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Jim O'Callaghan, T.D.
Dáil Éireann,
Leinster House,
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11th October, 2022

PQ: 46630/22

To ask the Minister for Health if funding for the cystic fibrosis drug kaftrio will be made available for children once they turn six years of age; and if he will make a statement on the matter.-Jim O'Callaghan

Dear Deputy O'Callaghan,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 46630/22), 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 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.

Ongoing pricing and reimbursement application for Kaftrio® (Elexacaftor/Tezacaftor/Ivacaftor):

- 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 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 have advised that a full HTA is 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. https://www.ncpe.ie/drugs/ivacaftor-tezacaftor-elexacaftor-kaftori-for-the-treatment-of-6-to-11-year-olds-with-cf-with-f508del-mutation-in-the-cftr-gene-hta-id-22022/

- The HSE commissioned a full Health Technology Assessment (HTA) on the 16th May 2022 as per HSE standard processes.
- The HSE Corporate Pharmaceutical Unit (CPU) is the interface between the HSE and the Pharmaceutical Industry in relation to medicine pricing and reimbursement applications.
- The HSE CPU engaged in 11 commercial meetings with Vertex Pharmaceuticals (the applicant company) between January 2022 and September 2022 to discuss this application for pricing and reimbursement of Kaftrio®. The HSE CPU and Vertex Pharmaceuticals discussed issues arising out of the Rapid Review assessment report specific to the subset of the licensed population of 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 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.
- Where additional budget impact arises, the HSE Drugs Group examines the product against the criteria set out in the Health (Pricing and Supply of Medical Goods) Act 2013 in advance of making a recommendation to the HSE Executive Management Team.
- 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 is committed to continuing to communicate and follow up with the applicant (Vertex Pharmaceuticals) in a proactive manner regarding this patient sub-population with a view to addressing the significant additional budget impact to the State proposed by Vertex to enable access to Kaftrio® for this patient group.

The application for pricing and reimbursement of this subset of the licensed population for Kaftrio® remains under consideration with the HSE. The HSE cannot make any comment on possible outcomes from the ongoing process'.

Yours sincerely,

Suzanne Doyle
Primary Care Eligibility & Reimbursement Service