NICE final draft guidance recommends new treatment for primary hyperoxaluria type 1 (PH1)

NICE has published final draft guidance which recommends Lumasiran (Oxlumo) for treating primary hyperoxaluria type 1 (PH1), a rare and potentially life-limiting inherited metabolic disorder affecting less than 100 people in the UK.

In PH1, the liver makes too much oxalate. Usually, this compound is present in small amounts, and as it isn't used by the body, it is filtered by the kidneys and removed in the urine. For those with PH1, the compound is continuously overproduced which means that the kidneys can't keep up with the removal process. As a result, oxalate mixes with calcium to form crystals which are harmful and if left untreated can lead to end-stage kidney disease as well as affecting other areas of the body.

Lumasiran (Oxlumo), made by Alnylam Pharmaceuticals, is the first treatment which targets the underlying cause of PH1. It uses a 'gene silencing' technique to target 'glycolate oxidase', one of the liver enzymes responsible for the overproduction of oxalate. The treatment reduces the production and subsequent accumulation of oxalate, thereby preventing long-term complications.

PH1 is a serious condition affecting people of all ages. It can be particularly devastating in infants and young children who generally have the most severe disease outcomes and whose kidney function can deteriorate rapidly, resulting in urgent hospital admission. Today's announcement is a welcome advance in the treatment of this disease that has life-long physical and emotional implications for patients, their families, and carers. This decision will provide much-needed reassurance for patients and their families that an effective option to manage their condition is available.

Metabolic Support UK was in the privileged position of representing the views of the PH1 community throughout the decision-making process. We would like to thank all those who shared their experience of life with PH1. This vital insight provided a clear understanding of the impact of PH1 and allowed us to produce our valuable resource 'Understanding Patient & Carers Experiences with Primary hyperoxaluria type 1 (PH1) and Lumasiran' which can be viewed here https://www.youtube.com/watch?v=SuI6vLf9h_8&t=276s .

We would also like to extend our gratitude to the wonderful team at the Oxalosis and Hyperoxaluria Foundation (https://ohf.org/) for their support and assistance in the engagement and insight work required for this appraisal.