EXTERNAL LUNDBECK POSITION PAPER 2013

HEALTH TECHNOLOGY ASSESSMENT (HTA) IN EUROPE

Background
For many years, new medicines in the developed world had to demonstrate quality, safety and efficacy before being authorized to enter the market. In recent years, many countries have also begun to use Health Technology Assessment (HTA) to appraise the cost effectiveness of new medicines before deciding whether they should be made available within their healthcare systems. While it is right that health services should seek value for money, it is also important that HTA follows certain principles to ensure that it does not simply serve as a cost containment tool and prevent patients from obtaining timely access to innovative medicines.

HTA systems are emerging and evolving, not only in Europe, but also in countries such as China and Japan. A current issue in the HTA debate in Europe is whether there should be a pan-European assessment of the relative clinical efficacy of new medicines, as an alternative to this being done at national level. While this potentially offers efficiency savings, the methodology would need to be very carefully constructed to ensure that it did not act as a barrier to innovation and was even-handed across different therapeutic classes.

Health Technology Assessments (HTAs) are used in most EU countries, and increasingly elsewhere, to evaluate the clinical and/or cost effectiveness of new medicines, and have a critical impact upon their access to the market.

The EFPIA Board recommended in June 2011 that industry should increase its focus on monitoring and exchanging information on country-level developments whilst working towards a gradual move to central assessments of the clinical aspects of HTA. Relative clinical efficacy assessments are increasingly used in EU Member States by policy makers seeking to identify the most valuable medicines. The European Medicines Agency’s (EMA) Road map to 2015 notes that together with EUnetHTA – the network of national HTA bodies across the EU – it is currently considering how the European Public Assessment Report (EPAR) could best be used in the assessment of relative clinical efficacy by HTA bodies in the EU Member States. EPARs are published by EMA for every medicine authorized through the centralized procedure in the European Union. They reflect the scientific conclusions reached by EMA’s Committee for Medicinal Products for Human Use (CHMP) at the end of the evaluation process, and include information on the assessment of the risks and the benefits of a medicine. EUnetHTA will be piloting joint European HTA assessments in 2012, on a voluntary basis and with the involvement of manufacturers.

Lundbeck position
Lundbeck recognizes the responsibility of governments to seek value for money from their healthcare budgets and accepts that HTA has a legitimate role to play. However it is very important that HTA systems are used to identify medicines that offer value and to accelerate patient access to them, rather than acting as a barrier to new medicines. Lundbeck supports the key principles for HTA set out by the European Federation of Pharmaceutical Industries and Associations (EFPIA). These include the following points:

- The criteria against which the value of a medicine is measured should be clear and broad enough to include any innovation of value to sub-groups of patients
- HTA methodology and data requirements should be transparent and consistent
- HTA should be based on early and inclusive dialogue with patients, healthcare professionals and industry
- Since information about a new medicine is inevitably limited at launch, companies must be able to submit data based on real-life clinical use throughout a product’s life
- Where HTA gives a positive evaluation of a medicine, payers should ensure that funding is provided to facilitate patient access to it
- HTA should not only be applied to innovative medicines, but to all healthcare interventions
- While international discussions of HTA methodology are desirable, the assessment of individual products should be done at national level to reflect local circumstances
- HTA should remain separate from regulatory review carried out for the grant of a marketing authorization
- Evaluations should take into account the indirect benefits of a new therapy, such as productivity gains and reductions in the cost of care
- HTA bodies should have an equitable process for handling appeals by stakeholders.
Lundbeck also believes that companies should face no more than one appraisal per Member State; additional appraisals should not be imposed at regional level. HTA methodologies need to take account of the fact that clear identification of patient subpopulations is considerably more complex for many brain diseases including the most prevalent one, depression, than for a disease like cancer. This makes it particularly difficult to demonstrate value simply from clinical trials. Also there is huge inter-patient variability which again increases variability in clinical studies and may mask positive responses. It is also very important that the measurement of societal costs, which can be very significant in the case of brain disorders, is improved and that these costs are fully factored into HTA evaluations.

Lundbeck agrees that moving towards the use of a centralized EU assessment of relative clinical efficacy could offer worthwhile efficiency gains for companies and governments, and supports the preparatory work that is currently taking place. However a centralized process which took an unduly restrictive view of what constituted therapeutic progress could have serious implications both for patients and for research-based pharmaceutical companies across the EU. Even a satisfactory centralized system would be pointless if its findings could be overturned at national level. One possible way forward would be for relative clinical efficacy appraisals to copy the EU regulatory approval process in offering a choice of a centralized procedure or mutual recognition of assessments made at national level. It is important that flexibility is maintained and that a ‘one size fits all’ approach is avoided.

As we specialize in brain disorders, it is important to us that HTA systems take full account of improvements in the patient’s quality of life, and of societal costs which fall outside the healthcare service, such as sickness absence. HTA systems, such as that operated by NICE, which measure the cost of medicines per Quality Adjusted Life Year (QALY), are unfavourable to those developing medicine against chronic, disabling diseases, since treatments for brain disorders typically relieve symptoms of disease, rather than providing a cure, and QALY’s favour products which prolong life. The lack of biomarkers for brain disorders also makes it more difficult to demonstrate the value offered by our products.

Safeguards also need to be put in place to ensure that the involvement of EMA in any centralized EU assessment of clinical efficacy does not compromise the existing separation between the marketing authorization process, with its clear criteria of quality, safety and efficacy, and the evolving HTA process.