

Sebelipase alfa for treating Wolman disease

Information for the public

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Sebelipase alfa is available on the NHS. It is a possible treatment for long-term enzyme replacement therapy in Wolman disease (also called rapidly progressive lysosomal acid lipase deficiency) in people who are 2 years or under when treatment starts.

If you, or your child, are not eligible for sebelipase alfa but are already taking it, you should be able to continue until you and your doctor decide when best to stop.

Is this treatment right for me?

Your healthcare professionals should give you clear information, talk with you about your options and listen carefully to your views and concerns. Your family can be involved too, if you wish. See [our webpage on making decisions about your care](#).

Questions to think about

- How well does it work compared with other treatments?
- What are the risks or side effects? How likely are they?
- How will the treatment affect my or my child's day-to-day life?
- What happens if the treatment does not work?
- What happens if I do not want to have treatment or I do not want my child to have treatment? Are there other treatments available?

Information and support

These organisations can give you advice and support:

- [MPS Society](#), 0345 389 9901
- [Children's Liver Disease Foundation](#), 0121 212 3839

You can also get support from your local [Healthwatch](#).

NICE is not responsible for the quality or accuracy of any information or advice provided by these organisations.

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