9th July 2019

Subject: NICE communications about access to nusinersen (EH105163)

Dear Liz, Gennadiy and Clare

Thank you for sharing your concerns about the communication surrounding the appraisal of nusinersen (Spinraza) to treat 5q spinal muscular atrophy (SMA).

As you know through your involvement in this appraisal, we have worked hard to support access for patients to nusinersen. We are always very conscious of the responsibility we bear when conducting appraisals and the impact our decisions have on patients, while recognising that we must consider the impact on the health system as a whole. We are also aware how important it is for people to be kept up to date with the progress of appraisals and we try do so in a comprehensive and timely way.

In this case, we accept that we didn't communicate clearly enough and we are very sorry for the confusion and distress this caused.

I have answered each of the points you made in your letter below.

The First Communication
We're sorry that patients and their families understood that access to nusinersen through our draft recommendations would be without restriction. We acknowledge that the news story that appeared on the NICE website was not clear enough.
The intention of the press release from NHS England and then the news story on the NICE website was to welcome that agreement had been reached in principle between NHS England and the company. We accept that it should have been more explicit about the criteria in the managed access agreement (MAA) but that detail was not available at the time. However, we could have been clearer that there would be criteria and that not all patients would be able to access treatment.

Given the length of time patients and their families had been waiting for news about access to nusinersen we were keen not to hold up the announcement or publication of the final appraisal documentation (FAD) and MAA. This meant that patient groups were given less notice than would usually, but not always, be the case.

The Second Communication and patient group meeting with NICE/NHSE
We acknowledge that we should have been clearer with patient groups that there was a lack of progress and communicated that we were proceeding to release the FAD.

Publication of the guidance and draft MAA
We tried to be clear in our meeting with patient groups that the MAA had been reached only after protracted and detailed conversations and was unlikely to change further. We agreed to provide greater clarity around some of the definitions and timelines outlined in the MAA but were conscious that major changes could put the whole MAA at risk and further delay access to treatment. We have taken on board these points and hope our more recent discussions with patient groups on 28 June reflect the current situation more clearly.

Our subsequent work with clinicians and the SMA Community
Thank you for sharing feedback from members of the patient community. We have received similar comments and I am sorry to read how this has affected some people in the SMA community.

We are grateful for your feedback, and your participation during this difficult appraisal. I hope that, through your involvement, you recognise that NICE does take the views of patient groups seriously and values their input.

In conclusion, we recognise that we could have been clearer and more sensitive in our communications about the outcome of this appraisal. Our senior team will reflect on your comments to identify how we can improve our communications for future appraisals to ensure that we provide as much clarity as possible.

Yours sincerely,

Sheela

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