Multi-stakeholder engagement to access to treatment for MPS IVA (Morquio A) – a model for the ultra-rare disease community

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Objectives

To achieve reimbursement for elosulfase alfa for MPS IVA patients resident in England

Background

• MPS IVA is an ultra-rare disease affecting less than 100 patients in England via the MAA

• In 2015, responsibility for the reimbursement decision process for treatments for rare diseases, formerly governed by the Advisory Group for National Specialised Services, was replaced by a joint process involving the Highly Specialised Technologies Evaluation Committee of NICE and the Programme of Care Group of NHS England

The only currently available elosulfase alfa was licensed by the European Medicines Agency on 28th April 2014

• The UK had been a major contributor to the Phase III clinical trial with 35 patients being enrolled out of the 176 recruited worldwide

• Interim funding was not available when elosulfase alfa was licensed and there was a high degree of interest and concern in continuing access to treatment in England

• Although patients who had taken part in the clinical trial continued to receive free drug, other English MPS IVA sufferers had no access to treatment.

Methods

On the 26th November 2014, a 10 year old boy supported by the MPS Society legally challenged NHS England’s scorecard decision method. They applied for an interim funding order for access to Vimizim® (elosulfase alfa) for treatment of MPS IVA in England. Of these, 27 patients previously took part in the clinical trials for elosulfase alfa, and 19 patients are receiving this new treatment for the first time.

Table 1. Response criteria for continued treatment

<table>
<thead>
<tr>
<th>Response criteria</th>
<th>Novel patient (St. 1st year of treatment)</th>
<th>Previously treated patients (2nd year or more on treatment)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Improvement in 15% or 21% Ambition Test</td>
<td>10% Improvement over baseline</td>
<td>Remains 5% above baseline</td>
</tr>
<tr>
<td>Improvement in FEV1 or FVC</td>
<td>75% Improvement over baseline</td>
<td>Remains 2% above baseline</td>
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<tr>
<td>Stabilisation defined as no adverse change in the numerical value in two of the following three measures:</td>
<td>Stabilisation</td>
<td>Stabilisation</td>
</tr>
<tr>
<td>- Quality of life as measured by the EQ-5D</td>
<td>- 3 meetings with the Minister for Life Sciences George Freeman</td>
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<tr>
<td>Reduction in urinary keratan sulfate</td>
<td>20% Reduction from baseline</td>
<td>Remains reduced at least 20% from baseline value</td>
</tr>
<tr>
<td>Decline in section fraction as measured by electrocardiogram</td>
<td>Decline of less than 10% from baseline</td>
<td>Decline of less than 10% from baseline</td>
</tr>
</tbody>
</table>

The MPS Society, BioMarin and a clinical expert

April 2014 UK patient was the first to receive an infusion of elosulfase alpha

January Phase II trial begins, 176 patients worldwide, 35 from the UK

2015

2016

2017

2018

NICE engaged with patients,letal families, MPS Society, media, and other stakeholders


27th January NICE England’s consultation on reimbursement decision making process is launched

26th January NICE England consultation on reimbursement decision making process is launched

21st April NICE drug supply due to end extended to 26th June 2015

30th April NICE decision is received - no formal decision to supply drug for individuals with MPS IVA

16th-17th March First NICE hearing

25th June Free drug supply due

25th June First set of drug ends

2nd July After public consultation on the principles and processes for making investment decisions, NICE England decides to make final funding decisions after NICE HTA appraisal process concluded

2nd-27th July 2nd NICE hearing

3rd September NICE further drug supply conditionally recommends elosulfase alfa with the MAA to generate further evidence through the collection of real-world data directly relevant to patients in the UK. They ask for a protocol for starting and stopping treatment to be developed

5th August Free drug is re-instated for all clinical trial patients

Conclusions

In an environment where health systems are having to choose between high cost drugs and the funding of other health resources, the MAAs, with a confidential financial arrangement, offers all patients meeting the treatment criteria access to reimbursement therapy in the first 12 UK, members of Parliament. The MAA’s will continue to be monitored for disease progression and whilst we are in the first year of this new initiative, MPS IVA patients have embraced the MAAs and recognised that adherence to the MAAs will ensure long-term access to treatment. Only time will tell if the stopping criteria are fair and if patients affected by common disorders will also benefit from similar requirements in the future to ensure equity across all aspects of health.

References


7. NICE. First NICE review of elosulfase alfa for treatment of MPS IVA (Morquio A): a model for the ultra-rare disease community

A patient’s experience of treatment

There have been a couple of signs of Vizmiz doing something. I have been in the garden for the first time in a long time last week and for the first time ever, I saw the legs of a caterpillar! This may seem daft and silly, but due to the clouding of my corneas I have never seen much detail on anything...

A patient’s experience of treatment

Figures

Figure 1. The reimbursement decision process

Figure 2. MPS IVA patients, families, the MPS Society and MPs, campaign for access to treatment

Figure 3. The Managed Access Agreement criteria

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