TreatSMA
Treat Spinal Muscular Atrophy

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PRESS RELEASE

"Wear your hurt on your shirt" campaign

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.

The decision by NICE on whether to recommend Spinraza™ (nusinersen) for use in the NHS for those with spinal muscular atrophy (SMA) was yet again delayed this week, with what will be the fourth committee meeting scheduled to take place, in private, next Wednesday 8th May.

We, TreatSMA, think this is outrageous as we have waited well over two years now for the decision makers to make their recommendation, following the European Medicines Agency approval of nusinersen in May 2017.

In those two years, 45 countries worldwide have approved nusinersen to treat SMA, with over five thousand children and adults receiving treatment in their respective

countries. Meanwhile here in the UK we wait and watch as our children and adults with SMA lose abilities and get weaker as each day passes and children have needlessly gained their angel wings while we have waited on the long drawn out and outdated appraisal process of NICE.

We have called upon the SMA community, the public and press to act once more. We are asking the SMA community, friends and family to wear in public on the 7th May a plain t-shirt or top with a personal message to NHS England and NICE telling them we will not wait any longer.

Schools and work places will be getting involved across the country including classmates and work colleagues by doing tops with supportive slogans. Local football teams, relatives , local shop owners, neighbours, local sports teams..... everyone is involved.

Each picture will be forwarded to

NICE - nice@nice.org.uk
Simon Stevens CEO NHS England - england.ce@nhs.net

We also want the support of the press to run our story on the TV or in newspapers and we ask that you run the story on Wednesday the 8th May 2019 to coincide with the meeting at NICE.

We WILL NOT be ignored, we WILL get our voices heard and we WILL NOT wait any longer.

Please help us give a final push.

The Treatsma team
WWW.TREATSMA.UK

https://www.facebook.com/events/2237535639800037/?ti=cl

Registered address: 1 Alder Lodge, Warberry Park Gardens, Royal Tunbridge Wells TN4 8GL.

Statements from TreatSMA families

For use in media articles

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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 blessed 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