Press release

NICE and NHS England’s decision to deny access to new medicines from 1 April 2017 for those suffering from ultra-rare diseases is not an April Fool’s Joke says the MPS Society

Without a PAUSE to reconsider the impact of this decision children and young adults will be condemned to death

28 March 2017

NICE and NHS England continue to remain silent as their deadline for implementing their decision to introduce and limit cost per QALY for drugs for Ultra-Rare diseases puts patient access to life saving treatments out of reach.

Reeling from NICE’s recent decision to change the arrangements for evaluating and funding drugs and other healthcare technologies assessed through NICE’s highly specialised technology appraisal MPS Society Chairman, Paul Moody said:

“A decision by NICE and NHS England to implement this new policy in just two and half weeks’ will affect the most vulnerable in UK Society and confirms that children and young adults with ultra-rare diseases going forward are economic pawns in a failing NHS and cheaper dead than alive. The UK government now needs to act at lightning speed and reverse this initial NICE & NHS England policy not just in the context of patients with ultra-rare diseases but also that of the life sciences industry who will see no incentive to investing in the UK market if their innovative medicines and technologies have no prospect of reaching the patient.”

Christine Lavery MBE, Group Chief Executive of the MPS Society who very rarely speaks personally said:

“Being born with an ultra-rare disease, a disease affecting less than 110 people in England, is not a life style choice; it is no one's fault; it happens albeit very rarely; it happened to my son. At that time, there was no treatment and Simon died aged 7 years. I can only imagine now how it might be to be faced with a child with an ultra-rare disease who could be treated with a highly-specialised medicine but is denied treatment on cost grounds. The pain for the family of seeing their child condemned to death by Andrew Dillon, Chief Executive of NICE; Simon Stevens, Chief Executive of NHS England and the UK Government is unimaginable. Let us also be clear to Members of Parliament, many of the babies and children who will be affected by this catastrophic decision are ‘yet to be born or diagnosed' members of your constituencies.”
This decision is particularly devastating to the MPS Society who felt they had worked constructively with NICE and NHS England throughout the consultation period to devise a response that offered budgetary solutions whilst ensuring patients likely to show benefit from highly specialised technologies were given the opportunity of treatment.

If we don’t stop these changes coming into force from April 1 this year, we will see the introduction of a £100,000 Quality-adjusted life year (QALY) threshold for medicines evaluated via NICE’s Highly Specialised Technologies (HST) programme, which assesses treatments for ultra-rare diseases. This threshold will effectively stop the flow of new medicines reaching patients with ultra-rare and complex diseases. Many treatments for ultra-rare conditions that are currently funded by NHS England have costs per QALY of more than £500,000 including the three medicines that have been approved by NICE’s HST process to date. It is widely acknowledged that QALY thresholds are not appropriate for evaluating medicines for ultra-rare diseases, due to the small patient populations and often limited data.

Ends

Notes to editors

Read the NICE press release

Download the full announcement from NICE and NHS England in the NICE board papers released on 15 March 2017

Download the MPS Society response to the consultation

Read this press release on our website

Contact

Christine Lavery
Group Chief Executive
c.lavery@mpssociety.org.uk
0345 389 9901

About the MPS Society

The Society for Mucopolysaccharide Diseases (The MPS Society) is the only registered UK charity providing professional support to over 1200 families in the UK affected by MPS and related disease. The MPS Society provides a pioneering advocacy service to individual families as well as fund innovative and life changing clinical and academic research. As a registered charity the Society is entirely supported by voluntary donations and fundraising.