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Denis Naughten, T.D.
Dáil Éireann,
Leinster House,
Kildare Street,
Dublin 2.

25th May 2022

PQ: 23711/22

To ask the Minister for Health when the HSE will approve the reimbursement under the high-tech drug arrangements for kaftrio for children under 12 years of age; the reason for the delay in approval in view of the fact that it has already been approved for children aged 12 years and over; the date when the applicant company submitted a rapid review dossier of evidence to the HSE; the steps taken by the HSE to consider this dossier; the dates on which these steps were taken; if all outstanding queries have been addressed by the applicant company; if so, when; and if he will make a statement on the matter. -Denis Naughten

Dear Deputy Naughten,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 23711/22), 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

In terms of the specific details of the application for pricing and reimbursement of Kaftrio® (Elexacaftor/Tezacaftor/Ivacaftor) indicated in a combination regimen with Ivacaftor (Kalydeco®) for the treatment of cystic fibrosis in patients aged 6 to 11 years who have at least one F508del 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 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 21st March 2022, the HSE received a Rapid Review dossier from Vertex pertinent to a pricing and reimbursement application for Kaftrio® for a subset of the licensed population in the 6-11 year cohort.

- This population is as follows:

    - treatment of cystic fibrosis (CF) in patients aged 6 to 11 years who are homozygous for the F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

    - treatment of cystic fibrosis (CF) in patients aged 6 to 11 years 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.*

- The HSE Corporate Pharmaceutical Unit liaised with the National Clinical Programme for Cystic Fibrosis (NCPCF) in relation to the reimbursement protocol to extend the scope of reimbursement in line with this subset of the licensed population.

- **The above subset of the licensed population for Kaftrio® is approved for reimbursement via the High Tech Drug Arrangements effective May 2022.**

- On the 11th April 2022, the HSE received a second Rapid Review dossier from Vertex pertinent to a pricing and reimbursement application for Kaftrio® for a further subset of the licensed population in the 6-11 year cohort.

- This population is as follows:

    - 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 five commercial meetings with Vertex (the applicant company) between January 6th 2022 and April 14th 2022 to discuss the application for pricing and reimbursement of Kaftrio® for the treatment of cystic fibrosis in patients aged 6 to 11 years who have at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

- The HSE CPU met with Vertex again on May 23rd 2022 to discuss 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 HSE CPU continues to engage with Vertex 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 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 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 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