Elosulfase alfa for treating mucopolysaccharidosis type IVa

Information for the public
Published: 16 December 2015
nice.org.uk

What has NICE said?

Elosulfase alfa (Vimizim) is recommended as a possible treatment for people with mucopolysaccharidosis type IVa (MPS IVa) only if they sign up to the managed patient access agreement in the managed access agreement.

The managed access agreement includes rules for starting and stopping treatment with elosulfase alfa, and for assessing how well the treatment is working. NICE will take the information about how well the treatment works into account when the guidance on elosulfase alfa is reviewed.

What does this mean for me?

If you (or your) child has MPS IVa, and your doctor thinks that elosulfase alfa is the right treatment, you (or your child) should be able to have the treatment on the NHS, providing you sign up to the managed patient access agreement.

Elosulfase alfa should be available on the NHS within 3 months of the guidance being issued. Your doctor should ask you if you are happy for details of your (or your child's) treatment to be collected.

However, if NICE does not recommend elosulfase alfa for NHS funding when a review of the guidance is published after 5 years, it will no longer be available for any patient.

The condition and the treatment

Mucopolysaccharidosis type IVa (MPS IVa), or Morquio syndrome, is a very rare, inherited condition. It is caused by a missing enzyme that the body needs for connective tissue growth. It causes a wide range of symptoms that get worse over time, including breathing problems, joint and skeletal abnormalities, hearing loss, problems with eye sight and heart valve disease.

The aim of treatment with elosulfase alfa (Vimizim) is to replace the missing enzyme.

Source of advice and support

- MPS Society, 0345 389 9901
  www.mpssociety.org.uk

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ISBN: 978-1-4731-1590-3