



Vaughan Gething AM  
Minister for Health and Social Services  
Welsh Government  
5th Floor  
Tŷ Hywel  
Cardiff Bay  
CF99 1NA

28<sup>th</sup> May 2019

Dear Mr Gething,

**Re: Access to SMA treatment Spinraza in Wales**

We are writing following the NICE recommendation of 15<sup>th</sup> May for Spinraza – the first treatment for the rare condition spinal muscular atrophy (SMA) – to be available for use on the NHS in England for children and adults with SMA Types 1, 2 and 3 following an agreement between the pharmaceutical company, Biogen, and NHS England.

The Managed Access Agreement (MAA) which has been reached means that patients will be able to be treated with Spinraza while more long-term data on its effectiveness is gathered.

Families have endured a long and frustrating wait of 16 months to hear the outcome of NICE's appraisal process and we are delighted that patients with SMA Types 1, 2 and 3 are facing a brighter future following this positive news.

In Scotland, Spinraza has been available to SMA Type 1 patients since May 2018, and this is set to be expanded to Types 2 and 3 soon under the Scottish Medicines Consortium's new ultra-orphan pathway. Meanwhile Spinraza may be available for patients with Types 2 and 3 through the Peer Approved Clinical System (PACS) Tier One system which allows for individual requests submitted by clinicians.

NICE guidance is now scheduled to be issued on 26<sup>th</sup> June. Therefore, we seek assurances that the All Wales Medicines Strategy Group will be ready for this publication date to support and implement the adoption of this guidance within NHS Wales.

We also urge subsequent ratification by the Welsh Government to be completed without delay so that eligible patients in Wales can access this life-changing treatment as soon as possible.

We look forward to your assurances on the above points to ensure fast implementation of the NICE guidance in Wales.

Yours sincerely,

Handwritten signature of Kate Adcock in black ink.

**Dr Kate Adcock**  
**Director of Research and Innovation**  
**Muscular Dystrophy UK**

Handwritten signature of Doug Henderson in black ink.

**Doug Henderson**  
**Managing Director**  
**Spinal Muscular Atrophy UK**