Hi there!

This is our first Managed Access Agreement (MAA) update newsletter especially for those who are receiving Asfotase alfa (Strensiq) for the treatment of paediatric-onset HPP. We have developed this newsletter to share news, to provide opportunities to participate in events, and to learn about ways to share your voice which is vital in the NICE decision-making process.

Managed Access Agreement Update

Who are we?

This newsletter has been produced by Metabolic Support UK who together with Soft Bones UK are representing the voices of HPP patients and their families in the NICE decision-making process.

Using our unique positions this joint collaboration between both groups looks to strengthen not only your voice but also the support and accessibility to information throughout the MAA.

What's going on?

Asfotase alfa has been recommended for use in patients with paediatric onset HPP by NICE in accordance with a 5 year Managed Access Agreement (MAA) which allows time for further data to be gathered to enable an informed-decision to be made.

The MAA lasts for five years. Towards the end of the MAA period, NICE will review all the patient data that has been collected and will make a new recommendation before 2nd August 2022.

If NICE does not recommend asfotase alfa for NHS funding, NHS England funded treatment may stop for all patients. If NICE recommends asfotase alfa for further NHS funding, then asfotase alfa will continue to be funded in England according to the arrangements between NICE and NHS England at that time.

You can read full information by clicking below:

Click Here
Our new consultation period will incorporate a number of virtual events that provide vital opportunities to ensure your voice is heard in the NICE decision-making process. We are therefore inviting you to participate in surveys, interviews and/or discussion groups which will enable us to provide important evidence that shows the impact of Strensiq on your day-to-day lives.

SURVEY

Take part in our online survey! This will help us to gain an insight into the impact of Strensiq and identify any benefits and challenges. Take the survey by clicking here: Asfotase alfa (Strensiq) - Your Experiences

If you would prefer to complete the survey over the phone, please contact helen@metabolicsupportuk.org

INTERVIEWS & FOCUS GROUPS

Our interviews and focus groups will be with a member of our team and will talk through key points from the survey. This will allow us to gain a deeper understanding of the impact of treatment and for you to share additional thoughts and insights not captured in the survey.

IMPORTANT EVENT ANNOUNCEMENT:

TUESDAY 29TH JUNE 2021

6PM - 7:30PM

Online via Zoom

MAA INFORMATION WORKSHOP

This important workshop will aim to provide clarity on the terms of the Managed Access Agreement. The event will be attended by NICE representatives and will be an opportunity to learn about the next steps in the decision-making process and what each stage means for you. During this event you can also find out why your voice matters and how you can contribute meaningfully to the process.

If you or your child is taking Strensiq, we highly recommend attending this meeting as it will contain important information about decisions which may affect your future treatment.

To register for this unique event and to put forward your questions please email Helen Morris on helen@metabolicsupportuk.org or call 0845 241 2173
Soft Bones UK Hypophosphatasia Foundation was founded by Meryl, mother of Maddox, who was born with Hypophosphatasia (HPP). Although Meryl and her family were supported by various existing charities it soon became apparent that there was very little knowledge about HPP and no specific support group in the UK.

Soft Bones UK’s aids are to raise awareness of HPP, to determine early diagnosis, and to support and advocate those diagnosed with HPP and their families in the UK.

You can stay up to date with all news and events, MAA developments and access peer support on the Soft Bones UK Facebook page by clicking here:

Metabolic Support UK is an umbrella patient advocacy organisation founded in 1981, supporting people living with Inherited Metabolic Disorders (IMDs) and their caregivers. There are approximately 30,000 people in the UK today living with one of over 500 IMDs.

Focusing specifically on the rarest of the IMDs, those which have no other patient organisation or support group, Metabolic Support UK delivers a wide range of support and advocacy services to address unmet needs. Using qualitative and quantitative data generated via various methodologies, our small dedicated team works to proactively identify priority needs and develop evidence-based outputs and programmes to ensure the maximum impact for individual patients, collective patient communities and the wider IMD community.