Empowering the GM2 gangliosidosis community: Developing a single clinical toolkit for clinicians, patients and caregivers in the UK

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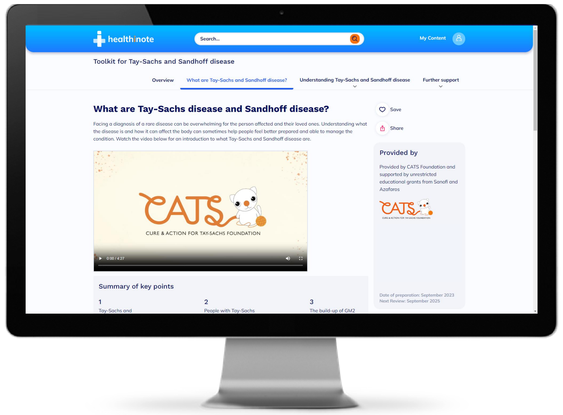
Visit the toolkit here!

RATIONALE

For patients and their families, understanding the mechanisms of a disease is important for disease awareness and management, but is often challenging - confounded by confusion and uncertainty during the diagnostic process.[1,2] Providing simple, accessible and reliable information to orient families at the point of diagnosing a rare disease can be difficult, owing to the low prevalence and subsequent lack of widespread knowledge, even amongst clinicians.[1,2]

The Cure & Action for Tay-Sachs (CATS) Foundation, a patient advocacy group in the United Kingdom, supports patients and families affected by GM2 gangliosidoses to understand these mechanisms.



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.

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

METHODS

By working in collaboration with key stakeholders including KOLs, clinicians, patients and their families, the CATS Foundation devised and developed a unique online information resource to help aid understanding of GM2 gangliosidoses.

Primary qualitative research was conducted in the form of two focus groups – one with healthcare professionals and advocacy groups, and another with the patients and caregivers affected by GM2 gangliosidoses. These focus groups sought to firstly understand the unmet informational needs at different timepoints in the patient journey, and secondly begin to co-design a solution to these issues.

'The Toolkit for Tay-Sachs and Sandhoff disease' launched in September 2023, and platform engagement data was analysed to understand initial engagement.

RESULTS & NEXT STEPS

Since initial dissemination to December 2023, 134 unique users viewed the Toolkit 168 times. Average session duration was 6 minutes and 49 seconds, and the most viewed content included animated content (pictured left) explaining what GM2 gangliosidosis is using highly visual content and simple analogies.[3]

134 unique users accessed the Toolkit during the pilot phase

The average session duration was **6 minutes and 49 seconds**

Highly visual content explaining GM2 was most viewed by users

Engagement data and anecdotal user feedback suggest high acceptability of the Toolkit. This reaffirms the need to provide reliable, multi-format, and accessible information to healthcare professionals, advocacy groups, and affected families. Further evaluation is underway, and the Toolkit will be iteratively developed based on user feedback.

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
2. EURORDIS. 2009 The voice of 12,000 patients: Experiences and expectations of rare disease patients on diagnosis and care in Europe. France: Boulogne-Billancourt
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COGNITANT

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