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

4th April 2024

PQ: 10300/24

To ask the Minister for Health if he will supply data, in tabular form, for the ten fastest, and ten slowest approvals of orphan drugs by the HSE, by year (for the period 2014 to 2023), stating the date on which the applications for said drugs were received by the HSE and the data 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 10300/24), 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 HSE does not maintain, within its reimbursement and financial payment systems details on whether a medicines is used for rare diseases and / or designated orphan drugs as the orphan status for a drug is subject to change (i.e. may be removed from the Community Register of designated Orphan Medicinal Products). As outlined above, the EMA does maintain such data on its website (https://www.ema.europa.eu/en/human-regulatory/overview/orphan-designation-overview) and orphan status may change over the product regulatory or reimbursement lifetime.

The HSE does not as a consequence maintain data on orphan status of any medicinal product reimbursable on its databases. The requested information is not currently available to the HSE. To provide the requested information would require a substantial review of applications received since 2014 which would consume significant resources not currently available within the HSE Corporate Pharmaceutical Unit.

The HSE National Service Plan (NSP) 2024 was published in February 2024. The HSE NSP outlines that the HSE will deliver increased visibility on the steps / progress made by each individual medicine through the HSE assessment and approval processes, and prioritise the recruitment necessary to optimise timelines for each application. There is a commitment to review the potential for streamlining controls and validations, while maintaining good governance.

The HSE Corporate Pharmaceutical Unit (CPU) is currently operating at full available capacity and continues to prioritise working on the receipt and progressing of drug applications and productivity work streams committed to under NSP 2024. The HSE CPU continues to collaborate with a range of bodies across the HSE to develop and deliver on large-scale programmes of reform in medicine pricing and utilisation and in wider reimbursement and / or eligibility programmes. This collaborative work represents billions of euros in cost reductions and avoided costs over the last decade.

The work streams of the HSE CPU are resource intensive and require technical staff with significant experience and expertise to ensure optimal outcomes. It is expected that under the 2024 NSP, the HSE Corporate Pharmaceutical Unit will recruit substantial additional resources during 2024 which will facilitate the commitments made.

The HSE does at the end of each year collate a report on the medicines approved during that year. Included in these papers is information in relation to the Orphan status at the point that those reports were produced i.e. in early January of the subsequent year.

The following list details the new medicines / new use of medicines that were approved for reimbursement in 2016-2023 YTD 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
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
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
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
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
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
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
2023		
Niraparib	Zejula	Apr-23
Ivacaftor, Tezacaftor, Elexacaftor (subset of 6-11 year licence)	Kaftrio	Apr-23
Mogamulizumab	Poteligeo	May-23
Risdiplam	Evrysdi	Sep-23
Voretigene neparvovec	Luxturna	Aug-23
Amikacin sulfate	ARIKAYCE Liposomal	Oct-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