Technology Review Committee for Rare Diseases –Terms of Reference

Responsibilities

The Rare Diseases Medicinal Products/ Technology Review Committee is responsible for:

1. reviewing proposals received from industry or expert groups in Ireland for funding of new products[1] for rare diseases[2], or expanded indications for existing products for rare diseases and making recommendations as to the implementation of the relevant recommendations from the National Rare Diseases Plan 2011-2018; and
2. providing contributions to the development of clinical guidelines for relevant Orphan Medicinal Products (OMPs) and supporting the implementation of guidelines in conjunction with the National Drugs Management Programme Office where applicable.

The Committee's remit covers licensed indications for medicines for rare diseases. The Committee's remit excludes unlicensed indications in Europe for medicines for rare diseases. The Committee's remit also excludes medicines for rare cancers and infectious diseases as there is an existing process undertaken by the National Cancer Control Programme (NCCP) for the relevant medicines.

The Committee's recommendations for reimbursement of OMPs are not intended to replace any part of the existing medicines appraisal or reimbursement process. The recommendations will be informed by a Health Technology Assessment (HTA) submission, or similar, by the National Centre for Pharmacoeconomic Evaluation (NCPE) or other body, Committee discussion, and guidelines developed by the relevant clinical group/Clinical Lead of Centre of Expertise.

Governance

The Committee reports to the National Director of the Acute Hospitals Division (or a nominee) and will make recommendations regarding the priority for consideration of funding and availability of a new treatments to the HSE Drugs Committee. The recommendations will be based on the degree of unmet clinical need, clinical effectiveness, alternative therapies available, toxicity (where relevant) and the cost effectiveness of the proposed technology. These criteria are based on Schedule 3, Part 3 of the *Health (Pricing and Supply of Medical Goods) Act 2013.*

Membership

Members of the Committee, observers and invited experts must keep all discussions and commercial information confidential.
Recommendations[3] of the committee to the Chairperson (to be appointed by HSE Management) will be communicated by the Chairperson to the HSE Drugs Committee, in the case of medicines, or other relevant HSE Group in the case of tests or other technologies, for potential funding and implementation planning.

Chairman:
Chair will be appointed by the National Clinical Advisor and Group Lead Acute Hospitals / National Director Acute Hospitals with input from the Clinical Lead for the National Clinical Programme for Rare Diseases for a two-year term. Reappointment may be made once, for a maximum term of four consecutive years in total.

Members:

1. A minimum of three members who are Consultants in Rare or highly specialised diseases, recommended by the relevant professional society, faculty or college, who have content experience in the specific discipline and are approved by the Clinical Lead for the National Clinical Programme for Rare Diseases (e.g. genetic and metabolic diseases, rare coagulopathies such as Haemophilia, and neurodegenerative diseases).
2. The Clinical Lead for the National Clinical Programme for Rare Diseases.

[1] Product is defined for this purpose as an item that has marketing authorisation in Ireland.

[2] A rare disease is defined in the EU as a disease or disorder affecting fewer than 5 in 10,000 of the European population (Source: National Rare Diseases Plan for Ireland 2014 – 2018, p. 7).

[3] The committee will provide an indicative recommendation in relation to each technology considered, indicating in each case whether the technology is:

- Recommended;
- Not recommended;
- Recommended pending an improved business impact following discussion with company required;
- Additional information required from company; or
- Other (to be defined in relation to the specific technology considered).

3. Chief II Pharmacist x 2 (to include one with Health Economical, Pharmacoeconomics or statistics and epidemiology expertise.).
4. A representative from PCRS.
5. A minimum of one invited participant from a related designated centre of expertise, recommended by the Clinical Advisory Group for the National Clinical Programme for Rare Diseases, as required, according to this speciality area.
6. One representative appointed by HIQA.
7. Two public / patient representatives from a selected panel of three.
8. Up to three additional members may be appointed.

Review of Terms of Reference and membership:
Terms of Reference and membership will be reviewed every three years.

Attendance:
Non-attendance at three consecutive meetings may lead to removal from the membership of the Committee and the appointment of a replacement.

Observers:
Programme Manager, National Clinical Programme for Rare Diseases
National Clinical Advisor and Group Lead, Acute Hospitals Division (or designate)

Invited Experts:
Experts in a particular area, who are not members of the committee, may be invited to attend specific meetings or for specific items at a meeting as appropriate.

Quorum:
At least five members, including a minimum of two clinicians from membership grouping 1(see list above).

Secretariat:
The secretariat to the Committee is provided by the NCPE / pharmacist support X 2 to the Committee.

Requirements of Chairman, members, observers & invited experts

- The Chairman, members and observers must complete a conflict of interest declaration annually. Invited experts may be required to complete a conflict of interest declaration.

- Members, observers or invited experts with a conflict of interest with regard to a particular technology should declare the conflict to the committee, or to the committee chair. The member / observer / invited expert may choose to withdraw from discussion of specific item, or withdraw at the request of the Committee, or the chair.