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Bernard J Durkan, T.D.
Dáil Éireann,
Leinster House,
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5th January 2023

PQ: 62832/22

To ask the Minister for Health the total number of rare and orphan drugs currently being evaluated for use in Ireland; the length of time that normally required for this process; the drugs that have taken an inordinate amount of time to evaluate and approve for use here; and if he will make a statement on the matter. -Bernard J. Durkan

Dear Deputy Durkan,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 62832/22), which you submitted to the Minister for Health for response.

Note on medicines for rare diseases: *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 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/human-regulatory/overview/orphan-designation-overview)

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 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 minutes of the HSE Drugs Group meetings are published and publically available online: https://www.hse.ie/eng/about/who/cpu/drugs-group-minutes/. The HSE Drugs Group recommendation for each medicine reviewed is also included in the published 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 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 following list details the new medicines/new use of medicines that were approved for reimbursement in 2021 and 2022 that maintained their orphan designation at the time of approval:

International Non-proprietary Name	Brand Name	HSE Reimbursement/Pricing Approval
2021		
Teduglutide	Revestive	Jan-21
Tezacaftor, Ivacaftor (paediatric)	Symkevi	Jan-21
Blinatumomab	Blincyto	Feb-21
Letermovir	Prevymis	Feb-21
Liposomal Daunorubicin + Cytarabine	Vyxeos Liposomal	Feb-21
Lutetium (177Lu) Oxodotreotide	Lutathera	Feb-21
Niraparib	Zejula	Mar-21
Burosumab	Crysvita	May-21
Tisagenlecleucel (ALL)	Kymriah	Jul-21
Tisagenlecleucel (DLBCL)	Kymriah	Jul-21
Ivacaftor, Tezacaftor, Elexacaftor	Kaftrio	Aug-21
Lanadelumab	Takhzyro	Sep-21
Midostaurin	Rydapt	Oct-21
Onasemnogene abeparvovec	Zolgensma	Oct-21
Patisiran	Onpattro	Oct-21
Cannabidiol (Dravet Syndrome)	Epidyolex	Dec-21
Cannabidiol (Lennox-Gastaut Syndrome)	Epidyolex	Dec-21
Cannabidiol (Tuberous Sclerosis Complex)	Epidyolex	Dec-21
Polatuzumab vedotin	Polivy	Dec-21

International Non-proprietary Name	Brand Name	HSE Reimbursement/Pricing Approval
2022		
Tafamidis	Vyndaqel	Mar-22
Axicabtagene ciloleucel	Yescarta	Apr-22
Ivacaftor, Tezacaftor, Elexacaftor (subset of 6-11 year licence)	Kaftrio	May-22
Blinatumomab	Blincyto	May-22
Daratumumab	Darzalex	Jun-22
Pasireotide	Signifor	Jul-22
Inotersen	Tegsedi	Aug-22
Obeticholic acid	Ocaliva	Oct-22
Somatrogon	Ngenla	Nov-22
Ketoconazole	Ketoconazole HRA	Dec-22
Cholic acid	Orphacol	Dec-22
Fedratinib	Inrebic	Dec-22
Brentuximab (CTCL)	Adcetris	Dec-22
Brentuximab (HL)	Adcetris	Dec-22
Brentuximab (sALCL)	Adcetris	Dec-22

In 2020 and 2021 the HSE approved approximately half of all applications for pricing and reimbursement within 60 days of receipt of the application. This includes applications for generic medicines, biosimilar medicines, hybrid medicines and new chemical entities (orphan and non-orphan) and excludes applications for parallel imported medicines.

Yours sincerely,

Suzanne Doyle
Primary Care Eligibility & Reimbursement Service