

Dear Mr Stevens, Mr Hancock, Mrs May and Sir Dillon

Cc: Mr Corbyn, Mr Ashworth

Congratulations on the new vision you have proposed on how you plan to develop our NHS into a World Class Healthcare system fit for 21st century! There are several exciting points, however it saddens our community to see that once again millions of people with rare diseases have been overlooked.

During the last two years we have patiently waited whilst the system, which you are all a part of, played with our lives condemning us and our children to suffer, deteriorate and die. We were told to trust the system. The system that we, as taxpayers, contribute to year on year. Your current vision for NHS still does not address the issues we have raised.

Spinraza is the only approved treatment that can change the course of the progressive neuromuscular disorder called Spinal Muscular Atrophy. The clinical community worldwide, including internationally recognised UK scientists/consultants, fully agree that right now, Spinraza is the only effective treatment for people with SMA – the only way to stop the condition from progressing and even to reverse the damage done. People of all ages and backgrounds who have received this treatment in other countries, have shown remarkable progress. Children in the UK who managed to get access *via* a compassionate program are learning to sit, walk, eat and going to school instead of being dead by their 2nd birthday as expected in the natural course of the condition.

The only reason this treatment has not been recommended officially by NICE is because under the current appraisal system, Spinraza is not viewed as cost effective! The UK is the only country in the developed world that views this treatment as too expensive. Poorer nations like Portugal, Italy and Poland have agreed to reimburse the treatment and even Scotland has a process in place to take treatment forward. This clearly underlines that the NHS is far from the “World Class Healthcare” we aspire to be! Also before using the list price as an example of costs, let us be clear that the list price is not the purchase price. The real price tag will be significantly less for healthcare providers such as NHSE.

We are familiar with the process appraisal and understand that NICE is the national institution that has been designed to provide citizens of this country access to the best treatment available, in the most cost effective way possible (and we must remember that Spinraza is THE ONLY treatment). Yet – NICE has not modernised to reflect the growing needs of our modern society with growing number of treatment for rare diseases. It has also completely neglected the human rights implications of refusing access to life-saving and life-changing treatment.

The appraisal system is simply flawed! For example, an SMA Type 1 child is expected to die before their second birthday. Without treatment the cost to NHS could be as high as £500k, but let’s say it is £100k. Therefore two years would cost £200k, and since after two years the child would die there will be no additional costs. If the treatment is administered and the child lives for 10 years without any improvement the cost to NHS is £1million. Let’s say that the child does improve and will require lower medical support by 75%. The cost is still £250k before we consider the cost of the drug. It does not matter how this is modelled – in this system it is cheaper for the child to die.

QALYs simply fail to take many factors into account and are therefore an inadequate measure of how accurate the cost effectiveness really is. Furthermore, if a couple of years ago we arbitrary reduced QALY to £30k without any statistical research, how can we simply justify that QALY is the best measure? Why is

it that a persons QALY 5 years ago was higher than it is now? Are NICE and NHS policy makers saying that British lives are now half the value they once were due to Brexit factors?

The validity and ability of the system to correctly appraise this treatment has been questioned from the start by clinicians, top figures including ex-CEO of Great Ormond Street Hospital, and even internal NICE personnel. Scotland revised its system early this year to include routes required for appraisal of medicines such as Spinraza with positive outcomes for NHS Scotland, patients and pharmaceutical company. The rest of the developed world has devised mechanisms to deal with rare diseases and situations like these. Yet at NHS/NICE you are not even willing to review the process. The appraisal system of treatments like Spinraza simply exposes the fact that our lives and those of children with SMA (or other rare diseases) are simply not valued the same as other citizens.

Staying on the current track will produce the same devastating. Families lose loved ones. People fight for their life. People become more dependent on others for their care including simple tasks like feeding or wiping their bottoms! This is completely irreparable damage that can and must be avoided. The reputation of an organisation such as NHSE and NICE will be tarnished, not due to lack of money, but lack of moral principles and inaction. (For evil to prevail, it is enough to do nothing)

Senior figures continue to claim that everything works exceptionally well, that pharmaceutical company must reduce the price – well we are burying our heads in proverbial sand and expecting that everything will blow over. This will not blow over; there will be more and more pressure to deal with rare disease as new treatments evolve. The country will suffer economically! Already, as a result of Brexit there is uncertainty around the pharmaceutical industry and science. We have witnessed clinical trials and research being moved out of the UK. Without changing how NICE and NHSE operates the UK will alienate pharma and investors, soon becoming very unattractive to work with. This does not affect only specialist treatment: the majority of specialist treatment manufacturers also make generic drugs, thus creating competitive markets. By shutting them out we lose not just specialist treatments, but also force the price up for basic drugs. It is in the interest of our UK citizens to avoid this. It is time to stop and reflect on what has happened. It is time to realise that people who can be treated should not die. It is time to change how we operate and include 4 million people with rare diseases in your vision of World Class Healthcare! Approving Spinraza would be the most important stepping stone in the process.

Finally, every time we have written to your offices, the replies we received were uninformative and patronising. As things stand, we are well aware of how the system works. I am personally on the appraisal committee for this drug as patient expert. A generic reply pointing fingers at NICE, Biogen, NHSE and hiding behind confidentiality agreements and the processes is not a reply – it is a waste of human time and money. Saying that the system is working “exceptionally well” is simply mocks your understanding of your own system.

Yours truly,
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Helena Plama
Zoe-Anne McKechnie
Rosie Davies
Joel Newell
Marianne E Walker
Julie 'Brown' Durkin (in memory of Conor Durkin-O'Brien)who passed away 9th November 2018
after fighting SMA Type 1 for 15 years & 5 months)
Annalouise Hughes
Susan Hoare
Penny Dyer
Jason Griffiths
Sophie Bennett
Claire Morris
Leah Rose White
Nila Watson
Daiva GirdvainyteMontviliene
Kate 'Richards' Ogborne
Stacie Swell
James Needham
Dianne Feakins
Tatjana Ivanova
Dawn Parker
Brian Parker (took his life due to SMA aged 27)
Alicia Loh
Christopher Cusack
Fleur Perry

Jeanette & Jeff Nazer
Shauna Yabsley
Ricky Yabsley
Lisa Gradziewleska who sadly passed away on 3rd June
Sue Tucker
Zanna Alexandra
Ruta Pabbi
Jody Wilkin
Wayne Burfitt
Freddie Sheffield
Eugenia Kulama
Ross Hovey
Mark Keen
Rakesh Pabbi
David Kenney
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Gabrielle Van Der Velde
David Ogborne
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Tammy Wright
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