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Pádraig O'Sullivan, T.D.
Dáil Éireann,
Leinster House,
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5th April 2023

PQ: 14116/23

To ask the Minister for Health if he was briefed following the HSE Drugs Committee meeting held on 14 March 2023 where the ongoing kaftrio dispute was discussed; when the HSE leadership team will meet to discuss the outcome of the meeting; if the team will prioritise this issue given the urgency of the situation; 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 14116/23), which you submitted to the Minister for Health for response.

The HSE is committed to providing access to as many medicines as possible, in as timely a fashion as possible, from the resources available (provided) to it.

The HSE robustly assesses applications for pricing and reimbursement to make sure that it can stretch available resources as far as possible and to deliver the best value in relation to each medicine and ultimately more medicines to Irish citizens and patients.

HSE decisions on which medicines are reimbursed by the taxpayer are made on objective, scientific and economic grounds.

There are formal processes which govern applications for the pricing and reimbursement of medicines, and new uses of existing medicines, to be funded and / or reimbursed.

The HSE considers the following criteria prior to making any decision on pricing / reimbursement, in line with the Health (Pricing and Supply of Medical Goods) Act 2013:
(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 has previously approved reimbursement of Kaftrio® (Elexacaftor/Tezacaftor/Ivacaftor) indicated in a combination regimen with Ivacaftor (Kalydeco®) under the High Tech Drug Arrangements for:

i. treatment of cystic fibrosis (CF) in patients aged 6 years and older who are homozygous for the F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

ii. treatment of cystic fibrosis (CF) in patients aged 6 years and older who are heterozygous for the F508del mutation and have one of the following mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene; *R117H, G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N, S549R, P67L, R117C, L206W, R352Q, A455E, D579G, 711+3A→G, S945L, S977F, R1070W, D1152H, 2789+5G→A, 3272-26A→G, and 3849+10kbC→T.*

iii. treatment of cystic fibrosis (CF) in patients aged 12 years and older who have at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

Pricing and reimbursement application for Kaftrio® (Elexacaftor/Tezacaftor/Ivacaftor) for treatment of cystic fibrosis (CF) in patients aged 6 to 11 years who are heterozygous for the F508del mutation and either a minimal function (MF) mutation, or an unknown mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene:

- On 7th January 2022 the European Medicines Agency (EMA) approved a change to the terms of the marketing authorisation for the medicinal product Kaftrio®. The full EMA authorised indication for Kaftrio® is now as follows; Elexacaftor/Tezacaftor/Ivacaftor is indicated in a combination regimen with Ivacaftor (Kalydeco®) for the treatment of cystic fibrosis in patients aged 6 years and older who have at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

- The first step in the process of pricing and reimbursement is the submission of a Rapid Review dossier.

- On the 11th April 2022, the HSE received a Rapid Review dossier from Vertex Pharmaceuticals pertinent to a pricing and reimbursement application for Kaftrio® in combination with Ivacaftor (Kalydeco®) for treatment of cystic fibrosis (CF) in patients aged 6 to 11 years who are heterozygous for the F508del mutation and either a minimal function (MF) mutation, or an unknown mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

- The HSE Corporate Pharmaceutical Unit commissioned, on the 12th April 2022, the National Centre for Pharmacoeconomics (NCPE) to conduct a Rapid Review for Kaftrio® in combination with Ivacaftor (Kalydeco®) for the treatment of cystic fibrosis in patients aged 6 to 11 years who are heterozygous for the F508del mutation and either a minimal function (MF) mutation, or an unknown mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

- The NCPE Rapid Review assessment report was received by the HSE on the 10th May 2022. The NCPE advised that a full HTA was recommended to assess the clinical effectiveness and cost effectiveness of Ivacaftor/Tezacaftor/Elexacaftor (Kaftrio®) with a regimen of Ivacaftor (Kalydeco®) compared with the current standard of care.

- The HSE commissioned a full Health Technology Assessment (HTA) on the 16th May 2022 as per HSE standard processes.

- The NCPE publishes details on 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. Further details are available at https://www.ncpe.ie/ivacaftor-tezacaftor-elexacaftor-kaftrio/

- The NCPE full pharmacoeconomic report was received by the HSE on the 9th February 2023. The NCPE recommended that Kaftrio® plus Ivacaftor be considered for reimbursement if cost-effectiveness can be improved relative to best supportive care: https://www.ncpe.ie/ivacaftor-tezacaftor-elexacaftor-kaftrio/

- The HSE Corporate Pharmaceutical Unit (CPU) is the interface between the HSE and the Pharmaceutical Industry in relation to medicine pricing and reimbursement applications. CPU have met with the applicant company (Vertex Pharmaceuticals) to discuss their application and recommendation(s) received from the NCPE for Kaftrio® (Elexacaftor/Tezacaftor/Ivacaftor).

- The 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 totality of clinical and economic evidence for Kaftrio® (Elexacaftor/Tezacaftor/Ivacaftor) was comprehensively and extensively reviewed by the Drugs Group at the March 2023 meeting. The Group recommended in favour of reimbursement of Kaftrio® (Elexacaftor/Tezacaftor/Ivacaftor) in combination with Ivacaftor (Kalydeco®) for treatment of cystic fibrosis (CF) in patients aged 6 to 11 years who are heterozygous for the F508del mutation and either a minimal function (MF) mutation, or an unknown mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

- 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 EMT supported reimbursement of Kaftrio® (Elexacaftor/Tezacaftor/Ivacaftor) in combination with Ivacaftor (Kalydeco®) for treatment of cystic fibrosis (CF) in patients aged 6 to 11 years who are heterozygous for the F508del mutation and

either a minimal function (MF) mutation, or an unknown mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

- The HSE has approved reimbursement of Kaftrio® (Elexacaftor/Tezacaftor/Ivacaftor) indicated in a combination regimen with Ivacaftor (Kalydeco®) for treatment of cystic fibrosis (CF) in patients aged 6 to 11 years who are heterozygous for the F508del mutation and either a minimal function (MF) mutation, or an unknown mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene under the High Tech Drug Arrangements effective 1st April 2023.

Yours sincerely,

Suzanne Doyle
Primary Care Eligibility & Reimbursement Service