Migalastat for treating Fabry disease

Information for the public
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What has NICE said?

Migalastat (Galafold) is recommended. It is a possible treatment for some people with Fabry disease who are over 16 and who would usually be offered enzyme replacement therapy.

What does this mean for me?

If you have Fabry disease, and your doctor thinks that migalastat is the right treatment, you should be able to have it on the NHS.

Migalastat should be available on the NHS within 3 months.

If you are not eligible for treatment as described above, you should be able to continue taking migalastat until you and your doctor decide it is the right time to stop.

The condition and the treatment

Fabry disease is an inherited disorder caused by an enzyme called alpha-galactosidase A not working properly. This leads to a harmful build-up of a certain type of fat in the body. This causes a range of symptoms, including widespread pain, and sometimes permanent damage to the kidneys and heart.

Migalastat is a drug that binds to alpha-galactosidase A, helping it to work better in some people with Fabry disease.

These organisations can give you advice and support:

- CLIMB, 0845 241 2173
- Genetic Alliance UK, 0207 704 3141
- MPS Society, 0345 389 9901

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

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Accreditation