TreatSMA
Treat Spinal Muscular Atrophy

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PRESS RELEASE

DO NOT LET ANY MORE PEOPLE DIE FROM TREATABLE CONDITION.

More than a thousand people in England and Wales who live with spinal muscular atrophy (SMA) are fighting for their lives whilst a very effective treatment is been viewed as too expensive to be funded through NHS. SMA, commonly known as the no. 1 genetic killer of babies and infants, is a progressive disease of motor neurones that causes early death of nearly 50 UK children a year and leaves hundreds more progressively losing muscle function ending in permanent paralysis.

The previous heart-breaking advice from the National Institute of Health and Care Excellence (NICE) not to fund the only effective drug, nusinersen, through the NHS has caused despair and outrage within the SMA community, forcing hundreds of families to join forces via social media to have their voices heard.

Nusinersen, which was approved as a breakthrough drug in the US in December 2016 and in the European Union in May 2017, is the only existing treatment that brings about a meaningful change in the lives of those affected by SMA. Developed by Ionis Pharmaceuticals and marketed by Biogen as Spinraza®, it has saved lives and muscle function of more than 6,000 children and adults living with SMA globally.

Nusinersen therapy has been approved and is available to all those who suffer from SMA across most of the developed world as well as in a number of developing countries. Currently, twenty-four countries of the European Union provide Spinraza under government funding, and many more outside, including Turkey, Australia, Canada, Japan, Norway, Switzerland, and the US. Moreover, the treatment will be available to most SMA patients in SCOTLAND! Regretfully, England and Wales are poised to become the only country in Europe where small babies diagnosed with SMA will be left to die in their first years of life while hundreds more will keep losing their muscle function until paralysis and untimely death.

Representing the voice of those affected by SMA and their parents and carers, TreatSMA has continuously advocated that the treatment must be made available for

all those with SMA regardless of age, genetic makeup, disease "type" or other arbitrary criteria. We have challenged the notion that Spinraza should be appraised for limited population or specific subgroups. We have challenged the whole appraisal system as it clearly not effective when it comes to appraisal of rare diseases. (See our recent post highlighting that there is a clear discrimination against rare disease community). We stressed that physical and emotional support for caregivers must be considered during the appraisal. We have stressed that appraisal models do not work well because SMA is a spectrum disease and affects people differently.

However, the currently adopted position by NICE is that the provision of the only effective drug for SMA is not "a cost-effective use of NHS resources", thereby we finally became the only country in the world to place generic price on human life!

We, the community are not prepared to see our lives and the lives of our children taken away from us. Several academic publications confirm that nusinersen has been proven an effective treatment even in the weakest of patients and across the entire spectrum of the disorder. We have closely followed the progress of clinical trials and the subsequent clinical practice and seen babies living and thriving worldwide whilst here in the UK children have been dying. We witnessed first hand how some of children who can access treatment through compassionate program achieved milestones that could never be achieved without the treatment.

TreatSMA, along with the hundreds of UK families, will be protesting against the health policies on the 6th March 2019. The protest will take place outside of NICE office, City Tower, Piccadilly, Manchester M1 4BT. Both protests will start at 12pm and last for several hours.

This is the third appraisal meeting of a long drawn out decision making process. Nusinersen has already received a negative recommendation.

We are asking that the press support us in our campaign, as they did in the past.

Media contact for the 6 Marchprotest: Kelly Jones, kelly.jones@treatsma.uk.

TreatSMA is a UK community of people with spinal muscular atrophy (SMA) as well as parents of children with SMA who have joined hands to fight for wide and equitable access to treatment. We work entirely on a voluntary and non-profit basis, being driven by our commitment to saving the lives of ourselves and of our dear ones.

For more information visit www.treatsma.uk.

