Collaboration is key: creating a clinical toolkit for the GM2 gangliosidosis community with, and for, clinicians, patients and caregivers in the United Kingdom

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RATIONALE

For patients and their families, understanding the mechanisms of a rare disease is, despite the clear benefits, often challenging and confounded by confusion and uncertainty following the diagnostic process [1,2].

In the UK, The Cure & Action for Tay-Sachs (CATS) Foundation, an advocacy group, promotes better understanding of these mechanisms in GM2 gangliosidosis for affected patients and families. Despite this work, at present, there is a paucity of simple and accessible information explaining the underlying mechanisms of the condition[2].

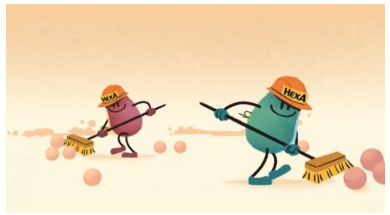
This project aimed to develop a unique online information resource for people diagnosed with GM2 gangliosidosis, and their families, to support better understanding of the disease.

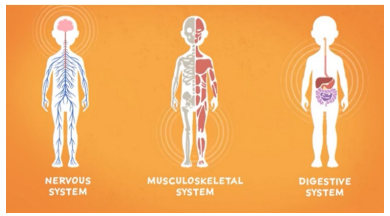
METHODS

The Toolkit was developed and devised in collaboration with key stakeholders, convened by the CATS Foundation, including KOLs, clinicians, patients, and their families. The Clinical Toolkit for Tay-Sachs and Sandhoff disease provides information about the diseases, how the symptoms can be managed, and simple signposting to navigate families to the support so often needed. The toolkit comprises bespoke video content, using lay language, to explain the disease mechanisms simply to non-scientific viewers.

By adopting an established co-creation methodology, called the Double Diamond approach, the toolkit was designed in collaboration with key stakeholders to ensure that information was in the right format, used appropriate language and contained meaningful information shaped by information gaps identified through lived experiences. Video content was also designed with families and included jointly refining the content and subsequent look and feel of animated content.

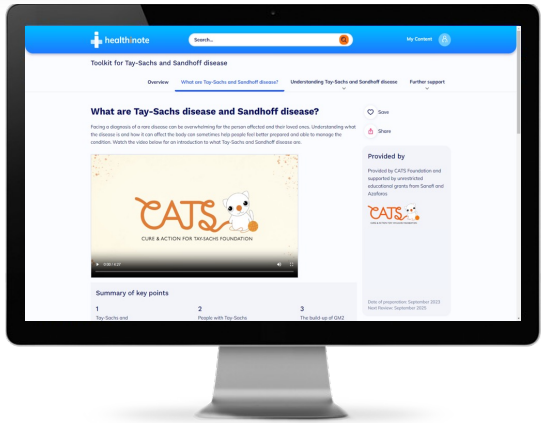
The toolkit was initially made available to families via The CATS Foundation website in December 2023, with healthcare professionals working with the GM2 community signposting to the digital resource.





Visual, engaging and easy to understand: Animated content on the Toolkit uses simple analogies to translate complex pathologies into easily accessible information

Online information resource for all: The Toolkit was designed to be accessible to clinicians, patients and their families, to educate and inform all individuals affected by Tay-Sachs or Sandhoff disease.



RESULTS & NEXT STEPS

The impact of this digital intervention was assessed through patient feedback and engagement metrics. Since initial dissemination to April 2024, 158 unique users viewed the Toolkit a total of 193 times. Overall, users spent an average of 4 minutes, 50 seconds viewing the toolkit and the most viewed pages focused on information about the diseases, symptoms and available clinical studies.

158 unique users accessed the Toolkit during the pilot phase

Highly visual content explaining GM2 was most viewed by users

The Toolkit will remain available to the GM2 community and will be iteratively developed following user feedback. Subject to future funding, the toolkit will be translated to additional languages.

References

1. Long JC et al. Needs of people with rare diseases that can be supported by electronic resources: a scoping review. BMJ Open 2022;12:1-10
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COGNITANT

CATS CURE & ACTION FOR TAY-SACHS FOUNDATION

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