Equitable access to medicines for rare disease patients in Wales

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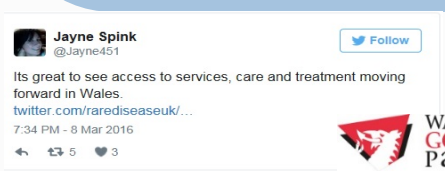
Introduction: The magnified impact of rare diseases raises the significance of innovative treatments arriving on the horizon. It is imperative that these potential cures and treatments are made available in Wales, for the benefit of patients, their families and wider society. Health technology appraisal (HTA) is vital in ensuring patient access to treatments. The nature of rare diseases presents a number of challenges that must be met to ensure an effective appraisal process can be carried out.

Aim: To develop an appraisal process for orphan and ultra-orphan medicines which considers multiple criteria and reflected value outside of the traditional cost effectiveness thresholds such as the quality adjusted life year (QALY) methodology.

Approach: Evidence sessions held by the Welsh Government review group gave an opportunity for clinicians and patient groups to identify issues which were not taken into consideration in the current system:

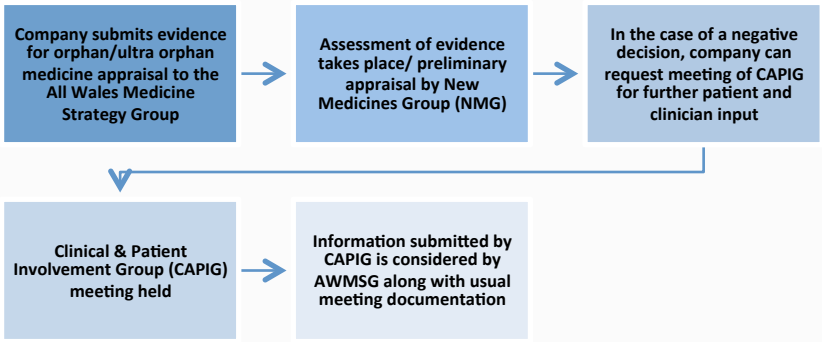
- Small patient populations make large population studies impossible leading to a lack of evidence regarding efficacy of medicines.
- The unmet need, a feature of most rare diseases means there may not be a comparator medicine to judge the new treatment against. New treatments for rare diseases are often compared against the existing treatment, palliative care, which is cheap.
- Fewer patients may receive the drug, limiting opportunities for medicine development costs to be recuperated, driving the price of orphan medicines upwards.

Consultation took place with patients, families and patient group representatives to discuss the issues and identify ways to ensure equitable access to orphan and ultra-orphan medicines in Wales.



Outcome:

New Appraisal Process for Orphan/Ultra Orphan Medicines



The Clinical and Patient Involvement Group aims to capture information from patients and families such as:

- Added value of the medicine to patients and caregivers
- Rarity and severity of the condition
- Whether the medicine addresses an unmet need
- Innovative nature of the medicine

Clinical evidence provides an approach that is centred on the needs of the patient and their family. A process which considers these additional criteria will increase the transparency and consistency of decisions for patients with rare diseases in gaining access to new orphan and ultra orphan medicines.

Conclusion: Genetic Alliance UK worked collaboratively with Welsh Government, patient organisations and the All Wales Therapeutics & Toxicology Committee (AWTTC) to support the development of an evaluation process which considers 'value' of a new medicine holistically. The new appraisal system aims to consult with patient and clinical experts on the value and impact of a medicine for the individual and caregivers.

Case Study: Ivacaftor (Kalydeco) for the treatment of Cystic Fibrosis

In May 2013, Kalydeco was rejected by the All Wales Medicines Strategy Group on the grounds of cost and clinical effectiveness. Following a review of the appraisal process as detailed, Kalydeco was appraised subsequently in November 2015. On this occasion, following a meeting of CAPIG and input from patient representatives and clinicians, the medicine was approved for use in Wales.

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