

# Vimizim treatment for Morquio A


## The Managed Access Agreement

A guide for patients and parents



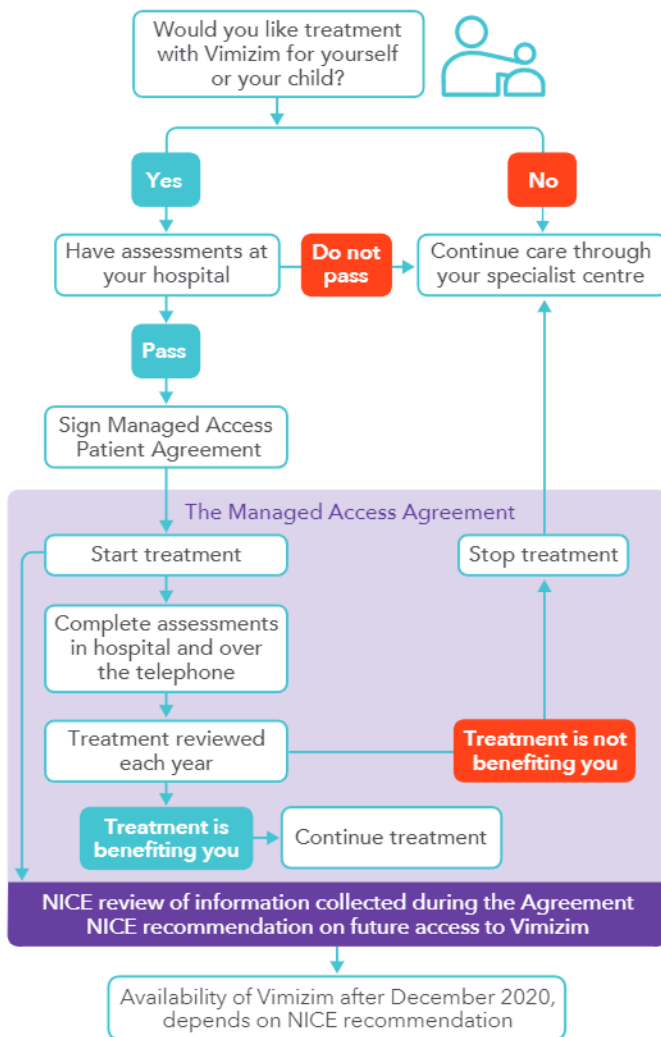
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## About this guide

Treatment for Morquio A in England with enzyme replacement therapy Vimizim is made available through a Managed Access Agreement. This guide explains how this process of obtaining and staying on treatment works.



## Disclaimer

The information contained in this booklet is intended as a guide to the MAA only and you should ensure that you understand the full content of the MAA Patient Agreement before signing it. This document does not provide medical advice, always seek the advice of your consultant with any questions you may have regarding your medical condition.

# Access to treatment with Vimizim in England

## Why we have a Managed Access Agreement

The only treatment currently available for Morquio A is elosulfase alfa (Vimizim), which was approved by the European Medicines Agency on 28th April 2014.

Before making this treatment available through the National Health Service (NHS) in England it was reviewed by the National Institute for Health and Care Excellence (NICE) who assessed both the benefits and costs of Vimizim.

Please note that this is only for England. Treatment decisions for Northern Ireland, Scotland and Wales are made by their own Health Authorities.

When deciding if they would recommend Vimizim as an NHS treatment, NICE felt that they needed more information before making a final decision.

As a result, the Managed Access Agreement (MAA) was developed to make treatment available while more information is collected. The MAA for Vimizim is a five year programme that started in December 2015.

## Content of the Managed Access Agreement

The MAA makes treatment available to those with Morquio A. It sets out who can start treatment and when treatment must stop.

## Access to treatment

If you would like treatment for yourself or your child, assessments will be carried out at your specialist centre.

Those passing the assessments will be asked to sign the Managed Access Patient Agreement. Treatment can then begin.

You or your child will have assessments each year to see if the treatment is of benefit. If Vimizim is benefiting you or your child, treatment will continue. If no benefit is shown, or the conditions set out in the patient agreement have not been met, treatment may need to stop.

The MAA ends in December 2020. Whether or not treatment will be available after this date is currently not known and will depend on a review of the information collected during the MAA and recommendation by NICE.

