XX MP
House of Commons
London, SW1A 0AA

XX March 2020

Dear xxxx

Patients with rare diseases are missing out on new treatments

I am writing to you as one of your constituents about an issue that is close to my heart, which I believe needs urgent attention. I am deeply concerned that people with rare and complex conditions will continue to miss out or face lengthy delays in accessing new treatments unless NHS regulators take urgent action.

More than three million people in the UK will be affected by a rare disease at some point in their lifetime. Many of these conditions are life-threatening and have few, if any, treatment options. Patients therefore rely on the health service to make new treatments available to them as soon as they come on the market, to improve or extend their lives.

[Personal story]

The NHS medicines regulator, the National Institute of Health and Care Excellence (NICE), will take on more responsibility in assessing drugs for rare diseases this year, and is currently reviewing its processes for doing so. NICE must become more flexible to ensure there is a level playing field for people with rare diseases.

Due to the small numbers of patients, there is often greater uncertainty in whether a new treatment for a rare condition will be effective. Drugs are all-too-often rejected because not enough is known about long-term benefit – even if no other drugs exist to treat the disease. Where uncertainty is the primary issue blocking access, NICE should judge in favour of conditional access for a set period until more data is gathered. Scotland has already introduced this approach and have devised a way to pay for it through the New Medicines Fund. I believe the rest of the UK should do the same.

I also think it's vitally important that the patient voice is prioritised in decision-taking. If there is uncertainty around a new treatment, understanding the lived experience of people with the condition and who have received the treatment is instrumental to making informed decisions about NHS access. The devolved nations have formal processes that involves patients, and I can see no reason why a similar set up shouldn't be offered in England.

People with rare diseases deserve the same access to drugs as people with common conditions. I'm afraid that failing to make these changes will mean people affected by rare diseases will miss out on the chance at a better life.

Yours sincerely