Final Draft Guidance Recommends Asfotase alfa (Strensiq) for use for the treatment of Paediatric-onset HPP

Metabolic Support UK are delighted to share that following a second committee meeting, NICE have now released final draft guidance to recommend the use of Asfotase alfa (Strensiq) for the treatment of all patients with perinatal and infantile forms of paediatric-onset hypophosphatasia and additional recommendations for those with juvenile forms who meet the entry requirements set by the Managed Access Agreement.

Metabolic Support UK have been representing the Hypophosphatasia community throughout this process, which has been ongoing since 2014, three years before the 5-year Managed Access Agreement was implemented in 2017. This started with working to identify those living with hypophosphatasia (HPP) from various clinics across the UK and building a community to improve peer support in addition to providing a space for learning and informing those who may be eligible to access Asfotase alfa (Strensiq) in the future. We were delighted that this community grew to become established as Soft Bones UK led by Meryl Chambers, a parent of a child with HPP for whom Strensiq has been lifesaving. Meryl was the first of our 'patient experts' who fed her experiences into the NICE process and continued to support Metabolic Support UK leading on the work going forwards.

The entirety of the Managed Access Agreement and the consultation period has been a huge learning process for everyone involved. We are grateful for the time dedicated by those living with HPP and parents and carers of children with HPP, who have shared their views, perspectives, and experiences with us. We valued the honesty from people sharing their uncertainties and questions too. We know each stage of this has meant unpicking what it has meant for those living with or caring for someone with HPP, and we have used our conversations to work alongside NICE, NHS England, the pharmaceutical company (Alexion), and consultants in inherited metabolic bone disorders to develop resources, webinars, and advice to provide clarity and information and to try and alleviate some of the anxiety felt along the way. This has been a joint effort by everyone involved and we are grateful to all those who have shared their expertise, knowledge, and guidance.

The input from the HPP community has been of particular importance in the later stages of this consultation, allowing us to represent 'the patient voice' in our submissions and during the first and second committee meetings. The outcome of the first committee meeting in October 2022 was to recommend the use of Asfotase alfa (Strensiq) for the treatment of perinatal and infantile forms only, meaning only those with symptoms presenting under 6 months of age would be eligible for treatment. We know that for this age group, Strensiq has been life-saving and has changed future outcomes significantly. For those over the age of 6 months, Strensiq has been life-changing and for those receiving treatment through the Managed Access Agreement the physical and psychosocial benefits have been far-reaching. The openness with which the HPP community has shared the effects of HPP, and the impact of treatments has been monumental for such a rare disorder, and this has helped towards changing the decision to encompass all those with paediatric-onset forms.

Throughout the latest stages of the consultation we were pleased to be joined by our nominated 'patient expert' Melanie Williams, who as a person living with HPP, as well as having a daughter and a granddaughter with HPP, has vast experience. Mel's knowledge, eloquence, and strong passion for advocacy and awareness-raising has had a massive impact on this process and we'd like to thank Mel for her unwavering energy and drive which contributed to getting this treatment over the line.

"As your patient representative on the NICE panel it was a great relief to firstly hear the news that perinatal and infantile onset patients would be able to receive Strensiq in the UK. But what a blow it was to hear that NICE was not minded to recommend for Juvenile onset. Then came the call to action from Metabolic Support UK for us to present our evidence to fight for Juvenile onset patients. Thank you so much for completing the questionnaires and writing your stories. This gave us the ammunition we needed to fight our corner. The process wasn't simple by any means and many weeks of work writing papers and learning went on behind the scenes to collect the varied evidence needed by NICE to be able to approve the medication. To hear we have approval for Strensiq for Juvenile onset HPP is monumental. As I live with HPP, have a daughter and granddaughter with the condition I know the toll this disease takes on not only the sufferer but on those caring for us too. I breathe a huge sigh of relief at this wonderful news and look forward to brighter futures."

The next stages of the process are underway, a Final Evaluation Determination (FED) has been submitted by the Committee. This has been shared with consultees to provide the opportunity to appeal or correct factual errors. Subject to any appeal, the FED may then be used as the basis for the guidance on the use of the treatment in the NHS. We know that people will have questions and uncertainties as this process concludes, and we will be working alongside our colleagues to provide this information in due course. However, in the meantime, if you do have any questions or anything you would like to discuss, please do contact Helen Morris at helen@metabolicsupportuk.org or by phone 0845 241 2173 who will be happy to help.