## The Managed Access Agreement



### You can start treatment if you:

- Complete and pass the hospital assessments
- Do not need a ventilator for breathing
- Do not have another serious, life-limiting condition
- Sign the MAA Patient Agreement



### To stay on treatment:

- Attend all your clinic appointments
- Complete the assessment questionnaires
- Do not miss your infusions
- The results of your assessments must show that Vimizin is benefiting you



### Treatment may stop if:

- You have not attended your clinic or completed your assessments
- You have missed too many infusions
- Vimizin is not benefiting you
- You wish to stop treatment
- There is a medical reason for stopping



### The end of the MAA:

- The MAA ends in December 2020
- The National Institute for Health and Care Excellence (NICE) will decide if Vimizin treatment will be available after this date

## Deciding to join the MAA

### Who can join the MAA?

You will need to discuss this with your consultant. They will run a series of assessments and ask some questions to confirm if you or your child can join the MAA.



Treatment under the MAA is available to those who:

- Have a confirmed diagnosis of Morquio A
- Complete the assessments described on page 6 \*
- Do not need a ventilator for breathing
- Do not have another serious, life limiting condition
- Sign the MAA Patient Agreement

### Can I join the MAA at a later date?

You can decide when you would like to join or like your child to join the MAA and start treatment.

If you want to start treatment at a later date, you will still need to pass the assessments before treatment begins. If by this time, your or your child's condition has changed and you no longer pass these assessments, then you or your child will not be able to start treatment.

\* allowances are made for children under five years of age who may not be able to complete all the tests

Access to treatment may not be available at a later date if the MAA has completed or is no longer enrolling onto the programme. The MAA is currently due to end in December 2020.

## **What is expected of those taking part in the Vimizim MAA?**

You will need to sign a Managed Access Patient Agreement. Staff at your specialist centre will go through the agreement with you. By signing the agreement, you are agreeing to all the conditions set out in the MAA. Please read the agreement carefully so that you understand what you need to do.

Treatment may be stopped if you do not meet the conditions, so do ask your consultant about anything you are unsure of.

You will be expected to:

- Attend all your clinic appointments to complete the hospital assessments
- Complete the assessment questionnaires as required
- Receive weekly infusions of Vimizim, missing no more than three infusions in any 14 month period (with the exception of a break in treatment for medical reasons)

## **How often do I need to attend the hospital?**

When treatment starts, you will need to attend the hospital each week to receive the Vimizim infusion. After this initial period you will move to a homecare package, where infusions will be given by a trained nurse once a week at home, school or in the workplace.

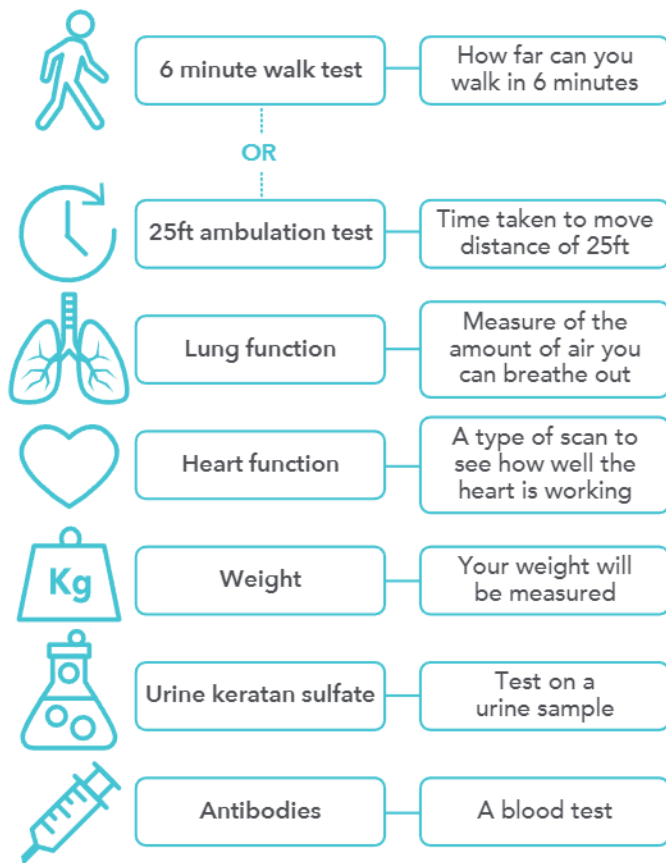
Patients and families are encouraged to learn to become independent or semi-independent with the infusion. Training will be given, please ask your specialist centre for more information on this.

You will also need to attend all the hospital assessments, which currently take place every six months.

