

Delivering the patient voice: XLH and the NICE HST appraisal process

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Summary

The experience of patients and families is an important aspect of many Health Technology Assessment processes. This process is often referred to as patient voice. The challenges and opportunities of supporting the voice of rare condition patient communities has been highlighted through our work with the X-linked hypophosphatemia (XLH) community. Patient Advocacy Groups (PAGs) require resources and support to enable them to provide guidance for patients for effective and impactful engagement in HTA processes.

Background

"The patient evidence is particularly important in rare diseases when there is often little published evidence about the impact of the condition. It helps the committee understand what it is really like to live with the condition or care for someone with the condition and sheds light on the time commitment required by families when caring for someone with a rare disease." - Former Associate Director, The [National Institute for Health and Care Excellence](#) (NICE)

Whilst NICE is a leading Health Technology Assessment (HTA) agency in terms of patient engagement with established process in place to ensure patient participation in HTA, there remain significant barriers to be overcome if the benefits of patient involvement and their voice are to be fully realised and heard. PAGs and individual patients are often not well resourced or able to access the required expertise to engage at a technical level.

NICE appraised Burosumab (Crysvita®) to treat X-Linked Hypophosphatemia (XLH) in children and young people in 2018.¹ XLH is a disease that primarily affects bones, muscles, and teeth due to the inability of kidneys to reabsorb phosphate causing its excessive loss. This example of the NICE Highly Specialised Technologies (HST) process highlights the barriers and opportunities associated with ensuring the patient voice informs HTA decisions.

Challenges facing rare disease communities:

Rare disease communities have few patients who are geographically scattered. They can be isolated and hard to locate making it harder for patients to share experiences. Many rare diseases do not have dedicated PAGs to support them. PAGs and individual patients usually have limited understanding of HTA, particularly when no previous treatments have been through an HTA process. Rare disease PAGs are often not well resourced or able to access the required expertise to engage effectively in HTA – whilst funding may be available from pharmaceutical companies this can raise concerns around perceived conflicts of interest that may make it difficult for PAGs to accept available funds.

Innovative solutions:

Metabolic Support UK is an umbrella organisation dedicated to supporting patients and families affected by inherited metabolic disorders, particularly those which do not have dedicated PAGs in place. This role has allowed MSUK to develop experience over a number of HTAs and to access some resources to support activities to inform HTA processes. MSUK was able to ensure strong patient engagement in the NICE HST assessment of Burosumab for XLH, using innovative approaches to capturing patient experience in a way that could effectively inform the HTA. Metabolic Support UK:

- Held Patient Days to bring the XLH community together to discuss their issues and share their knowledge.
- Captured "Video Evidence" allowing patients unable to travel to provide their expert perspectives.
- Used online platforms to facilitate data collection from wider communities.

By using a combination of face to face and online engagement, MSUK were able to describe the impact of XLH on patients and families, particularly the pain, fatigue and impact on everyday activities that was poorly captured by common quality of life metrics such as the EQ-5D. This engagement also allowed the HST committee to form a better understanding of the limitations of conventional treatment for XLH. The conventional treatment was demanding and complex; corrective surgical intervention was often needed and while they corrected any deformities, patients continued to live with joint and bone pain. Parents and patients were able to describe the impact of XLH on children's attendance at school, with effects on their education and the significant improvements in their symptoms, emotional state, independence and involvement in sporting activities and social events since starting treatment with Burosumab. These factors were highlighted in the final NICE guidance.

- April 2017: NICE HST Approval Process starts
- July 2017: MSUK Patient Day
- Nov 2017: International XLH Patient Meeting
- Dec 2017: MSUK recognised as official consultee & NICE Scoping Document published
- Jan 2018: MSUK Patient Survey
- Feb 2018: Expert Patient Recruitment & Patient Survey submitted
- May 2018: NICE Patient Evaluation Meeting
- June/July 2018: NICE reject Burosumab, MSUK assembles Patient Views for Submission & Patient HST Evaluation Meeting
- Aug 2018: Final NICE Evaluation
- Sept 2018: NICE approve Burosumab

Conclusion

The value of patients' contribution and the role/impact that their insight provides need to be clear and recognised. HTA processes are highly complex and jargon laden. Policy makers need to understand the role of PAGs in supporting patients to understand and engage with the intricacies of the NICE appraisal processes. PAGs need to be able to access appropriate independent guidance, skills and resources to effectively engage with HTAs.

Acknowledgement

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References:

1. National Institute for Health and Care Excellence. Burosumab for treating X-linked hypophosphataemia in children and young people. <https://www.nice.org.uk/guidance/hst8> (accessed 15 April 2020).