



Pádraig O'Sullivan, T.D.
Dáil Éireann,
Leinster House,
Kildare Street,
Dublin 2.

10th November 2022

PQ: 53046/22

To ask the Minister for Health if a decision has been made on the reimbursement of a drug (Luxturna); if he will provide an update on the process; if it has not been approved, if he will provide an indicative timeline for same; and if he will make a statement on the matter. -Pádraig O'Sullivan

Dear Deputy O'Sullivan,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 53046/22), 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 Voretigene neparvovec (Luxturna®):

The HSE received an application for pricing / reimbursement of Voretigene neparvovec (Luxturna®) on the 23rd of September 2019 from Novartis for the treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic *RPE65* mutations and who have sufficient viable retinal cells.

- The first step in the process is the submission of a Rapid Review dossier. The HSE commissioned the Rapid Review process on the 24th of September 2019. The NCPE Rapid Review assessment report was received by the HSE on the 23rd of October 2019. The NCPE advised the HSE that a full Health Technology Assessment (HTA) was required for this medicine.
- The HSE commissioned a full Health Technology Assessment (HTA) on the 29th of October 2019 as per agreed processes.
- The NCPE Health Technology Assessment report (<http://www.ncpe.ie/wp-content/uploads/2019/10/Technical-Summary-document-voretigene-neparvovec-Luxturna.pdf>) was received by the HSE on the 18th of September 2020. The NCPE recommended that Voretigene neparvovec (Luxturna®) not be considered for reimbursement unless cost-effectiveness can be improved relative to existing treatments.
- The HSE Corporate Pharmaceutical Unit (CPU) is the interface between the HSE and the Pharmaceutical Industry in relation to medicine pricing and reimbursement applications. CPU engaged in commercial negotiations with Novartis in November 2020 and June 2022 regarding their application for Voretigene neparvovec (Luxturna®).
- 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. In February 2021 the Drugs Group requested Patient and Clinician Engagement input via the Rare Diseases Technology Review Committee (RDTRC) to assist the group in making its recommendation to the HSE Executive Management Team regarding reimbursement of Voretigene neparvovec (Luxturna®). The Drugs Group specifically requested a prescribing guideline for Voretigene neparvovec (Luxturna®) to inform their deliberations. The prescribing guideline was received on the 19th May 2022. The totality of clinical and economic evidence for Voretigene neparvovec was then comprehensively and extensively reviewed by the Drugs Group at the July 2022

meeting. The Group unanimously recommended in favour of hospital pricing approval of Voretigene neparvovec subject to the establishment of a HSE Medicines Management Programme-led managed access programme. <https://www.hse.ie/eng/about/who/cpu/drugs-group-minutes/hse-drugs-group-minutes-july-2022.pdf>

- The decision making authority in the HSE is the HSE Executive Management Team. The HSE Executive Management 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 uses 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. The HSE EMT supported hospital pricing approval of Voretigene Neparvovec (Luxturna®) for the treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallelic RPE65 mutations and who have sufficient viable retinal cells, subject to a managed access programme being implemented.
- As a condition of reimbursement, an individual patient approval system will be put in place by the HSE, to enable reimbursement for patients who meet the pre-defined criteria as per a Medicines Management Programme (MMP) devised managed access programme. The processes necessary to implement this required managed access programme (MAP) are currently being developed by the HSE MMP.

As part of the National Service Plan 2022 and budgetary process, the HSE worked closely with the Department of Health to secure an allocation of €30m for new medicines in 2022. Funding of Voretigene Neparvovec (Luxturna®) will be from this allocation. The HSE cannot comment on the specific timeline for the HSE approval to be formalised while the processes required to implement the managed access programme are ongoing.

Yours sincerely,



Suzanne Doyle
Primary Care Eligibility & Reimbursement Service