The draft toolkit was presented to a Fabry patient audience at the 'Fabry Matters' conference, 1–3 March 2024, hosted by the MPS Society.
- In total, 57 audience members with FD completed a live feedback survey during the presentation, captured via the conference app (with informed consent prior to participation). Further feedback was received during the question-and-answer session.

## Engagement with the toolkit from the Fabry community during its development

*"A very helpful tool to organise ideas and prompt questions"*

*A person living with Fabry*

*"Think it's a great way of getting people thinking about what to mention in next meeting, and how to explain it"*

*A person living with Fabry*

*"As a father and husband to people who suffer from Fabry disease, it is hard to overestimate the importance of better understanding your condition and the options available to you. Navigating the national health system to find the best care is unfortunately a necessary additional burden for many people suffering from rare diseases. This helpful toolkit allows you to take more ownership for your own care, focusing your engagement with the health service on what matters most to you and helping you make informed choices around that"*

*A trustee of the MPS Society*

## Outputs

- The **'Living Well with Fabry: A Shared Decision-Making Toolkit'** was refined and finalised (Figure 2).
- Available in print (for distribution by HCPs within clinics) and an editable version for use on electronic devices, the toolkit is supported with documentation to facilitate ease of use and adaptability (Figure 3).
- While designed in English, with culturally appropriate translation it could be usable in any healthcare context.

Figure 2. Living Well with Fabry: A Shared Decision-Making Toolkit



Figure 3. Living Well with Fabry: A Shared Decision-Making Toolkit – Supporting documentation

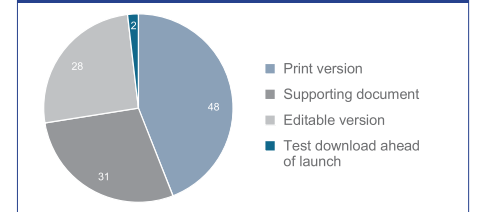


## Engagement with the toolkit via the MPS Society website

- The toolkit was launched on the MPS Society website on 23 August 2024, with website analytics (number of views and downloads) tracked to 24 December 2024, a period of 4-months.
- We are continuously collecting feedback to improve the usability and to determine the impact of the toolkit on patient's lives.
- Users who have accessed the toolkit via the MPS Society website have the option to register their interest to complete a short survey to provide their feedback.
- In the 4-months post-launch, a total of **269 unique visitors** accessed the resource page on the MPS Society website out of **313 total visits**.
- The top points of access were direct links (195), LinkedIn (20), and Facebook (3).
- Of these visitors, **68 individuals** downloaded a version of the toolkit, resulting in **109 total downloads**.
- The **most downloaded** version was the **print format** (Figure 4). Supporting documents were also accessed, contributing to overall engagement.

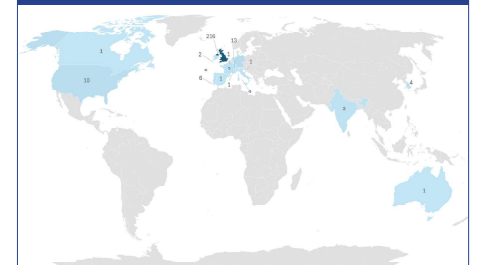


Figure 4. Living Well with Fabry: A Shared Decision Making Toolkit – Number of downloads



- Despite this being a UK resource, developed by UK experts and shared through a UK patient group, the toolkit has achieved a geographical reach to **16 countries**. Most visitors have come from the UK (216) then Germany (13) and the United States (10) (Figure 5).

Figure 5. Number of visitors to the toolkit page by country



## Social media

- To build awareness of the campaign, Chiesi posted about the SDM toolkit on LinkedIn.
- In total, the post garnered **1,618 impressions**, **130 engagements** and **82 clicks**.
- Notably, there were **9 reposts**, demonstrating positive engagement and content dissemination.

## Conclusions

- The **'Living Well with Fabry: A Shared Decision-Making Toolkit'**, co-created by patients with FD and FD specialists, is designed to empower patients to actively participate in their care decisions and addresses an unmet need in FD management.
- In the 4-months following its launch, engagement with the toolkit's landing page on the MPS Society website has shown promise.
- Increased engagement increases empowerment within the FD community and creates an awareness of the importance of the SDM process.
- We are currently collecting ongoing feedback and data on actual usage of the toolkit via a survey, results from which will contribute to its refinement and optimise decision-making processes for FD patients.

**References:** 1. Mehta A, and Hughes DA. (2002). Fabry Disease. In MP Adam (Ed) et al. GeneReviews<sup>®</sup>, University of Washington, Seattle. 2. Morales M et al. Orphanet J Rare Dis. 2024;19:389. 3. National Institute for Health and Care Excellence. NICE. Shared decision making. Available at: <https://www.nice.org.uk/guidance/ing197/resources/shared-decision-making.pdf> (Accessed December 2024). 4. Care Quality Commission (CQC). Better care in my hands: A review of how people are involved in their care. Available at: [https://www.cqc.org.uk/sites/default/files/2016/05/19\\_Better\\_care\\_in\\_my\\_hands\\_FINAL.pdf](https://www.cqc.org.uk/sites/default/files/2016/05/19_Better_care_in_my_hands_FINAL.pdf) (Accessed December 2024).

**Disclosures:** Tom Kenny, Katy Bunn and Kamran Iqbal are full-time employees of Chiesi. Stuart Gaffney is a former employee of Chiesi.