Seirbhís Aisíoca Príomhchúraim
Bealach amach 5 an M50, Án Bóthar Thuaidh, Fionnghlas, Baile Átha Cliath 11, D11 XKF3
Guthán: 01 8647100

Primary Care Reimbursement Service
Exit 5, M50, North Road, Finglas, Dublin 11, D11 XKF3
Tel: 01- 8647100

Colm Burke, T.D.
Dáil Éireann,
Leinster House,
Kildare Street,
Dublin 2.

19th October 2021

PQ: 48435/21

To ask the Minister for Health the position regarding the pricing and reimbursement negotiations for the gene therapy drug zolgensma (details supplied); and if he will make a statement on the matter. -Colm Burke

Details supplied:
In April 2020 the National Centre for Pharmacoeconomics (NCPE) received a reimbursement application dossier for Onasemnogene abeparvovec (Zolgensma). On the 13th May 2020, the NCPE completed a rapid review of the application and recommended a full Health Technology Assessment (HTA) to assess the clinical effectiveness as well as the cost effectiveness of Zolgensma compared with the current standard of care. The HTA was completed in May 2021. The NCPE recommended that Zolgensma not be considered for reimbursement unless cost effectiveness could be improved relative to existing treatments. Following this decision pricing/reimbursement negotiations commenced in July 2021.

Dear Deputy Burke,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 48435/21), which you submitted to the Minister for Health for response.

The European Medicines Agency (EMA) is a centralised agency of the European Union (EU) responsible for the scientific evaluation, supervision and safety monitoring of medicines in the EU.

Onasemnogene abeparvovec (Zolgensma®) has received a Marketing Authorisation (MA) from the EMA for the following indications:

- Treatment of patients with 5q spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type 1, or patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene.

The Summary of Product Characteristics is available at:

https://www.ema.europa.eu/documents/product-information/zolgensma-epar-product-information_en.pdf

Distinct from the EMA authorisation process, there exists a formal statutory process which governs the pricing and reimbursement of medicines in Ireland and the application process for new medicines to be funded and/or reimbursed.

In line with these statutory processes, in October 2021 the HSE approved the reimbursement of Onasemnogene abeparvovec (Zolgensma®) for a restricted subgroup of the licensed population.

Reimbursement is restricted to the following subgroup of the licensed population:

- Patients with SMA with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA Type I, or pre-symptomatic patients with 5q SMA with a bi-allelic mutation in the SMN1 gene and up to 3 copies of the SMN2 gene

The approved subgroup is in line with the application from the applicant (Novartis Gene Therapies), who had applied for reimbursement for the restricted population outlined above.

Prescribers will be required to apply for reimbursement approval on an individual patient basis.

Reimbursement approval for Onasemnogene abeparvovec (Zolgensma®) in Ireland follows joint assessment and negotiations under the Beneluxa Initiative. Following joint negotiations, Belgium, Ireland and the Netherlands reached an agreement with Novartis Gene Therapies on the pricing of Onasemnogene abeparvovec (Zolgensma®).

The joint process for Onasemnogene abeparvovec (Zolgensma®) consisted of a Health Technology Assessment, followed by a price negotiation. HSE decisions on which medicines are reimbursed by the taxpayer are made on objective, scientific and economic grounds. The HSE, as standard, engages in commercial negotiations with drug manufacturers to ensure the HSE delivers the best possible prices for new drugs. This enables the HSE to reimburse as many medicines as possible, to as many patients as possible, within the resources provided to the HSE.

Yours sincerely,

Suzanne Doyle
Primary Care Eligibility & Reimbursement Service