2nd PID FORUM on Health Technology Assessment
Hosted by Glenis Willmott
European Parliament, 6 December 2011

Recommendations

1. The cost-effectiveness assessments of rare diseases therapies must factor in the fact that these are life-saving and their impact on patients quality of life.

2. The macro-economic and societal benefits of diagnosing patients with a chronic and life threatening rare disease early and treating them with the appropriate therapy are significant. Such factors must be taken into account while performing HTA appraisals, particularly in the case of rare diseases therapies that are well established.

3. No choice concerning the life-saving treatment rare diseases patients need should be made without consulting them or in a non-transparent fashion. HTA can and should take patients' views into account.

4. In addition of its policy goals, HTA techniques must have a macro-economic approach and provide clear information on the impact of decisions on patients' quality of life.

5. Economic arguments should not be used to limit access to well-established life-saving medicinal products- especially when these can prevent unnecessary expenses due to repeated disease-related hospitalisations or days-off work.

6. Physicians should be protected from any sort of pressure, including economic considerations aiming at limiting access to well established life-saving rare diseases therapies.

7. The European Union must continue to support patients suffering from rare diseases and give them the means to participate in HTA appraisals.

8. Member States have the full responsibility for the decisions made concerning access to treatment and the impact these have on patients' lives, even after an HTA appraisal.

9. When assessing rare diseases therapies, future HTA networks and Member States should promote the collection of macro-economic data at European level as to better assess the impact of early diagnosis and treatment - and factor in their impact on patients' quality of life and society as a whole.

10. The European Commission and Member States should make sure that the spirit of the Cross-border Healthcare Directive is respected by ensuring appropriate consultation of stakeholders and therefore patients in HTA processes.