

Asfotase alfa (Strensiq) for treating paediatric-onset Hypophosphatasia Managed Access Agreement

Patient and carer update May 2021

The National Institute for Health and Care Excellence (NICE) make recommendations about the use of new treatments in England based on the evidence that is presented to them during an appraisal. Where a new drug shows promising potential, but there are remaining questions about how well the drug works, NICE may recommend that the treatment is made available via the NHS for a time limited period as part of a Managed Access Agreement (MAA).

NICE recommended asfotase alfa (Strensiq) for treating paediatric-onset Hypophosphatasia (HPP) for use in England via the NHS as part of a 5-year MAA in August 2017. The MAA enables patient access to Strensiq whilst more information on the treatment is collected from you/your child (via your treatment centre). The information that is collected during the MAA will be reviewed in 2022 by NICE who will then make a recommendation on whether Strensiq will continue to be available to access via the NHS after August 2022 (the end date of the MAA).

We would like to thank you/your child for your continued participation and support of the MAA data collection over the last year, particularly in light of the many challenges presented by the COVID-19 pandemic. The questionnaires and medical tests that you/your child complete are essential information for NICE's review in 2022.

We are aware that the pandemic caused disruption to in-person hospital visits in 2020. This has resulted in some missing data (in particular, 6-minute walk test (6MWT) data), which is very important for future decisions about the use of Strensiq after August 2022 (the end date of the MAA).

To try and collect as much information as possible on treatment with Strensiq ahead of NICE's review in 2022, **we would encourage you to consider the following steps where it is safe and possible to do so:**

1. Ask your HPP treatment centre about arranging an in-person hospital visit for your next Strensiq review.
 - If you feel anxious about attending hospital in-person, there is no requirement for you to do so. However, please ask your treatment centre about the changes they have put in place to prevent COVID-19 transmission and keep patients and visitors safe. A number of hospitals in England are now COVID-19 free.

2. Where it is safe and appropriate, please ask your HPP treatment centre about whether they can perform the 6MWT as part of your in-person hospital visits.
3. Continue to complete the questionnaire assessments where possible. If you have missed any questionnaire assessments or don't have a future appointment scheduled for your next questionnaire, please contact Metabolic Support UK (details below).
4. If you or your child are awaiting an outcome about whether you can start treatment with Strensiq, please contact your HPP centre for an update in the first instance.
 - If you have not been able to attend an in-person hospital assessment, this should not delay your referral for consideration of treatment. However, please be aware that additional time may be required for the National Authorisation Panel to confirm an outcome for complex adult referrals where medical history of childhood onset needs to be determined.

If you have questions or concerns about this information, please let us know (contacts below).

Metabolic Support UK and Soft Bones UK will provide further information over the coming months about how decisions will be made concerning continued NHS access to Strensiq after August 2022.

A workshop session will be held to give you an opportunity to ask questions about the MAA on **Tuesday 29th June 2021** at **6pm-7:30pm** via Zoom.

For further information, please contact:

Metabolic Support UK
Tel: 0845 241 2173
contact@metabolicsupportuk.org
www.metabolicsupportuk.org

Soft Bones UK
info@softbonesuk.co.uk
www.softbonesuk.co.uk