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Bernard J. Durkin, T.D.
Dáil Éireann,
Leinster House,
Kildare Street,
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14th October 2021

PQ: 47138/21

To ask the Minister for Health the extent to which medicines for the treatment of rare diseases continue to be approved with particular reference to those deemed to make a considerable beneficial impact; the number of such medicines currently under examination; the number approved in the past year; the expectation for the future; and if he will make a statement on the matter. -Bernard J. Durkan

Dear Deputy Durkin,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 47138/21), which you submitted to the Minister for Health for response.

The Committee for Orphan Medicinal Products (COMP) is the European Medicines Agency's (EMA) committee responsible for recommending orphan designation of medicines for rare diseases. This designation is for medicines to be developed for the diagnosis, prevention or treatment of rare diseases that are life-threatening or very serious. In the European Union (EU), a disease is defined as rare if it affects fewer than 5 in 10,000 people across the EU. The European Commission decides whether to grant an orphan designation for the medicine based on the COMP's opinion. The HSE CPU does not maintain a register of medicines for rare diseases and/or designated orphan medicines as the orphan status for a medicine is subject to change (i.e. may be removed from the Community Register of designated Orphan Medicinal Products). As outlined above, the appropriate repository for this information is the EMA website (https://www.ema.europa.eu/en).

There is a national decision process for new medicines and new uses of existing medicines which is underpinned by primary legislation (Health (Pricing and Supply of Medical Goods) Act 2013). The HSE must comply with the relevant legislation when considering investment decisions around new medicines. HSE decisions on which medicines are reimbursed by the taxpayer are made on objective, scientific and economic grounds.

The HSE considers pricing applications for new medicines and new uses of existing medicines in line with the criteria set out under the Health (Pricing and Supply of Medical Goods) Act

2013. The HSE considers the following criteria prior to making any decision on funding / reimbursement:

(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

The HSE Drugs Group is a national committee and it is tasked with providing a recommendation to the HSE Executive Management Team (EMT) in relation to the pricing and reimbursement of new medicines / new uses of existing medicines, and it considers all the criteria in the Health (Pricing and Supply of Medical Goods) Act 2013. The Drugs Group considers the health technology assessment (HTA), the outputs from commercial negotiation, as well as a range of other information in advance of providing its advice to the EMT. Minutes from Drugs Group meetings are publically available at: 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 (EMT) 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 Health (Pricing and Supply of Medical Goods) Act 2013 does not include provision for a different rule set when assessing medicines for rare diseases and/or designated orphan medicines.

The HSE approved 8 applications for funding for designated orphan medicines in 2020.

The HSE has approved 14 applications for funding for designated orphan medicines in 2021 to date (as of 13th October 2021).

Approvals of orphan medicines to date include medicines for the treatment of rare cancers, amyloidosis and cystic fibrosis.

There are 26 pricing and reimbursement applications in process for designated orphan medicines (encompassing new medicines and new uses of existing medicines), as of 13th October 2021.

The HSE cannot make any comment on possible outcomes in relation to applications in the ongoing process.
Yours sincerely,

Suzanne Doyle
Primary Care Eligibility & Reimbursement Service