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Pádraig O'Sullivan, T.D.
Dáil Éireann,
Leinster House,
Kildare Street,
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11th August, 2022

PQ: 40607/22

To ask the Minister for Health the number of orphan medicinal drugs approved for reimbursement for the years 2016 to 2021 and to date in 2022, in tabular form; 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 40607/22), 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 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 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.

Medicines with designated orphan status at date of approval for funding from 2016 to August 10th 2022 are listed below. Medicines are listed more than once in cases where further indications (uses) were approved.

International Non-proprietary Name	Brand Name	HSE Reimbursement/Pricing Approval
2016		
Lenvatinib	Lenvima	Jan-16
Pomalidomide	Imnovid	Feb-16
Ibrutinib (1L high risk CLL)	Imbruvica	Aug-16
Ibrutinib (previously treated CLL)	Imbruvica	Aug-16
Ibrutinib (MCL)	Imbruvica	Aug-16
Ibrutinib (WM)	Imbruvica	Aug-16
Ponatinib	Iclusig	Dec-16

International Non-proprietary Name	Brand Name	HSE Reimbursement/Pricing Approval
2017		
Ivacaftor	Kalydeco	Jun-17
Ivacaftor	Kalydeco	Jun-17
Nintedanib	Ofev	Jun-17
Pitolisant	Wakix	Sep-17
Obinutuzumab	Gazyvaro	Oct-17
Migalastat	Galafold	Nov-17
Olaparib	Lynparza	Nov-17

International Non-proprietary Name	Brand Name	HSE Reimbursement/Pricing Approval
2018		
Carfilzomib	Kyprolis	Aug-18
Daratumumab	Darzalex	Apr-18
Eliglustat	Cerdelga	Sep-18
Elosulfase alfa	Vimizim	Apr-18
Glycerol phenylbutyrate	Ravicti	Aug-18
Isavuconazole	Cresemba	Jun-18
Ixazomib	Ninlaro	Dec-18
Venetoclax	Venclyxto	Dec-18

International Non-proprietary Name	Brand Name	HSE Reimbursement/Pricing Approval
2019		
Ataluren	Translarna	May-19
Blinatumomab	Blincyto	May-19
Blinatumomab	Blincyto	May-19
Dinutuximab	Qarziba	May-19
Idebenone	Raxone	May-19
Inotuzumab	Besponsa	May-19
Ivacaftor	Kalydeco	Mar-19
Mercaptamine	Procysbi	Jul-19
Nusinersen	Spinraza	Jul-19
Obinutuzumab	Gazyvaro	May-19
Sebelipase Alfa	Kanuma	Jul-19
Tezacaftor, Ivacaftor	Symkevi	Jan-19

International Non-proprietary Name	Brand Name	HSE Reimbursement/Pricing Approval
2020		
Carfilzomib	Kyprolis	Oct-20
Cerliponase alfa	Brineura	Nov-20
Daratumumab+BOR+DEX	Darzalex	Oct-20
Gemtuzumab Ozogamacin	Mylotarg	Nov-20
Ivacaftor	Kalydeco	May-20
Ivacaftor	Kalydeco	Jul-20
Ivacaftor	Kalydeco	Dec-20
Ivacaftor, Tezacaftor, Elexacaftor	Kaftrio	Oct-20

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		
Axicabtagene ciloleucel	Yescarta	Apr-22
Blinatumomab	Blincyto	May-22
Tafamidis	Vyndaqel	Mar-22
Ivacaftor, Tezacaftor, Elexacaftor (subset of 6-11 year licence)	Kaftrio	May-22
Daratumumab	Darzalex	Jun-22
Pasireotide	Signifor	Jul-22
Inotersen	Tegsedi	Aug-22

There are a number of pricing and reimbursement applications for new medicines (or new uses of existing medicines) with orphan status currently in process. The HSE anticipates that a number of additional medicines with orphan status may be approved throughout 2022.

Yours sincerely,

Suzanne Doyle
Primary Care Eligibility & Reimbursement Service