## What are the hospital assessments?

These assessments are used to measure the effects of treatment. You or your child will do the following assessments when you attend the hospital:

### Hospital assessments



## What are the assessment questionnaires?

During the MAA you will need to complete a number of assessment questionnaires. These are used to measure the effect of treatment on you or your child's quality of life.

A parent or guardian completes the questionnaires on behalf of children under the age of 12 years.



## Assessment questionnaires

|                |  |
|----------------|--|
| Once a year    | <b>EQ-5D-5L</b><br>A short questionnaire used to measure quality of life   |
| Once a year    | <b>MPS Health Assessment Questionnaire</b><br>A longer questionnaire looking at daily tasks and how much help is needed to do them |
| Every 4 months | <b>Pain questionnaire</b><br>These short questionnaires are completed the day before infusion and again the day after infusion     |
| Once a year    | <b>Beck Depression Inventory</b><br>This is used in those aged 13 years or over to assess mood                                     |

## How do I complete the assessment questionnaires?

Questionnaires are usually completed over the telephone with a member of the MAA team at MPS Commercial (part of the MPS Society). A member of the team will contact you to book a convenient time to complete the questionnaires.

MPS Commercial will telephone you every four months to book the necessary appointments and complete your questionnaires. The first call will take approximately 45 minutes, the other calls will be much shorter.

If English is not your first language, an interpreter will be provided on the telephone call to help you.

# Conditions for receiving treatment on the MAA

## To stay on treatment



Attend all your clinic appointments to complete the assessments



Complete all the required assessment questionnaires



Do not miss your weekly infusions



The results of your assessments must show that Vimizim is benefiting you

To stay on Vimizim treatment you must meet the conditions set out in the Managed Access Patient Agreement.

The conditions are that you attend all your clinic appointments to complete the hospital assessments, complete all the questionnaire assessments and receive weekly infusions.

Treatment with Vimizim also needs to be benefiting you or your child. The results of the assessments are used to measure the benefit of treatment.

## Completing the hospital assessments



While on the MAA you will need to attend your clinic to complete the hospital assessments every six months. These assessments are an important part of the MAA and the results are used to determine if the treatment is benefiting you or your child. The assessments are listed on page 6.

## Completing the questionnaire assessments



You will also need to complete the questionnaire assessments every four months. It is important that all these assessments are completed as the results of these are also used to assess if the treatment is working for you or your child. The questionnaires used are listed on page 7.

## Weekly infusions of Vimizim



Vimizim infusions are administered once a week. When treatment starts, you will need to attend the hospital each week to receive the Vimizim infusion. After this you will move to a homecare package, where infusions will be given by a trained nurse once a week at home, school or in the workplace.

Patients and families are encouraged to learn to become independent or semi-independent with the infusion. Training will be given, please ask your specialist centre for more information on this.

### **Why is it important not to miss infusions?**

To gain the maximum benefit from treatment it is essential that infusions are not missed. It is also a requirement of the MAA that no more than three infusions are missed in any 14 month period (with the exception of a break in treatment for medical reasons).

### **What if infusions are missed for medical reasons?**

Sometimes it may not be possible to receive an infusion for medical reasons. These could be problems with a port or cannulation for example. If you miss several infusions due to medical reasons, you may be able to continue treatment with Vimizim once the medical issue has been resolved. Discuss this with your consultant who will be able to advise you.

### **Can we go on holiday whilst on the MAA?**

You will not be permitted to take treatment away with you on holiday, however, you may be able to take a short holiday if it means that you will not miss more than three infusions in any 14 month period.

If you wish to take a break from infusions due to a holiday, discuss this with your consultant beforehand to make sure you will not break the conditions of the MAA, as this could lead to treatment being stopped.

### **What if I decide to stop my treatment or my child's treatment, but later decide that I would like treatment to continue?**

Once you have made the decision to stop treatment, you will not be allowed to re-start treatment with Vimizim at a later date while the MAA is running. As yet, the arrangements for after the MAA closes in December 2020 are not known.

## How treatment benefit is measured

- In addition to the conditions outlined above, evidence that
- Vimizim is benefiting you is needed to stay on treatment under
- the MAA.

The results of the hospital assessments completed at your specialist centre and the questionnaire assessments are reviewed each year.

Treatment benefit is defined as passing on four of the five measures below:

1. Mobility – 6 minute walk test or 25ft ambulation test
2. Lung function
3. Assessment questionnaires
4. Urine keratan sulfate level
5. Heart function

Definitions for passing on these measures are detailed in the MAA to ensure that everyone is treated equally and fairly. Your consultant will discuss the measures with you and explain whether they show that treatment is benefiting you or your child.

