Dear

My name is and I am a voter who lives in your constituency. I care for someone who lives with the rare disease myasthenia gravis. I am writing to you today to express my concerns for the future of myasthenia patients and their healthcare. Myasthenia gravis (MG) is an autoimmune disease that affects around 20,000 people in the UK. It is a condition that causes poor nerve-to-muscle communication and destruction of the neuromuscular environment, leading to potentially lethal muscle fatigue. It is also a disease with a sincere lack of options in terms of treatment.

Patients in the UK are still being treated with the older broad-acting treatments which often involve the long-term use of steroids, with lifechanging side effects, or other expensive treatments such as IVIg or plasma exchange. The quality of life for many myasthenic patients is poor, their ability to earn a living can be compromised and they live in fear of a myasthenic crisis which will involve a hospital stay and can be life threatening.

Recently, two potential medicines with proven efficacy and safety in treating MG have received negative final draft guidance (FDG) from the National Institute for Health and Care Excellence (NICE). A third medicine has received initial negative draft guidance after one committee meeting. The appraisal of two more medicines was terminated before reaching committee. It is becoming increasingly clear that the existing framework through which NICE assesses new medicines is not suitable for the rare diseases, where the patient population is smaller, and often more diverse. Action needs to be taken to overhaul the methodology through which these novel medicines are assessed for cost effectiveness.

UK myasthenic patients are being left behind with access to treatment for this chronic, disabling, and, in some cases, life limiting disease. Patients in many other countries of the world have access to one or more of these newer therapies. These countries include many in Europe, the Americas, China, Egypt, Israel, Japan, Oman, Saudi Arabia, South Korea, Taiwan, Turkey, and United Arab Emirates. Soon, the UK will stand out as a country in which patients with myasthenia have no access to treatments that can revolutionise their lives.

This disparity will make the UK become less viable as a marketing opportunity for novel and effective drugs and their manufacturers. The life sciences sector in the UK benefits widely from the investment of the pharmaceutical industry, and the same missed opportunities felt by MG patients will echo across different disease areas with time.

I am asking you to take the following steps to address my concerns:

- Please contact NICE to see if there is anything from a government perspective that can be done to progress the ongoing appraisals towards a positive outcome and the concerns that exist regarding the assessment of medicines for rare diseases.

- Emphasise the importance of the patient voice in this process, as I fear that, despite significant testimony from patients, that this debate is continuously being reduced to a financial matter.

I would like to hear back from you within a month. When you reply to me, please would you tell me what action you will take to address my concerns.

Thank you for your help and support, and I look forward to hearing from you.

Yours Sincerely,