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Bernard J Durkan, T.D.
Dáil Éireann,
Leinster House,
Kildare Street,
Dublin 2.

5th January 2023

PQ: 62748/22

To ask the Minister for Health the availability to the fullest possible extent of medications to treat cystic fibrosis; the extent to which such drugs are readily available having been tested and approved at European and national level; the extent to which kaftrio is readily available to patients who require its use as a matter of urgency; if there are any issues surrounding its availability which require his intervention; and if he will make a statement on the matter. -Bernard J. Durkan

Dear Deputy Durkan,

The Health Service Executive has been requested to reply directly to you in the context of the above Parliamentary Question (Reference 62748/22), 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 Long Term Illness (LTI) Scheme is a non-means tested, condition specific, prescription charge exempt primary care scheme overseen by the Primary Care Reimbursement Service (PCRS) within the HSE. The scheme commenced in 1970 through the Health Act (1970) and was last amended in 1975. To qualify, a patient must have one (or more) of sixteen eligible conditions. Cystic Fibrosis is one such specified eligible condition.

Drugs and non-drug items reimbursable under the Long Term Illness (LTI) Scheme are intended for the treatment of the primary condition. Core Lists were developed following detailed consultation with Medical Officers, HSE Pharmacists and HSE Medicines Management Programme. The HSE is satisfied that all medicines that should be necessary for the treatment of each primary LTI condition are provided on these Core Lists. The Core Lists are published on the HSE website at https://www2.hse.ie/services/schemes-allowances/lti/approved-medications/.

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 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. 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. A full Health Technology Assessment (HTA) is currently being carried out by the NCPE.

- 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 pharmacoeconomic report will be reviewed by the HSE Drugs Group along with the outputs of commercial negotiations, and any patient group submission(s) received. The HSE Drugs Group will consider all of the evidence and make 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