When I heard the Government was conducting a review of the LSDP I felt compelled to offer my story as someone who benefits greatly from the program, not financially or politically but directly as a patient. I have Hunter Syndrome (Mucopolysaccharidosis type II) and have been receiving Elaprase, thanks to the LSDP, since 2009.

When you are diagnosed with Hunter Syndrome there is no good news. You are told you have an ultra-rare condition and that you are likely to die at a much younger age than normal (most with MPS disorders don't live long into adulthood). You are told that your health and body will deteriorate. You are told to expect pain. You are told your joints and muscles will likely stiffen and tighten over time. You are told that Hunter Syndrome can affect any and all parts of your body. You are told there is no cure and up until 2009 you were told there was no treatment.

Now, thanks to Elaprase and the LSDP there is at least some positive news when diagnosed with Hunter Syndrome. Although it isn't a cure and does not treat all aspects of the condition, Elaprase does make a huge difference in the lives of those suffering with this terrible, debilitating disease.

I am extremely grateful to the LSDP for allowing me access to Elaprase, however there are some aspects of being on such a program that I feel could be improved. While everyone is aware that a drug is made available through the LSDP because the cost would otherwise make it prohibitive to patients, there is a perception, rightly or wrongly, of the Government funding the access to these life-saving drugs begrudgingly. A perception that they are looking for reasons to stop access to such drugs and to limit the number of people accessing these expensive treatments wherever possible. It seems rather cruel to me to finally have a drug developed to treat a condition, only to have access to that treatment constantly under threat. I have always, and will happily continue to fulfil all my obligations in regard to monitoring and compliance as asked by the LSDP but I have to admit that it is not a very nice feeling to constantly have a cloud of uncertainty overhead with regard to availability of access to Elaprase. At each of my assessments there is a real sense of pressure of having to perform well to allow continued access to treatment. Life can be tough enough living with such a medical condition without the added worry of whether you will be able to continue with your treatment, the ONLY treatment available.

Nobody asks to be born with a condition such as Hunter Syndrome. It is not the fault of those with an ultra-rare disorder that treatments or indeed cures aren't commercially viable because of the lack of numbers suffering from the disease. We are extremely grateful that research is being carried out and that treatments are being sought for all of the MPS disorders and other rare diseases but treatments are only good if they are available to those who suffer from those conditions.

IF access to Elaprase were stopped for whatever reason it is not as though I could change to another more expensive brand or a less palatable medicine. Elaprase is the ONLY treatment

available for Hunter Syndrome and as such it is currently the only hope for people like myself.

While some elements can be monitored clinically ie liver size etc, there are other areas that can't be physically measured or truly appreciated by anyone not suffering from Hunter Syndrome and I would like to see this VERY important element given more credence. Nobody would choose to undergo weekly infusions EVERY week if there was an alternative. There is no High or Buzz to be gained from receiving Elaprase. So when a patient comments that Elaprase makes a positive difference to their lives, then that statement should not be dismissed lightly. Since being on ERT my quality of life has improved. My wellbeing has improved not only in ways that can be measured clinically but also in more subtle ways. I find it easier to do basic tasks such as tie my shoelaces. I find it easier to comb my hair. For the first time in as long as I can remember it feels that my body actually relaxes instead of the muscles always feeling tense even when laying down. These things can't be measured clinically and it feels at times that they are dismissed out of hand as if it doesn't matter, but believe me those little things matter a whole lot to the patient suffering from such a debilitating disease.

I would ask that you give consideration to the people relying on the LSDP for their ONLY source of treatment, to not only continue providing access to these life-saving drugs but also to somehow seek to remove some of the stress and worry associated with uncertainty over continued access now and in the future. I also ask that you maintain that one piece of positive news that Doctors can give people or parents of children being diagnosed with Hunter Syndrome that although there is no cure, there IS a treatment available through the LSDP.