Feidhmeannacht na Seirbhíse Sláinte, Seirbhís Aisíocaíochta Cúraim Phríomhúil
Bealach amach 5, M50, An Bóthar Thuaidh, Fionnghlas
Baile Átha Cliath 11, D11 XKF3
Fón: (01) 864 7100 Facs: (01) 834 3589

Health Service Executive, Primary Care Reimbursement Service
Exit 5, M50, North Road, Finglas,
Dublin 11, D11 XKF3
Tel: (01) 864 7100 Fax: (01) 834 3589

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

23rd May 2024

PQ: 21075/24:
To ask the Minister for Health to provide a list of all orphan medicines reimbursed in 2023; to confirm the date of the HSE executive management team decision; the date on which a reimbursement decision was subsequently made; 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 21075/24), which you submitted to the Minister for Health for response.

Note on orphan drug status: *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.*

Medicines are reimbursed and funded across a range of different systems e.g. in hospitals, in community services and under National Community Drug Schemes and centrally funded arrangements.

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 2023 that maintained their orphan designation at the time of approval provided alongside the HSE Executive Management Team decisions for the relevant medicines.

Number	International Non-proprietary Name	Brand Name	Indication	Applicant	HSE Executive Management Team Decision	HSE Reimbursement/Pricing Approval
2023						
1	Voretigene neparvovec	Luxturna®	Indicated for the treatment of adult and paediatric patients with vision loss due to inherited retinal dystrophy caused by confirmed biallellic *RPE65* mutations and who have sufficient viable retinal cells	Novartis	Approved for funding/reimbursement (July 22)	Aug-23
2	Mogamulizumab	Poteligeo®	Indicated for the treatment of adult patients with mycosis fungoides (MF) or Sézary syndrome (SS) who have received at least one prior systemic therapy	Kyowa Kirin	Approved for funding/reimbursement (March 2023)	May-23
3	Niraparib	Zejula®	As monotherapy for the maintenance treatment of adult patients with advanced epithelial (FIGO Stages III and IV) high-grade ovarian, fallopian tube or primary peritoneal cancer who are in response (complete or partial) following completion of first-line platinum-based chemotherapy	GSK	Approved for funding/reimbursement (March 2023)	Apr-23
4	(Ivacaftor / Tezacaftor / Elexacaftor) + (Ivacaftor)	Kaftrio® + Kalydeco®	Ivacaftor/Tezacaftor/Elexacaftor (Kaftrio®) in a combination regimen with Ivacaftor (Kalydeco®) in cystic fibrosis patients aged 6 -11 years who are heterozygous for the F508del mutation and either a minimal function (MF) mutation or an unknown mutation in the CFTR gene	Vertex	Approved for funding/reimbursement (March 2023)	Apr-23
5	Amikacin sulfate	ARIKAYCE Liposomal®	Indicated for the treatment of non-tuberculous mycobacterial (NTM) lung infections caused by Mycobacterium avium Complex (MAC) in adults with limited treatment options who do not have cystic fibrosis	Insmed	Approved for funding/reimbursement (May 2023)	Oct-23
6	Asciminib	Scemblix®	For the treatment of adult patients with Philadelphia chromosome-positive chronic myeloid leukaemia in chronic phase (Ph+ CML-CP) previously treated with two or more tyrosine kinase inhibitors	Novartis	Not applicable	Nov-23

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