Wednesday 25th January 2017
STATEMENT

Temple Street Children's University Hospital approval for Nusinersen Expanded Access Programme (EAP) to treat children with Spinal Muscular Atrophy (SMA) Type 1.

In late 2016 Biogen and Ionis Pharmaceuticals announced encouraging interim results from their clinical trials study called ENDEAR Phase III which involved children with SMA Type 1. The name of the drug being studied was Nusinersen. SMA1 is a rare genetic degenerative condition that make it difficult to babies to crawl, walk, breath and swallow and for 95% of children with this condition, life expectancy is less than 2 years.

Following these results Biogen announced that it was commencing an Expanded Access Programme (EAP) to provide Nusinersen to children with SMA1 in order to address a high unmet medical need in this patient population.

On 18th January 2017, Temple Street Children's University Hospital was approved for this EAP. Patients eligible for this programme must have a genetically confirmed diagnosis of SMA1 with onset of clinical signs and symptoms at less than six months of age.

At present the EAP is limited to children who have SMA1. However there clearly continues to be an unmet need to treat patients with SMA Types 2 and 3 and studies exploring Nusinersen as a treatment for children with this condition are on-going.

According to Dr Declan O'Rourke, Consultant Paediatric Neurologist, Temple Street Children's University Hospital who treats these children "It is our understanding that Biogen is continuing to explore the possibility and feasibility of opening an EAP for patients with SMA Types 2 and 3. However, there is no clear timeline if and when this will happen and such a decision to open this EAP lies solely with the pharmaceutical company"

In the USA the EAP has closed because the Food and Drug Administration (FDA) has granted a broad licence for use of Nusinersen and therefore all children with SMA who are deemed eligible are now receiving Nusinersen.

At present Nusinersen is an unlicensed product in Europe. Biogen submitted a licencing application in October 2016 to the European Medical Agency (EMA). This product is currently undergoing an accelerated assessment pathway by the EMA with an anticipated decision regarding the licencing to be made in June or July 2017.

"If this product is granted a licence by the EMA it will need to undergo assessment by local regulatory authorities before patients can avail of this and the local regulatory authority in Ireland is the HPRA. In Ireland this process also involves a funding review and recommendation via the National Centre for Pharmacoeconomics (NCPE) and subsequently the HSE Drugs Group and in many instances this process can take up to 12 months. We understand that this is a frustrating and distressing process for families. We will update you as soon as more information is available from Biogen, Ionis and the regulatory authorities" continued Dr O'Rourke

For further information about the Nusinersen EAP, families can email patientcenter@biogen.com