Richard Pengelly
Permanent Secretary
Department of Health
C5.11
Castle Buildings
Stormont
Belfast
BT4 3SQ

28th May 2019

Dear Mr Pengelly,

Re: Access to SMA treatment Spinraza in Northern Ireland

We are writing following the NICE recommendation of 15th 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 is 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 26th June. Therefore, following concerns raised at the All Party Group on Muscular Dystrophy meeting in the Northern Ireland Assembly on 20th May, we seek assurances that the Department of Health will be ready to support and implement the adoption of this guidance within Health and Social Care in Northern Ireland.

The Duchenne muscular dystrophy treatment, Translarna, was given approval by the Department of Health within a day of NICE guidance being issued, and we urge Spinraza to be approved in the same timeframe so that eligible patients in Northern Ireland can access this life-changing treatment as soon as possible. The lack of a Northern Ireland Executive is causing additional alarm for families desperate for fast decision-making and implementation,

and we are writing to the Secretary of State for Northern Ireland, Rt Hon Karen Bradley MP, to seek her assurances this will not be a barrier to accessing Spinraza.

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

Yours sincerely,

Dr Kate Adcock
Director of Research and Innovation
Muscular Dystrophy UK

Doug Henderson
Managing Director
Spinal Muscular Atrophy UK