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

PQ: 12031/23

To ask the Minister for Health the average length of time it takes to approve an EMA-approved orphan medicine in Ireland, from receipt of application for reimbursement to actual approval; how this compares with EU counterparts; 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 12031/23), which you submitted to the Minister for Health for response.

The European Medicines Agency (EMA) is a centralised agency of the European Union (EU) responsible for the scientific evaluation, supervision and safety monitoring of medicines in the EU. The EMA plays an integral role in the authorisation of medicines in the EU.

'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

Pharmaceutical companies are required to submit formal applications to the HSE if they wish their medicines to be added to the list of reimbursable items / funded via hospitals. The decision of pharmaceutical companies to market licensed medicines i.e. whether or not to submit a formal application, are outside the control of the HSE.

The rate of approval of new medicines (and new use of medicines) has accelerated in line with the provision of significant funding for medicines allocated within the National Service Plans in 2021 and 2022.

In 2021, the HSE approved 29 new medicines and 21 new uses of existing medicines. In 2021 the HSE also approved expanded reimbursement of two further drugs. Of these 2021 approvals, 19 new medicines and new uses of existing medicines maintained their orphan designation at the time of approval.

In 2022, the HSE approved 30 new medicines and 30 new uses of existing medicines. Of these 2022 approvals, 16 medicines and new uses of existing medicines maintained their orphan designation at the time of approval.

To date in 2023, the HSE has approved one new medicine and one new use of an existing medicine. There are 15 new medicines / new uses of existing medicines pending formal approval (subject to the implementation of managed access protocols / completion of administrative processes). Of these medicines / new uses of existing medicines pending formal approval, five have been designated an orphan medicine.

The HSE endeavours to provide access to as many medicines as possible within the resources provided to it and in as timely a fashion as possible. The comprehensive assessment of new

medicines, including in-depth pharmacoeconomic evaluation, the required commercial negotiations and the Drugs Group review processes require significant expertise and due diligence. These processes are by their nature resource intensive. The HSE is committed to making up-to-date information regarding drug reimbursement publicly available. All Drugs Group minutes are published online. The National Cancer Control Programme (NCCP) publishes ongoing information on cancer drug approvals and the Primary Care Reimbursement Service (PCRS) publishes monthly updates to the list of reimbursable items.

The HSE is working on certain improvements identified during the negotiation of the Framework Agreements with Industry in 2021, including a number of enhancements in the application process and further transparency around the application process. These enhancements were committed to by the State so as to assist with the timely processing of applications for pricing and reimbursement.

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 additional resources and investment in IT infrastructure will be required to bring forward the required enhancements to have metrics, such as the average length of time for an application for pricing and reimbursement to be progressed, readily available.

Yours sincerely,

Suzanne Doyle
Primary Care Eligibility & Reimbursement Service