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Pádraig O'Sullivan, T.D.
Dáil Éireann,
Leinster House,
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27th March 2023

PQ: 12035/23

To ask the Minister for Health the number of EMA-approved orphan drugs that were approved for reimbursement in each of the years 2016 to 2022; the date these drugs were applied for initially, 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 12035/23), 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 first step in the process is the submission of a Rapid Review dossier (a clinical and economic dossier) to the National Centre for Phamacoeconomics (NCPE) for assessment.

The NCPE publishes details of medicines where the HSE has commissioned a Rapid Review assessment and / or a full health technology assessment on their website. The website is updated at regular intervals and includes assessment outcomes and updates on reimbursement for each individual medicine and indication listed. Details of NCPE assessments are available at https://www.ncpe.ie/assessments/

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 2016 – 2022 that maintained their orphan designation at the time of approval:

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 (R117H)	Kalydeco	Jun-17
Ivacaftor (2-5 years)	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		
Daratumumab	Darzalex	Apr-18
Elosulfase alfa	Vimizim	Apr-18
Isavuconazole	Cresemba	Jun-18
Carfilzomib	Kyprolis	Aug-18
Glycerol phenylbutyrate	Ravicti	Aug-18
Eliglustat	Cerdelga	Sep-18
Ixazomib	Ninlaro	Dec-18
Venetoclax	Venclyxto	Dec-18

International Non-proprietary Name	Brand Name	HSE Reimbursement/Pricing Approval
2019		
Tezacaftor, Ivacaftor	Symkevi	Jan-19
Ivacaftor (1-2 years)	Kalydeco	Mar-19
Ataluren	Translarna	May-19
Blinatumomab (adult)	Blincyto	May-19
Blinatumomab (paediatric)	Blincyto	May-19
Dinutuximab	Qarziba	May-19
Idebenone	Raxone	May-19
Inotuzumab	Besponsa	May-19
Obinutuzumab	Gazyvaro	May-19
Mercaptamine	Procysbi	Jul-19
Nusinersen	Spinraza	Jul-19
Sebelipase Alfa	Kanuma	Jul-19

International Non-proprietary Name	Brand Name	HSE Reimbursement/Pricing Approval
2020		
Ivacaftor (6 months+)	Kalydeco	May-20
Ivacaftor (R117H 6 months+)	Kalydeco	Jul-20
Carfilzomib	Kyprolis	Oct-20
Daratumumab+BOR+DEX	Darzalex	Oct-20
Ivacaftor, Tezacaftor, Elexacaftor	Kaftrio	Oct-20
Cerliponase alfa	Brineura	Nov-20
Gemtuzumab Ozogamacin	Mylotarg	Nov-20
Ivacaftor	Kalydeco	Dec-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		
Tafamidis	Vyndaqel	Mar-22
Axicabtagene ciloleucel	Yescarta	Apr-22
Blinatumomab	Blincyto	May-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
Obeticholic acid	Ocaliva	Oct-22
Somatrogon	Ngenla	Nov-22
Cholic Acid	Orphacol	Dec-22
Ketoconazole	Ketoconazole HRA	Dec-22
Fedratinib	Inrebic	Dec-22
Brentuximab (CTCL)	Adcetris	Dec-22
Brentuximab (HL)	Adcetris	Dec-22
Brentuximab (sALCL)	Adcetris	Dec-22
Pomalidomide (Licence extension)	Imnovid	Dec-22

A recently published report prepared by Mazars, examined the governance arrangements around the HSE's drug reimbursement process. An Implementation Working Group led by the Department of Health is being established to consider in greater depth the progression of various recommendations arising from this report. The Working Group will have regard to the policy, legal and institutional framework within which the HSE operates, in particular the requirements set out in the Health (Pricing and Supply of Medical Goods) Act 2013, the Health Act 2004 and the existing processes described in Schedule 1 of the 2021 Framework Agreement on the Supply and Pricing of Medicines. The initial focus of the Group will be to ensure progress of the development by the HSE of the necessary proposals, business case and specifications for:

- the introduction of an application tracker on the HSE's website which details when a reimbursement application is received and whether the application is progressing through the process;
- the introduction of indicative timelines for completing the application process

The HSE is committed to engaging proactively in the Working Group. The HSE has identified that additional resources and investment in IT infrastructure will be required for an online application tracker that will make readily available the date on which new medicines applications are received into the HSE.

Yours sincerely,

Suzanne Doyle