Rt Hon Karen Bradley MP
Secretary of State for Northern Ireland
Northern Ireland Office
1 Horse Guards Road
London
SW1A 2HQ

28th May 2019

Dear Ms Bradley,

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 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 26th June. The Duchenne muscular dystrophy treatment, Translarna, was given approval by the Department of Health in Northern Ireland within a day of NICE guidance being issued.

Following concerns about access to Spinraza aired at a meeting of the All Party Group on Muscular Dystrophy in the Northern Ireland Assembly on 20th May, 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.

We are writing to you because 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 Department of Health Permanent Secretary, Richard Pengelly, to seek his 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