Living well with Fabry: A Shared Decision-Making Toolkit
Supporting documentation

This toolkit has been funded by Chiesi Limited and developed in collaboration with Rare Disease Research Partners.

WHAT IS SHARED DECISION MAKING?

Shared decision-making (SDM) Is a collaborative process that involves a person and their healthcare professional working together to reach a joint decision about care, involving choosing tests, treatments, management or support packages based both on evidence and on the person's individual preferences, beliefs, and values.

SDM enables a person to be at the centre of their care decision-making.[1] It is appropriate in any non-life-threatening situation in which a decision needs to be made.

Why does SDM matter?

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.[2] 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.

How can SDM benefit patients?

- Improved patient experience: By allowing people to choose their level of involvement in the management of their care and empowering them to make decisions aligned with their priorities.
- Improved patient satisfaction and confidence: Studies have found that SDM conversations result in increased patient satisfaction and reduced decisional regret. [3, 4]
- Improved knowledge of condition and management strategies: The use of SDM aids has been shown to improve people's knowledge of their condition and of the benefits and risks associated with their choices.[5] It provides a framework for individuals to get the information they need to make the right choice for them.
- Improved adherence to management recommendations: Research spanning conditions such as asthma[6], diabetes[7] and multiple sclerosis[8] indicates that when people are involved in making decisions about their care they are more likely to follow the management strategies recommended by their doctors.

Does SDM improve health outcomes?

There is also a growing evidence base which suggests that participating in discussions surrounding your care leads to improved outcomes.[3]

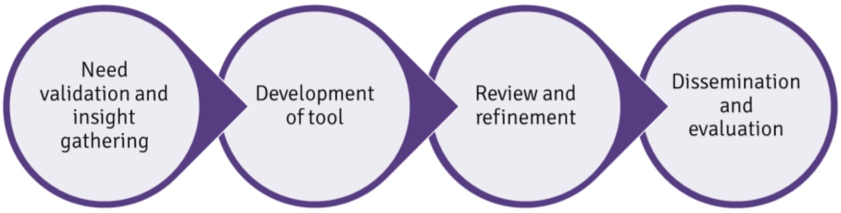
Why is SDM important in Fabry?

Fabry disease is a rare, life-limiting disease characterised by a deficiency of α-galactosidase A enzyme causing progressive multisystem organ dysfunction. Its management may include Fabry-specific therapies and/or symptom management. Given the rarity and complexity of Fabry disease coupled with evolving therapeutic strategies, SDM is an important framework to enable patients and their Fabry specialist to align their perceptions on symptoms, incorporate patients' values and preferences into management goals and build a shared understanding of the best approach to support the quality of life of each individual.

DEVELOPMENT OF THE LIVING WELL WITH FABRY TOOLKIT

In designing this toolkit, the aim was to address an unmet need for people affected by Fabry. It would support people to live well with Fabry by empowering them to ask questions about their symptoms, request information and, to the degree they wish to, play an active role in the decisions made about the management of their condition.

The development process



This toolkit was co-designed with patients and clinicians: Insights were gathered during two focus groups, one with 8 individuals with Fabry and the other with 4 Fabry specialist consultants and 1 Fabry specialist nurse working in the UK. The development of this toolkit was an iterative process of review and refinement, incorporating feedback on content, format, and design from focus group participants and a broader patient audience at a Fabry patient conference (where 57 patients provided live feedback during a presentation of the draft toolkit).

Insights[9]	How the insights were used to develop this toolkit
Fabry symptoms that have the greatest impact on patients' lives	
Both patients and Fabry specialists agreed on the Fabry symptoms that have the greatest impact on patients' lives. • **Pain** management is a priority for patients but is challenging and often required regular review and change of management options. • **Gastrointestinal (GI) issues** impact daily life in various ways, affecting school attendance, limiting social activities, and restricting employment opportunities. • **Fatigue** has significant impacts on mood and wellbeing, patients felt drained and controlled by their lack of energy leading to feeling dependent and isolated.	Focus on the three symptoms identified.
Barriers to raising concerns	
People gain experience in managing their symptoms over time but can feel that they have no choice other than to put up with them. • **Fear of being dismissed** due to past experiences of not being believed pre-diagnosis creates a barrier to seeking support or leads people to feel the need to prove and evidence their symptoms to their Fabry specialist. • **Is it Fabry?** People struggle to differentiate Fabry-related symptoms from others, making it difficult to know if they should raise their concerns or decide who to discuss them with. • **Embarrassment** can arise from symptoms, such us GI issues, making people reluctant to discuss them. • **Normalisation** of symptoms that people have experienced since childhood can stop them from seeking help.	Validating these barriers by including them using patients' own language and providing responses that aim to normalise seeking help to give patients confidence to raise concerns about their symptoms and access support to manage them when needed.
Challenges in communicating the impact of symptoms	
It can be challenging for people to recall and articulate the impact of their symptoms and their priorities when talking to their Fabry specialist. Fabry specialists felt that understanding what matters most to people is key to having productive conversations about care.	This toolkit provides prompts and spaces for patients to record their symptoms and encourages them to think about what is most important to them and record this in a way that can support the communication of these issues to their specialist.
Problem-solving	
People often referred to 'solving problems' rather than making decisions on treatment and management of their Fabry. It was often an iterative process involving multiple conversations over time.	This toolkit aims to reflect this approach by guiding patients through decision making processes that leave time and space for reflection and review of decisions. The language used reflects the language used by patients to describe decision making.

