Spinal Muscular Atrophy Support UK
Help for today • Hope for tomorrow

Muscular Dystrophy UK
Fighting muscle-wasting conditions

The SMA Trust
Dedicated to finding a cure and treatments for Spinal Muscular Atrophy

Rt Hon. Jeremy Hunt MP
Secretary of State for Health
Richmond House
79 Whitehall
London
SW1A 2NS

Dear Mr Hunt,

We are writing to you jointly as representatives of the three leading patient advocacy groups working for the spinal muscular atrophy (SMA) community in the UK. We are asking for your assistance in ensuring nusinersen (Spinraza) is made available at the earliest possible time for the treatment of spinal muscular atrophy for patients in this country.

SMA is a rare inherited neuromuscular condition affecting the lower motor neurons. The condition may affect crawling and walking ability, arm, hand, head and neck movement, breathing and swallowing.

SMA Type 1 is the most severe form of SMA. It accounts for between 50 – 70% of cases of childhood onset SMA. SMA Type 1 is a life-limiting condition. Though it is not possible to accurately predict, for the majority of children (approximately 95%) life expectancy is less than two years. In general, babies diagnosed within the first few weeks or months of life have a significantly shorter life expectancy.

Nusinersen is an investigational, potentially disease-modifying therapy for the treatment of SMA. It is manufactured by the company Biogen. The drug has been trialled in infants with SMA Types 1,2 and 3. The trial results have shown a statistically significant improvement in the achievement of motor milestones compared to those patients on placebo and for that reason Biogen took the decision to file early for US and European licences based on interim data.

Due to these promising results – and the exceptional severity and life-shortening nature of SMA Type 1 – Biogen, took the decision last August to make the drug available free to SMA Type 1 patients via an Expanded Access Programme (EAP). This is taking place whilst the company seeks a marketing authorisation from the European Medicines Agency, potentially for all types of SMA. Indeed, the Food and Drug Administration in the United States gave broad approval for all SMA types last December.

However, administering the drug is intensive and lack of capacity at specialist clinics is currently preventing the EAP being rolled out for affected infants. The drug is administered via a lumbar puncture injection into the spinal cord initially every fortnight. This requires high levels of training to be performed safely, the involvement of a number of specialist healthcare professionals, anaesthetists and theatre space.

Clinicians and our three patient groups are taking all the steps we can within the resources available, but so far only one patient has been enrolled on the EAP, in Northern Ireland. This is because whilst the drug is being made available for free, there is no funding to support the additional infrastructure and capacity required by centres to deliver the treatment.

The centres currently approved to carry out the procedure are Great Ormond Street Hospital, Newcastle and Belfast, although a few other paediatric neuromuscular centres are also looking into the feasibility of providing the treatment.

As you can imagine, this situation is exceptionally distressing for families whose children would otherwise be eligible for the EAP. They know that a drug is available which could significantly help their child, but that they are currently unable to access it.

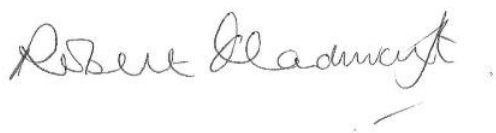
This is the first treatment for SMA and it is important that the process here in the UK doesn't fail babies and their parents. As stated earlier babies with Type I SMA typically die before they reach their 2nd birthday and parents are understandably desperate for access to this treatment as soon as possible. The EAP is designed to enable this to happen whilst EMA, Health Technology Assessment bodies and NHS England do their work.

Sadly the EAP in the UK is failing because whilst the drug is provided free of charge there is no funding to support the centres to administer the treatment. This means that babies will continue to die before being given the opportunity to receive this treatment because of a lack of funding. EAP centres in other countries are successfully managing and the UK is being left behind.

We are therefore writing to ask what support could be provided to centres from the Department of Health, and if you and your officials will have urgent discussions with NHS England, patient groups and Biogen on a way forward to ensure patient access to the treatment while we wait for licensing/funding decisions?

We very much hope that you will be able to help progress this urgent issue.

Yours sincerely,



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