NHS England's Statement about access to 'combination therapy' trials

"The NHS England policy of funding for excess treatment costs only applies in the case of non-commercial research.

While we understand that exceptions to the policy may have been granted in previous exceptional circumstances, these were only in cases where a treatment was already routinely commissioned by NHS England. For further information on the excess treatment costs policy please see:

- NHS England and Department of Health & Social Care: Excess treatment costs (PDF)
- National Institute for Health and care Research (NIHR): Excess Treatment Costs

In view of the policy, NHS England is not able to accommodate funding for nusinersen or risdiplam treatment alongside participation in a commercial clinical trial in these circumstances. Furthermore, the following considerations in relation to these SMA treatments should be understood:

- These treatments are not yet considered established practice in the NHS, as NICE was unable to make a routine recommendation until further evidence is available.
- Both treatments in these managed access agreements (MAA) are commissioned as monotherapy only. The MAA entry criteria for both nusinersen and risdiplam make clear that a treating clinician is agreeing to use the treatments as monotherapies. See nusinersen and risdiplam
- The managed access agreements have been designed to collect data of people on monotherapy treatment within clinical practice. Allowing additional treatment within a clinical trial would undermine the data collection agreement and the ability to collect patient data that is needed to inform a long-term commissioning recommendation beyond the time-limited period of the MAAs.
    - The commercial arrangements between NHS England, Biogen, and Roche that enable patient access to nusinersen and risdiplam only apply where patients access the treatment through the MAAs.

We fully recognise the appetite among patients, families and treating clinicians to explore combination therapies to treat SMA.

However, for the reasons set out above we cannot enable provision of excess treatment for patients currently receiving nusinersen or risdiplam through the MAAs. There are no restrictions placed on patients who wish to exit the MAA to enter a commercial clinical trial if funding for treatment through other avenues is available.

In securing the MAAs for nusinersen and risdiplam NHS England applied commercial flexibility to ensure that eligible patients with SMA could benefit from treatment at the very earliest opportunity; not waiting for further evidence but enabling treatment to commence while data collection took place in parallel.

Our primary focus is to provide the most comprehensive data collection through the MAAs with a view to enabling long-term NHS commissioning of these treatments."

22nd December 2022