Joint Committee on Health
Houses of the Oireachtas
Leinster House
Dublin 2

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OPENING STATEMENT

Processes and Criteria Used by the NCPE when evaluating Orphan Drugs

The mission of the NCPE is to facilitate healthcare decisions on the reimbursement of technologies [usually pharmaceuticals], by applying clinical and scientific evidence in a systematic framework, in order to maximize population wellness. The NCPE assessment considers the clinical effectiveness and health related quality of life benefits and all relevant costs including potential savings from reduced healthcare resource use (e.g. hospitalisation), which a new treatment may provide and whether the price requested by the manufacturer is justified. The NCPE will then advise the HSE in relation to the cost effectiveness [value for money] and budget impact associated with the specific pharmaceutical product.

A medicinal product is designated as an orphan medicinal product if it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than 5 in 10,000 persons in the European Union at the time of submission of the designation application. The NCPE has a standardized process and criteria for the evaluation of pharmaceutical products including Orphan Drugs. All assessments are conducted in accordance with published Health Technology Assessment [HTA] guidelines by the Health Information and Quality Authority [HIQA]. These guidelines were produced by the HTA Directorate in HIQA in consultation with it's HTA Scientific Advisory Group. The guidelines were first published by the Authority in 2010 and subsequently updated in 2014 and are available on the HIQA website www.hiqa.ie.

The NCPE HTA process is well established and usually commences when the relevant pharmaceutical company receives notification from the HSE Corporate Pharmaceutical Unit [HSE-CPU] of the requirement for a Rapid Review Submission following a price application for a new medicine. The manufacturer submits the Rapid Review document according to the template available on the NCPE website [www.ncpe.ie]. The document includes a range of information on the relevant product including the regulatory status, the clinical condition, the proposed licensed indication, anticipated place in therapy, relevant comparators, clinical evidence, safety and tolerability in addition to economic considerations.

The NCPE reviews this document within a four week period to determine whether a full pharmacoeconomic assessment is required or not. If a full pharmacoeconomic assessment is not required the product is usually reimbursed. A published assessment of the process from 2010 to 2015 indicates that approximately 50% of products do not require a full assessment and are reimbursed in a short period of time.

If a full pharmacoeconomic assessment [HTA] is required the manufacturer is directed to the applicant template for submission of a full assessment which is available on the NCPE website. A full HTA investigates in detail the value for money proposition associated with medications. Orphan Drugs are assessed through the same mechanism as other drugs. The assessment includes a description of the relevant condition and its management and a detailed outline of the intervention under assessment. The clinical evidence supporting the efficacy of the product is reviewed. The manufacturer is required to outline in detail the health economics in relation to the product and provide an estimate of the incremental cost effectiveness of the drug i.e. the added benefit for the additional cost. A budget impact analysis is also required in addition to the status of HTA assessments in other jurisdictions.

The HTA submission process also facilitates submissions by patient groups who wish to have their views taken into consideration during the assessment process. The patient group submission template is also available on the NCPE website. Having reviewed all the available documentation the NCPE submits its report to the HSE Corporate Pharmaceutical Unit following a check for factual accuracy by the manufacturer.

Examples of Orphan Drugs that have been considered by the NCPE include Agalsidase alpha [Replagal] α Agalsidase beta [Fabrazyme] for Fabry disease [2004], Eculizumab [Soliris] for Paroxysmal Nocturnal Haemoglobinuria [PNH] in 2010 and 2013, Ivacaftor [Kalydeco] for Cystic Fibrosis [2013], Ataluren [Translarna] for Duchenne Muscular Dystrophy [2016], Lumacaftor/Ivacaftor [Orkambi] for Cystic Fibrosis [2016], Elosulfase alpha [Vimizim] for mucopolysaccharidosis, type IV A [Morquio A syndrome] [2016], Human alpha 1 proteinase inhibitor [Respreeza] for emphysema in adults with documented alpha 1 proteinase inhibitor deficiency [2016] and Migalastat [Galafold] for Fabry disease [2017].

The HSE-CPU will forward the NCPE Report and any other relevant information to the HSE Drugs Group for their consideration. The final decision on reimbursement of any drug, including Orphan drugs, is made by the HSE, not the NCPE