

IPPOSI submission to the Joint Oireachtas Committee on Health

Health (Pricing and Supply of Medical Goods) (Amendment) Bill April 2018

Find below a written submission from the Irish Platform for Patient Organisations, Science & Industry (IPPOSI) to the Ammendment Bill above, created by the IPPOSI Executive and endorsed by the IPPOSI Board.

Submission

Overall, it is evident to IPPOSI that the 2013 Act does not sufficiently recognise or allow for orphan medicinal products to be considered differently than non-orphan medicines.

Section 2: Definition

- IPPOSI welcomes the addition of the definition of Orphan Medicinal products within the proposed Ammendment Bill. IPPOSI believes that an additional sub-definition of 'ultra-orphan' should be considered (see next point below).
- The Scottish Government [recently introduced a new definition of 'ultra-orphan medicines'](#) that can treat very rare conditions affecting fewer than 1 in 50,000 people. (Rare Diseases prevalence is < 1:2000 people). This new definition allows the Scottish Medicines Consortium (SMC) the ability to treat some medicines for rare orphan diseases as ultra-orphan medicines. The change means if the medicine meets the new definition of an ultra-orphan medicine and the SMC considers it clinically effective, then it will be made available on the NHS for at least three years while information on its effectiveness is gathered. IPPOSI encourages consideration of this model for defining and assessing ultra-orphan medicinal products for Ireland.

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Section 3: Assessment Criteria

- IPPOSI believes a more ‘principled’ approach to medicinal product assessments, such as in Sweden, should be developed in Ireland. This approach of using underpinning principles that actively discriminate FOR vulnerable groups and patients in the greatest need, ensures that solidarity and treatment takes precedence over cost effectiveness. While Sweden uses QALY to assess value, there is no strict threshold.
- As part of this principled approach to all medicinal product assessments, it is incumbent on the State to prioritise solidarity with those living with a rare disease by ensuring assessments take into account the specific challenges of orphan medicinal products.
- Recent data indicates that Ireland is struggling to provide timely access to orphan medicines. It is therefore appropriate that an alternative assessment pathway should be developed for orphan medicines that preserves the integrity of the NCPE and its processes yet achieves the intended level of access to these medicines.
- The establishment of the Rare Diseases Medicinal Products Technology Review committee for therapy submissions in Ireland has been a welcome step, in particular with the level of patient involvement. However, the committee does not assess all orphan medicinal products, and only becomes involved in the process at the request of the HSE Drugs Group.
- In light of the ongoing debate around the use of QALY and the different outcome measures used in other countries, the amendment to ensure that ICER-related threshold assessments are to be considered as non-relevant for orphan medicinal products is supported by IPPOSI.
- However, the proposed Amendment appears to assume that all orphan medicinal products are expensive, which is not in fact the reality. IPPOSI is concerned that this may result in higher pricing strategies from companies.
- QALY can still be used to assess value of orphan medicinal products, but without the need for strict ICER thresholds. The inclusion of outcomes-based contracts as a criterion can help to determine value-for-money in this regard. Also, evaluation outcomes should be presented in QALYs AND in life years gained – including for evaluations where the main objective of the intervention is to prolong life expectancy.

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- As outlined in the MRCG-IPPOSI ‘Drug Iceberg’ reports¹, only via a thorough root-and-branch-review of the entire process of assessment & reimbursement by an independent academic, will the actual levels of access to innovative drug therapies in Ireland be independently determined, and recommendations made on improving levels of access for patients in Ireland.

Section 4: New /Adjusted Criteria

- It is appropriate that the existing criteria (a), (b), (c), (e), (f), and (g) remain in place and unchanged.
- The modification to criteria (d) is to be welcomed in order to make assessments more relevant to the rare disease population in question. We believe that ‘innovative pricing models’ should also be included in this criterion, as a number of cell & gene therapies for rare diseases are potentially curative.
- The addition of criteria (i) is welcomed by IPPOSI, from the perspective of making the assessment and reimbursement process more transparent and relevant to patients and their representative organisations.
- The addition of criteria (j) is welcomed by IPPOSI as patients are often unclear as to the degree of weight that is given for diseases that are often chronic in nature with an associated burden on patients, their families and on the state. In this regard, evaluation and analysis should move to consider both health and societal perspectives, and costs should consider direct costs (health care) and indirect costs (costs to other government bodies, informal caregivers etc). We also believe that ‘life expectancy’ should also be considered as part of this criteria to allow for consideration of the specific challenges of end-of-life medications.
- The modification to criteria (h) is welcomed from the perspective of tailoring the assessment to accommodate for the inherent difficulties of collecting the data in rare disease populations.

¹ https://www.ipposi.ie/wp-content/uploads/2018/02/IPPOSI_MRCG-Drug-Iceberg-2.0.pdf



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- The modification to criteria (k) is welcomed but should be modified further to ‘the availability of the medicine in other relevant European countries’, to avoid comparison with countries with little or no relevance to the Irish context.
- Any changes to criteria within the existing Act should be accompanied with the necessary changes to infrastructure and data that will allow the insertion of orpha-codes into existing IT systems in both hospital and primary care settings, and will also allow the monitoring of patient outcomes of most relevant to patients to be measured.

About IPPOSI

The Irish Platform for Patient Organisations, Science and Industry (IPPOSI – www.ipposi.ie) is a patient-led organisation that works with patients, government, industry, science and academia to put patients at the heart of policy and innovations. IPPOSI seeks to build consensus among member groups on existing and emerging health issues and policies.

IPPOSI membership includes over 100 patient organisations and medical charities, over 250 individuals from a scientific/clinical and healthcare background and 22 healthcare industry companies.

IPPOSI has been co-funded by the Health Research Board (HRB) on behalf of the Department of Health since 2007. HRB co-funding, accounts for approximately 25% of IPPOSI annual income whilst the remainder is generated from a combination of leveraged public EU and national grants as well as industry member subscriptions and sponsorships.

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