



19 November 2021

Dear Liz and Libby

In response to your request, we are delighted to share that NICE has today published a Final Appraisal Document (FAD) recommending risdiplam as a treatment option for 5q spinal muscular atrophy (SMA) in people 2 months and older with a clinical diagnosis of SMA types 1, 2 or 3 or with pre-symptomatic SMA and 1 to 4 SMN2 copies. This recommendation is subject to the terms of a managed access agreement (MAA). We are pleased to have worked closely and collaboratively with both NICE, NHS England and members of the SMA community to achieve this important outcome.

Today's news would not have been possible without the support, collaboration and passionate advocacy of patient organisations and individuals across the UK SMA community. We would like to thank you for all you have done to achieve this milestone.

We recognise that improving access to treatment is just one element of supporting individuals affected by SMA. We are committed to continuing to work with you into the future to improve the lives of everyone affected by SMA across the UK.

Please do not hesitate to contact us if you have any questions. We anticipate that following the publication of NICE guidance risdiplam will be available in England from early 2022. We have included information on the transition from EAMS and the UK access to risdiplam outside England below.

Yours sincerely,

Jack on behalf of the UK SMA team

Jack Robinson
Policy, Value and Access Partner
Roche Products Ltd

What will happen now to patients who are enrolled in the risdiplam Early Access to Medicines Scheme (EAMS)?

Patients in England who have been enrolled in the risdiplam Early Access to Medicines Scheme (EAMS) and are eligible for treatment under the MAA should move across to NHS funding for



this medicine 30-days after the publication of NICE guidance. Roche will work with prescribing centres to ensure that this process runs as smoothly as possible.

What is the status of access to risdiplam in Wales, Northern Ireland and Scotland?

Roche UK is in discussions with the relevant authorities in Wales and Northern Ireland respectively to ensure that this NICE guidance on risdiplam can be adopted as soon as possible in these countries.

In Scotland, Risdiplam is currently under evaluation by the Scottish Medicines Consortium and we anticipate guidance in early 2022.