

HSE Refuse Funding

The HSE have confirmed that they WILL NOT FUND the life changing drug VIMIZIM for those suffering with the ultra rare disease MORQUIO. This announcement comes 3 years after Vimizim gained licensing approval from the European Medicines Agency (EMA) on 28th April 2014.

The Irish Society for Mucopolysaccharide Diseases is very disappointed with this decision on behalf of all patients with Morquio in Ireland who have waited so long for a treatment to be developed and are now being denied access to it.

The HSE states that it has “decided to refuse to reimburse Vimizim in Ireland due to lack of clinical data” in spite of the fact that it is currently funded by over 10 European countries including Northern Ireland. We find this very surprising that the HSE has taken such a stance and based on points that have been well documented and understood in other countries and have not been the cause for refusal elsewhere.

It is proven that Vimizim (Elosulfase alfa) brings multiple benefits: Avoiding/delaying the requirement for invasive ventilation. Improving cardiac function and muscle strength. Improving respiratory function giving improved sleep which reduces sleep apnoea. Lessens chest infections and breathing difficulties and increases energy and stamina. Vimizim also shows improved growth developing stronger posture which benefits mobility and organ function. Vision is preserved by preventing corneal clouding. Pain levels become more manageable.

Collectively, these changes enable those with Morquio to complete normal day to day activities independently and offer the hope of very significant long-term benefits through extended use. One of these very significant benefits is to prolong life expectancy.

Vimizim has been provided compassionately by BioMarin for the past three years to those who participated in the clinical trials. BioMarin has agreed to continue providing Vimizim compassionately until the 05th December 2017. After this date, vulnerable children with this degenerative condition Morquio will no longer have access to this life changing drug.

Vimizim, manufactured by BioMarin in the Republic of Ireland is considered a major employer. This drug is now being reimbursed in more than 10 European countries including Northern Ireland. Thus it is difficult to accept that some patients living on the Island of Ireland have access to this vital drug while others will not.

“We are hugely grateful to BioMarin for their solid and continued support in providing free this drug to those who were involved in the clinical trials and working hard to enable access to Vimizim for

everybody with Morquio. It is also important to note that there is already one child being funded by the HSE which is decidedly unequitable and discriminatory.

We are hopeful that the HSE will see sense, consider the clear positive benefits and make the right decision to ensure continuity of care for everyone and we will work hard for a positive decision.”

End

Morquio (MPS IVA)

Morquio (MPS IVA) is a rare inherited degenerative disease. Patients with the syndrome appear healthy at birth, but within 24-36 months start to show symptoms of severe health problems including heart disease, skeletal abnormalities, difficulty breathing and vision and hearing loss. There is no cure for Morquio, and, untreated, patients rarely live beyond their twenties.

Vimizim (Elosulfase alfa)

Vimizim is the first effective treatment for Morquio. It was developed by the pharmaceutical company BioMarin and was licenced by the European Medicines Agency (EMA) on 28th April 2014 after extensive trials. It is an Enzyme Replacement Therapy (ERT) which overcomes the enzyme deficiency causing the disease.

About the Irish MPS Society

The Irish Society for Mucopolysaccharide Diseases (The Irish MPS Society) is the only registered ROI charity providing support to families affected by MPS and related disease. The Irish MPS Society provides an advocacy service to individual families as well as supporting funding towards innovative and life changing clinical and academic research.

As a registered charity, the Society is entirely supported by voluntary donations and fundraising.

Contact

Mary Boushel

Hon PRO

Irish Society for Mucopolysaccharide Diseases

Telephone: 087 649 3885

email : irishmpssociety@gmail.com