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Michael Healy Rae, T.D.
Dáil Éireann,
Leinster House,
Kildare Street,
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26th June 2024

PQ: 26584/24

To ask the Minister for Health the reason pegvaliase, a game-changing medicine for a handful of phenylketonuria patients, has taken more than four years to go through the various stages of the HSE's appraisal process (details supplied); where this currently is in the system; the other steps required; when they will be completed; and if he will make a statement on the matter. -Michael Healy-Rae

Dear Deputy Healy Rae,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 26584/24), 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 Pegvaliase (Palynziq®):

The HSE received an application for pricing / reimbursement on the 10th January 2020 from Biomarin (the applicant) for Pegvaliase (Palynziq®) indicated for the treatment of patients with phenylketonuria aged 16 years and older who have inadequate blood phenylalanine control (blood phenylalanine levels greater than 600 micromol/l) despite prior management with available treatment options.

- 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 13th January 2020.

- The NCPE Rapid Review assessment report was received by the HSE on the 25th February 2020. The NCPE advised the HSE that a full HTA was recommended to assess the clinical effectiveness and cost effectiveness of Pegvaliase (Palynziq®) compared with the current standard of care.

- The HSE commissioned a full Health Technology Assessment (HTA) on the 26th February 2020 as per agreed processes.

- The NCPE Health Technology Assessment Report was received by the HSE on the 29th September 2022. The NCPE recommended that Pegvaliase (Palynziq®) be considered for reimbursement if cost-effectiveness can be improved relative to existing treatments. https://www.ncpe.ie/pegvaliase-palynziq-hta-id-21057/

- 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 met with the applicant company (Biomarin) to discuss their application for Pegvaliase (Palynziq®).

- 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. The HSE Drugs Group consider all of the evidence and make a recommendation to the HSE Executive Management Team. Pegvaliase (Palynziq®) was reviewed by the Drugs Group at the June 2023 meeting. At the June 2023 meeting 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 Pegvaliase (Palynziq®). The RDTRC

Statement was received in December 2023. The totality of clinical and economic evidence for Pegvaliase (Palynziq®) as well as the RDTRC statement was comprehensively and extensively reviewed by the Drugs Group at the January 2024 meeting. The Drugs Group by majority did not recommend in favour of reimbursement of Pegvaliase (Palynziq®). https://www.hse.ie/eng/about/who/cpu/drugs-group-minutes/

- 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 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. The HSE Executive Management Team supported the Drugs Group recommendation not to reimburse Pegvaliase (Palynziq®) indicated for the treatment of patients with phenylketonuria aged 16 years and older who have inadequate blood phenylalanine control (blood phenylalanine levels greater than 600 micromol/l) despite prior management with available treatment options.

The application for Pegvaliase (Palynziq®) remains under consideration. The HSE cannot make any comment on possible outcomes from the ongoing process.

Yours sincerely,

Suzanne Doyle
Primary Care Eligibility & Reimbursement Service