Access to newly licensed medicines

Shire

IPTRs can arise when approval from the Scottish Medicines Consortium (SMC) has not been forthcoming. We appreciate this is not the sole reason a clinician, in partnership with a patient, might raise an IPTR but we have specific experience of this particular situation and would like to offer our comments accordingly.

The clause on “exceptionality” within the IPTR process is a particular issue in rare diseases. The case study below illustrates this as in one case the physician and patient group pursuing an IPTR were asked to prove how one Hunter patient was exceptional compared to the only other 3 known patients in the country. The condition itself is exceptional and this is something we suggest is considered within any such process.

Assessment processes for new medicines for the treatment of rare diseases pose serious challenges – particularly where no previous treatment has been available

- The SMC process of assessment makes adjustments for various conditions associated with rare conditions, but in practice these are of limited effect.

- Where existing comparator products are available, the SMC process is generally more capable of producing an assessment based on comparative clinical and cost effectiveness – even where the comparator has not been reviewed by the SMC having been launched before it was set up.

- Where no comparator is available because the new treatment is the first available for that particular rare disease, the SMC process will generally produce a negative result on the basis of simple cost effectiveness even taking into account the “modifiers” introduced by SMC.

- The process applicable to medicines for rare conditions needs to be redesigned to take into consideration the special conditions applying to rare diseases – the most important of which is the rarity itself of the condition and the likely resulting high individual cost per patient.

- SMC has established a globally recognised process for assessment of new therapies, however in rare diseases with individually expensive therapies, given the likely outcome of such reviews, the industry now thinks long and hard before submitting new therapies for review by SMC. This may delay or even deny patients in Scotland with such
conditions from ever having the chance to access therapy unless an IPTR is brought forward.

We believe that there is a unique opportunity in Scotland to establish a dedicated process through which to assess and provide access to medicines for rare diseases in a planned way that will improve patient experience and outcomes whilst also managing NHS budgets effectively.

- In England the Advisory Group for National Specialised Services (AGNSS)\(^i\) has led the way in developing a specific new methodology and process for the assessment of future highly specialised treatments and interventions – a first in the world and subject to current intense interest globally.

- Shire was invited by AGNSS in 2011 to provide specialist input from an industry perspective in the process that developed the AGNSS decision-making framework. Representatives from the National Services Division (Dr Mike Winter) in Scotland were observers to the process of developing this methodology.

- In July 2012, the Department of Health announced that NICE would now take on the work of AGNSS and it will consult on its developing methodology in 2013\(^ii\)

- The design of a new system for Scotland will undoubtedly be different, but the AGNSS framework could provide a useful example of a holistic framework for appraising technologies of which cost-effectiveness is not the primary consideration, thus recognising the challenges with the HTA of orphan drugs

Case Study

In 2007 Shire launched Elaprase in the UK, a novel treatment for Hunter syndrome (Mucopolysaccharidosis II or MPS II), a debilitating and life limiting inherited condition caused by a genetic mutation characterised by accumulation of waste products within cells. This leads to multiple symptoms including poor growth & short stature, stiff joints, respiratory and cardiac problems and in some patients significant and progressive CNS dysfunction. Most patients do not live into adulthood. Hunter syndrome is a very rare condition affecting less than 5 patients across Scotland.

In 2007 we submitted an application for Elaprase to the SMC. This was duly rejected on the grounds of poor cost effectiveness. This was not wholly unexpected as SMC’s methodology made no allowance for the cost bring driven by the ultra rarity of the condition. No comparator existed or currently exists for Elaprase, therefore costs are purely additive and considerable for each patient, with no possibility of meeting existing QALY thresholds despite SMC modifiers.
Subsequently we understand there have been two IPTRs raised for patients to access Elaprase. Both have been successful. However, we also understand that the criteria for successful granting of an IPTR posed particular challenges for the applicants – namely, the need to prove exceptionality of the patient from the clinical trial. In a very rare condition such as Hunter syndrome where Scottish patient numbers are so small, each person is an exception.

Proving exceptionality within rarity poses hurdles that further disadvantage these patients. In these cases the success in gaining an IPTR required considerable angst, emotional and legal pressure from the patient groups and families coupled with dedicated and committed physicians who strongly believed this was the best option for their patients. It is our understanding that 5 years on, where they might otherwise have not been expected to live, both patients are doing well and have benefitted from the instigation of this therapy.

About us

As one of the world’s leading specialty biopharmaceutical companies, Shire is focussed on a single purpose: to enable people with life-altering conditions to lead better lives. We focus on developing treatments for rare diseases and symptomatic conditions treated by specialist physicians.

Through our Shire Human Genetic Therapies (HGT) business, we pursue opportunities on behalf of patients and families facing such rare diseases as Fabry disease, Hunter syndrome, Gaucher disease, hereditary angioedema, and metachromatic leukodystrophy - patients whose very lives often hinge on the discovery and delivery of extraordinary medicines. Through our Specialty Pharma business, we develop and distribute an innovative portfolio of treatments for patients with ADHD, and gastro-intestinal disorders such as ulcerative colitis and chronic constipation.

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14 September 2012

1 http://www.specialisedservices.nhs.uk/info/agnss
2 http://www.nice.org.uk/newsroom/news/NICEToAssessHighCostDrugsForRareConditions.jsp