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Naoise Ó Cearúil, T.D.
Dáil Éireann,
Leinster House,
Kildare Street,
Dublin 2.

23rd July 2025

PQ: 38374/25

To ask the Minister for Health if the HSE's managed access protocol and individual patient approval system is available to adults with spinal muscular atrophy who were previously excluded from treatment solely due to their age at the time of reimbursement approval; and, if so, the process or criteria that would apply in such cases; and if she will make a statement on the matter. -Naoise Ó Cearúil

Dear Deputy Ó Cearúil,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 38374/25), which you submitted to the Minister for Health for response.

The HSE is committed to providing access to as many medicines as possible, in as timely a fashion as possible, from the resources available (provided) to it.

The HSE robustly assesses applications for pricing and reimbursement to make sure that it can stretch available resources as far as possible and to deliver the best value in relation to each medicine and ultimately more medicines to Irish citizens and patients.

HSE decisions on which medicines are reimbursed by the taxpayer are made on objective, scientific and economic grounds.

There are formal processes which govern applications for the pricing and reimbursement of medicines, and new uses of existing medicines, to be funded and / or reimbursed.

The HSE considers the following criteria prior to making any decision on pricing / reimbursement, in line with the Health (Pricing and Supply of Medical Goods) Act 2013:

- (1) The health needs of the public,
- (2) The cost effectiveness of meeting health needs by supplying the item concerned rather than providing other health services,

- (3) The availability and suitability of items for supply or reimbursement,
- (4) The proposed costs, benefits, and risks of the item or listed item relative to therapeutically similar items or listed items provided in other health service settings and the level of certainty in relation to the evidence of those costs, benefits and risks,
- (5) The potential or actual budget impact of the item or listed item,
- (6) The clinical need for the item or listed item,
- (7) The appropriate level of clinical supervision required in relation to the item to ensure patient safety,
- (8) The efficacy (performance in trial), effectiveness (performance in real situations) and added therapeutic benefit against existing standards of treatment (how much better it treats a condition than existing therapies) and,
- (9) The resources available to the HSE.

In terms of the specific details of the application for pricing and reimbursement of risdiplam (Evrysdi®):

Reimbursement approval is in place for risdiplam (Evrysdi®) for the treatment of 5q spinal muscular atrophy (SMA) under the High Tech arrangements from the 1st September 2023.

Reimbursement support is for a subgroup of the licensed indication.

As a condition of reimbursement, an individual patient approval system has been put in place by the HSE, to enable reimbursement for patients who meet the pre-defined criteria as per a HSE devised managed access protocol.

[Managed Access Protocol – Risdiplam \(Evrysdi®\) for the treatment of 5q Spinal Muscular Atrophy](#)

If the manufacturer of risdiplam (Roche) submits a de novo application for adult patients with a clinical diagnosis of SMA Type 1, Type 2 or Type 3, that application will be duly progressed through the formal processes governing the pricing and reimbursement of medicines.

In terms of the specific details of the application for pricing and reimbursement of onasemnogene abeparvovec (Zolgensma®):

In October 2021, onasemnogene abeparvovec (Zolgensma®) was approved for reimbursement in Ireland following joint assessment and negotiations under the Beneluxa initiative for the treatment of patients with spinal muscular atrophy (SMA) with a bi-allelic mutation in the SMN1 gene and a clinical diagnosis of SMA type 1, 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.

As a condition of reimbursement, an individual patient approval system has been put in place by the HSE, to enable reimbursement for patients who meet the pre-defined criteria as per a HSE devised managed access protocol.

[Managed Access Protocol – Onasemnogene abeparvovec \(Zolgensma®\) for the treatment of 5q Spinal Muscular Atrophy \(SMA\)](#)

In terms of the specific details of the application for pricing and reimbursement of nusinersen (Spinraza®) for adult patients (>18 years):

The HSE approved funding of nusinersen (Spinraza®) effective 1st July 2019 for the treatment of 5q Spinal Muscular Atrophy (SMA) Type I, Type II and Type III in children under 18 years.

The HSE received a further application for pricing and reimbursement of nusinersen (Spinraza®) on the 17th September 2020 from Biogen (the applicant) indicated for the treatment of 5q Spinal Muscular Atrophy (SMA) in adult patients (>18 years).

- The first step in the process is the submission of a Rapid Review dossier (a clinical and economic dossier) to the National Centre for Pharmacoeconomics (NCPE) for assessment. The HSE commissioned the Rapid Review process on the 28th September 2020.
- The NCPE Rapid Review assessment report was received by the HSE on the 29th October 2020. The NCPE advised the HSE that a full Health Technology Assessment (HTA) was recommended for nusinersen (Spinraza®) to assess the clinical and cost-effectiveness compared to the current standard of care.
- The HSE commissioned a full HTA on the 9th November 2020 as per agreed processes.
- The NCPE publishes details of medicines where the HSE has commissioned a Rapid Review assessment and / or a full HTA on their website. The website is updated at regular intervals and includes assessment outcomes and updates on reimbursement for each individual medicine and indication listed. Details of the assessment(s) of nusinersen (Spinraza®) for adult patients are available at: <https://www.ncpe.ie/nusinersen-spinraza-hta-id-20044/>
- The HSE Corporate Pharmaceutical Unit (CPU) is the interface between the HSE and the Pharmaceutical Industry in relation to medicine pricing and reimbursement applications. The CPU met with Biogen regarding their application for nusinersen (Spinraza®).
- The Drugs Group is the national committee which the HSE has in place to make recommendations on the pricing and reimbursement of medicines. The membership of the HSE Drugs Group includes public interest members. Pharmacoeconomic reports are reviewed by the HSE Drugs Group along with the outputs of commercial negotiations, and any patient group submission(s) received. The HSE Drugs Group considers all of the evidence and makes a recommendation to the HSE Senior Leadership Team.
- The decision making authority in the HSE is the HSE Senior Leadership Team. The HSE Senior Leadership Team decides on the basis of all the demands it is faced with (across all services) whether it can fund a new medicine, or new use of an existing medicine, from the resources that have been provided to it in line with the Health (Pricing and Supply of Medical Goods) Act 2013.
- A completed HTA is required from the applicant (Biogen) to progress this application, as per the formal processes governing the pricing and reimbursement of medicines.

The application remains under consideration with the HSE. The HSE cannot make any comment on possible outcomes from the ongoing assessment processes.

Yours sincerely,

Suzanne Doyle
Primary Care Reimbursement Service

The Health Service Executive operates the General Medical Services Scheme, which includes Medical Cards and GP Visit Cards, under the Health Act 1970, as amended. It has established a dedicated contact service for members of the Oireachtas specifically for queries relating to the status of Medical Cards and GP Visit Cards applications, which the Deputy / Senator may wish to use for an earlier response. Tel: 01-8647180 / email: Oireachtas.pcrs@hse.ie