If exceptional circumstances have temporarily affected any of these measures (e.g. recent surgery), these can be taken into consideration when deciding whether to continue treatment or not.

### Allowances for children under five years of age

As young children may not be able to complete all the assessments, children under five years of age are automatically passed on the measures above until they turn five and are then assessed for treatment benefit like everyone else.

## Stopping treatment

Treatment may be stopped if you do not meet the conditions of the MAA or if the assessments are not showing a benefit of treatment.

There may be other circumstances that mean that treatment would need to be stopped, and you may request to stop treatment yourself at any time.

### Reasons that treatment may be stopped



You have not completed the required hospital and questionnaire assessments



You have missed more than three infusions in any 14-month period



Your assessment results show that Vimizim is not benefiting you



You wish to stop treatment



Medical reasons such as severe reactions to Vimizim infusions or another serious illness

## What if I feel Vimizim is providing no benefit to me or my child?

If you feel that Vimizim treatment is not providing any benefit, please discuss this with your consultant. If you decide not to continue on the MAA, you can withdraw from treatment.

## What if my or my child's Morquio A symptoms get worse?

If you feel that your or your child's symptoms are getting worse while receiving treatment with Vimizim, please discuss this with your consultant.

## Are there any other reasons why treatment may be stopped?

If you or your child is unable to tolerate infusions due to a severe infusion related reaction that cannot be resolved, treatment with Vimizim will need to be stopped.

If another life threatening illness is diagnosed that takes priority, continued treatment with Vimizim may need to be reviewed. Your consultant would discuss this with you before any changes to your treatment were made.

## What happens if Vimizim treatment is stopped?

If treatment needs to be stopped for any reason your consultant will discuss this with you. You will continue to receive care and support from your hospital.

The MPS Society are also able to provide support to you and your family. You can find their contact details on page 13.

## Can I appeal if I or my child are taken off treatment?

You can appeal if you feel that the assessments have been performed incorrectly or information was not collected appropriately. If this happens, you can ask for the assessments to be repeated at another hospital. Any travel and associated costs will be at your expense.

## The end of the MAA



The MAA is scheduled to end in December 2020. Whether or not Vimizim will be available after that date will depend on a review of the information collected on the effects of Vimizim treatment during the MAA. This review by NICE will take place during 2020 and NICE will issue their recommendation by the end of 2020.

## How long will I or my child receive treatment with Vimizim for?

Currently, treatment will be available to those on the MAA until it ends in December 2020, providing the conditions for continued treatment are met.

Beyond December 2020, the ongoing availability of Vimizim will depend on the recommendation that NICE makes based on all the information collected during the MAA.

## Other questions



### **What if I become pregnant or want to start a family?**

If you become pregnant or wish to start a family during your treatment with Vimizim, please speak to your consultant straight away.

### **Will I be kept informed of the final decision made by NICE on access to treatment with Vimizim?**

Your consultant will let you know of any decisions that may change your access to treatment.

### **I have stopped treatment, why am I being asked to continue the questionnaire assessments?**

If you have stopped treatment, MPS Commercial would like to continue the questionnaire assessments with you for research purposes. This research helps us to understand more about the effects of treatment with Vimizim and the course of Morquio A when treatment is stopped. Your participation in this research is voluntary.

## Where to go for further information and support

### **Your consultant**

Talk to your consultant if you are unsure of anything concerning your treatment or the MAA.

### **The MPS Society**

You can contact the MPS Society for support and advice at any time.

Call: **0345 389 9901**

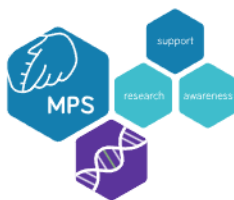
Out of hours support line: **07712 653258**

E-mail: **advocacy@mpssociety.org.uk**

Web: **www.mpssociety.org.uk**

### **MAA documents**

The full text of the MAA is available at: **<https://www.nice.org.uk/guidance/hst2/resources/managed-access-agreement-december-2015-2238935869>**



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MPS Commercial

MPS House, Repton Place, White Lion Road

Amersham, Buckinghamshire, HP7 9LP, UK

☎ +44 (0) 345 260 1087

✉ [info@mpsact.com](mailto:info@mpsact.com)

Production of this guide was supported by BioMarin Europe Ltd.

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