NHS England's Landmark Agreement for Access to Zolgensma for Eligible Children who have SMA Type 1

On 8th March, NHS England announced it was:

"...ready to fast-track the introduction of the highly complex and innovative gene therapy and will not wait until NICE publish final guidance to get going. This approach is backed by NICE given the importance of administering the one-off treatment as early as possible.

The terms of the deal mean that some young children that currently fall outside the NICE recommendation criteria will also be eligible to be considered for treatment by a national multidisciplinary clinical team (MDT) made up of the country's leading experts in the treatment of SMA.

This means as many as 80 babies and young children could potentially benefit from the life-changing gene therapy a year."

On 16th March, NHS England clarified which groups the treatment would include:

(a) Children with SMA Type 1 up to 6 months of age

(b) Children with SMA Type 1 over 6 months of age and below 12 months of age

(c) Children with type 1 SMA who are over 12 months old within the scope of the drug's European Medicines Agency (EMA) marketing authorisation

(d) Children with type 1 SMA who have been using treatment e.g., with nusinersen (Spinraza) or Risdiplam (Evrysdi) and fall within the scope of the European Medicines Agency marketing authorisation.

(e) Any pre-symptomatic SMA infants identified who have up to 3 copies of the *SMN2* gene

Children in any of these groups who are having more than 16 hours per day of non-invasive ventilation, or who have a tracheostomy, are not eligible, under draft NICE recommendations. These recommendations could change following NICE consultation.

Children in groups b, c and d will be considered by a national multi-disciplinary team (NMDT) made up of clinical experts from the gene therapy centres and other centres. The NMDT will consider a number of clinical factors in their decision making based on clinical experience in the UK and abroad, data from the trials and the European consensus statement.

Children in groups a and e will be considered for treatment by the gene therapy centre they have been referred to and will only need wider NMDT discussion if their case is complex.