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INTRODUCING THE LIVING WELL WITH FABRY SHARED DECISION-MAKING TOOLKIT

What is this toolkit?

The Living Well with Fabry: a shared decision-making toolkit is designed to support individuals with Fabry and their Fabry specialists have effective conversations and make collaborative decisions about their care.

Why should I use this toolkit?

We know that living with and managing Fabry can be difficult and sometimes it is not always clear what the right path for you is. Drawing from the experience and knowledge of both people living with Fabry and Fabry specialists, this toolkit can help you discuss your concerns and navigate your Fabry care with confidence. With this toolkit, you can:

- **Take charge of your Fabry care:** When we asked people living with Fabry we found that 57% had less say in making decisions about their care than they would like.[9] This toolkit can help you to feel prepared to play an active role in your care.
- **Feel empowered and confident to raise and discuss your health concerns:** 58% of patients we spoke to felt that this toolkit would encourage them to talk to their Fabry specialist if they were concerned about something.[9]
- **Help your healthcare team better understand your needs and preferences:** This toolkit is designed to help you think about the things that matter most to you and support you to effectively communicate this to your specialist, ensuring that your voice is heard, and your care reflects what is important to you.
- **Feel informed about your condition and care:** This toolkit can help you to ask the right questions to get the information you need to manage your Fabry and make decisions about your care.

WHAT THE FABRY COMMUNITY HAS TO SAY …

"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

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This toolkit can be used to support any discussions you have with your Fabry specialist about your care. It mainly focuses on the top three symptoms that people living with Fabry identified as having the most significant impact on their daily lives.[9] Bring this toolkit along with you to your next appointment and talk through your answers with your Fabry specialist.

This toolkit is designed to be personalised and flexible

This toolkit is designed to suit your needs, depending on your situation you may want to use all or only some of the pages. There are 3 different versions of this toolkit to choose from:

- Editable pdf (a file that you can edit and save on your laptop, tablet or phone)
- Printable pdf (to print and fill out by hand)
- Large print option for visual impairment (available in both the editable and printable formats)

Prepare	The following pages of this toolkit can help you to prepare for your next appointment with your Fabry specialist, work through these pages before your next discussion and keep a note of any questions you have on the pages provided.	**Page 3: 'Talking about your symptoms'** can help if you are not sure whether to talk to a doctor about your health concerns or symptoms or you are worried about whether your specialist will listen to your concerns **Page 4: 'Keeping track between appointments'** can be used if you are worried about changes in your symptoms or you are struggling to remember everything you would like to discuss with your specialist **Page 4: 'How your symptoms are affecting you'** can help if you find it difficult to describe how your symptoms are affecting your daily life
Discuss	Some things are not easy to talk about, even when they are impacting your daily lives. These pages can help you to talk to your specialist about three key symptoms of Fabry.	**Page 7 & 8: Talking about pain** **Page 9 & 10: Talking about stomach and digestive issues** **Page 11 & 12: Talking about fatigue**
Decide	After talking to your specialist you may have a choice to make about your care. It can be difficult to know what the right option is, the following pages can help you to make a choice that is right for you.	Fill out **Page 14 'Making choices about your care'** before you discuss your care options with your specialist to help you think about what you need to make a choice about Fill out **Page 15 & 16 'Making choices about your care'** with your specialist to help you think about your options when you have a choice to make

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How to use this toolkit: Guidance for Fabry specialists

This toolkit aims to support conversations between you and your Fabry patients.

We found that patients often encounter challenges discerning Fabry related symptoms from other health issues and in describing the impact of their condition. This toolkit aims to support your clinical practice by facilitating discussions about key symptoms and related concerns and empower patients to actively participate in their care.

This toolkit provides a framework to foster patients' autonomy and make the best use of their consultation time by supporting them to consider their preferences and priorities in advance to ensure that decisions are aligned with their individual needs and values.

Prepare	These pages can help your patients prepare for consultations, encourage them to work through these pages before their next appointment and note any questions they have in the pages provided.	**Page 3: 'Talking about your symptoms'** can help patients to feel more confident in raising concerns about their **Page 4: 'Keeping track between appointments'** can help patients to keep a record of changes in their symptoms and anything concerns they would like to discuss between appointments **Page 6: 'How your symptoms are affecting you'** can help patients to understand and articulate how their symptoms impact daily life and their care priorities
Discuss	This toolkit focuses on the 3 symptoms that patients told us impact their everyday lives the most. These pages are designed to support your conversations with patients about these symptoms.	**7 & 8: 'Talking about pain'** **Page 9 & 10: 'Talking about stomach and digestive issues'** **Page 11 & 12: 'Talking about fatigue'**
Decide	These pages are designed to support SDM conversations between you and your patient. The toolkit can help patients to prepare for decision making conversations, take notes and reflect on their options.	**Page 14: 'Making choices about your care'** can help patients to prepare for decision making conversations and consider what they need to make a decision **Page 15 & 16: 'Making choices about your care'** provides a framework for patients to consider the benefits and risks of their options and understand how each option would impact their quality of life

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