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Health Service Executive

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Deputy Éamon Ó Cuív TD
Dáil Éireann,
Leinster House,
Kildare Street,
Dublin 2

18 January 2021

PQ 2043/21 To ask the Minister for Health the consideration being given to making a therapy (details supplied) available to those for whom this therapy is the best hope of their retaining vision; the stage the process is at; the estimated number of persons in Ireland that would benefit from the therapy each year; and if he will make a statement on the matter - Luxturna (produced by Novartis)

Dear Deputy Ó Cuív

This PQ relates to the drug Voretigene Neparvovec (Luxtturna)

Voretigene Neparvovec is a gene transfer vector that employs an adeno-associated viral vector serotype 2 (AAV-2) capsid as a delivery vehicle for the human RPE65 protein complimentary deoxyribonucleic acid (c DNA) to the retina. It is licensed for the treatment of inherited retinal dystrophy. It is administered as two subretinal injections, one to each eye, once in a lifetime. The drug may improve functional vision in some patients but questions remain as to the duration of treatment effect in those who respond. The clinical evidence is limited due to the very small number of patients studied in the clinical trials.

It is an expensive therapy with the cost per patient at €810,750 (inclusive of VAT) for the single treatment course (drug cost only). The estimated number of patients eligible for treatment ranges from 9 to 13 and there may be up to two new patients per year thereafter. The 5 year net Budget Impact estimates range from €7,200,000 to €12,300,000 (inclusive of administration and monitoring costs).

In relation to timelines the HSE commissioned the National Centre for Pharmacoeconomics (NCPE) to carry out a full Health Technology Assessment (HTA) of the drug on the 29/10/2019 to determine the cost-effectiveness of the product. The NCPE did not receive a submission from the manufacturer (Novartis) until the 3/4/2020. The NCPE completed the assessment on the 18/9/2020 and found the drug not to be cost effective as the incremental cost-effectiveness ratio (ICER) was €189,037/QALY (quality adjusted life year) well above the cost-effectiveness threshold of €45,000/QALY. The NCPE recommended that the drug should not be reimbursed unless cost-effectiveness can be improved. This usually results in pricing negotiations between the HSE and the manufacturer.

Following receipt of the NCPE report in late September 2020 the HSE will bring the product before the HSE Drugs Group in 2021 (date to be confirmed) who will make a reimbursement

recommendation to the HSE Leadership Team. The HSE Leadership Team will make the reimbursement decision.

I trust this answers your question to your satisfaction.

Yours sincerely

Sharon Hayden
General Manager
Office of the CCO