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Statements from TreatSMA families

For use in media articles

1. **Kacper Rucinski, co-founder of TreatSMA, father of Lia, 9, who lives with spinal muscular atrophy type 2**

Our entire family is in shock. Ever since Lia was diagnosed with SMA at 19 months, we have been waiting for a treatment that would stop the deadly deterioration of her muscle abilities. In 2016, we shared the joy of the entire world when the first treatment for SMA was found safe and effective in all patients and soon received marketing authorisation in USA and later in Europe.

Lia is unable to walk. Her hands are weak. Her spine is developing scoliosis. She is already dependent on a wheelchair and requires help in all her daily tasks. Unless she receives an effective treatment in the next few months, her scoliosis will force her to live in a spinal jacket or have spinal rods implanted.

This could all be avoided. Lia, just as other children like her as well as adults, can now be saved from deterioration. Doctors and researchers have confirmed on countless occasions that the nusinersen treatment reverses the course of the spinal muscular atrophy, bringing about steady, constant improvement.

It beggars belief that NICE appears to have ignored most of the evidence presented by academics as well as patient groups like TreatSMA. Its recommendation, if finalised in its current form, will only result in tens of unnecessary deaths and hundreds people who live with SMA becoming permanently disabled each year.

Equally, I am bitterly disappointed that the manufacturer, Biogen, has been unwilling to offer a price that would allow the health authorities look at the drug more favourably.

I trust and hope, on behalf of my daughter and of all others with the same condition, that the NHS, NICE and the manufacturer will find a way to allow this treatment to happen in this country, the way it is already available in other countries.

Lia and her parents

2. **Gary Mckie, volunteer at TreatSMA, father of Sam who is 6 years old with spinal muscular atrophy type 2**

My name is Gary Mckie, I am father to Sam who is 6 years old and has type 2 SMA. I have been asked to supply a reaction to the news that NICE will NOT be recommending Nusinersen for treatment in Spinal muscular atrophy for any type.

I am horrified, devastated, sad and angry all at the same time.

I'm horrified at the decision, considering that nusinersen has been approved in many many countries in the civilised world. I cannot believe that a country as developed as the UK cannot find it in itself to recommend treatment for our children and adults who are suffering with this condition. There are many countries who do not have the financial backing that we do, yet they seem to be able to find the money to treat their children. Countries like Iran, Macedonia and Italy.

I'm devastated because I now have to explain to my child why he can't get treatment. He knows there is a treatment that could change his life and now i have to break his heart and tell him that the UK will not fund it to help him and others.

I'm sad because every day I have to look at photos and videos of people all over the world who are receiving treatment and posting on media about how beneficial Nusinersen is to them. I have to watch as others progress and know that without treatment my son will decline more and more as each day, month and year passes.

I'm angry as I attended the committee meeting in June. I watched as a complete bunch of strangers with no connection to SMA slowly picked apart the information provided to them by clinicians and patient and parent voices, and still decided not to recommend this treatment.

But one thing is for sure. With all of these emotions comes one more.

Determination

I am determined to push forward and do what we can to get this decision overturned. This is not the end, we have to pull together as a community and fight this decision. We will be heard. Our children and adults living with SMA will get the treatment they deserve. This is the ONLY treatment approved for SMA. How can you deny it?

Gary with his family

3. **Kelly Jones, volunteer at TreatSMA, mother of Vinnie, 3, who has spinal muscular atrophy type 1**

I am deeply disappointed with the recent NICE decision. Not only are they stopping people with SMA from benefitting from a life changing drug they are sentencing babies to death.

This drug has shown to have lifesaving effects within the Type 1 community and for adults living with Type 2 and 3, they are now gaining some lost abilities therefore regaining their independence.

This will be a massive blow to all the community here and families are feeling like the UK is once again being left behind where rare disease drugs are concerned.

As a mum of a type 1 child who has participated in the trial since October 2015 I have seen first-hand the results that Spinraza has had on someone with SMA and it has been nothing short of a miracle and I feel bless to watch my child grow and progress. I am saddened to know that the families here will not get the chance to witness that themselves.

Vinnie, Kelly's son