



## 17<sup>th</sup> Workshop

### EURORDIS Round Table of Companies (ERTC)

#### *The Value of Orphan Drugs: Efficient for the Patients, Useful to Society?*

September 28, 2012 – 9:00 to 17:30 - Barcelona, Spain

#### CONCEPT PAPER

European health systems are under pressure and their financing has been put into question. This is due to the evolving demography, the healthcare offer and organisation restructuring, introduction of new and most often expensive technologies and an increase in the demand for quality health care.

At the 5<sup>th</sup> European Conference on Rare Diseases (ECRD) in Krakow, 2010, Dr Claudia Wild of the Ludwig Boltzmann Institute of Health Technology Assessment, Vienna, commented on the evolution from the discovery of insulin in the 1920's that cost around € 500 per patient per year and could prolong life for decades, to enzyme replacement therapy marketed in 2010 that cost € 500 000 per patient per year.

Overall the pharmaceutical industry is also confronted with less innovation, expiring patents or loss of exclusivity, and reallocation of marketing resources.

Some health care authorities react, for example, by proposing conditional reimbursement. When a product is newly authorised, additional studies are requested to provide more data on its utility once it is on the market (i.e. phase IV studies or creation of registries with all treated patients). The product is provided, and reimbursed, to patients under the condition that further evidence is to be generated.

To follow up the conditional reimbursement decisions, a database is being developed within work package 7 of EUnetHTA. This database, called "Eiffel tool", will collect data from Member States where such conditions are decided, and all Member States can exchange information in this respect.

It is expected that a large European cooperation on assessment of orphan drugs will be possible in the near future, based on horizon scanning and early assessment of the utility of a new orphan drug, and via CED (Coverage under Evidence Generation, or conditional reimbursement).

Other authorities react by requesting cost-effectiveness studies for orphan drugs where limited information is available at the time of marketing authorisation or some years later. Performing these studies in small



populations is difficult while a knowledge base is lacking on the real health cost as well as the social and financial burdens on the family. Furthermore, to assess the outcomes of these studies health authorities most often use tools that are not necessarily adapted to rare diseases; for example how appropriate is the QALY for the assessment of the benefits in small populations of patients with severe disabilities?

By examining all the steps involved in the full assessment of a medicinal product: beginning with benefit/risk (current marketing authorisation), to how the drug performs compared to existing products (Relative Efficacy, Clinical Added Value) or in the broader context of care (Effectiveness and Relative Effectiveness), to examining cost aspects (Full HTA report, Cost Effectiveness Analysis, Budget Impact Analysis), and finally the appraisal (societal preferences: for example some Member States place a high value on people living with rare diseases and orphan drugs, other less so), one realises the extent of the complexity of the task.

Benefit-risk evaluation is now harmonised. Relative efficacy could also be harmonised across Europe to a very large extent. Clinical Added Value has now been adopted by the EUCERD as the Clinical Added Value of Orphan Medicinal Product-Information Flow potentially establishing a process of exchange of information between the EU Centralised Procedure and the National Competent Authorities for health assessment. Full HTA and even more so, societal preferences, will be far more difficult to harmonise because the health care and medicines cost structures are so different across Europe.

The first part of the morning session of the 17<sup>th</sup> EURORDIS Round Table of Companies (ERTC) Workshop on *“The value of Orphan Drugs”*, will be dedicated to patients’ organisations who will describe how they collaborate to increase knowledge and to assess medicines from a different perspective to that of benefit/risk (i.e. how to demonstrate the utility of the medicines for society, and what requirements are necessary for their reimbursement).

The second part of the morning will demonstrate the efforts of the HTA initiatives to harmonise methods used to assess the utility of orphan medicines for society, beyond the evaluation of the risk/benefit ratio. We will close the morning session with an academic researcher who will present ideas on how to reform the way QALYs are used to better take into account the public preferences.

In the afternoon, we will listen to the industry perspective beginning with a method to demonstrate the value of orphan drugs from the Office of Health Economics followed by the perspective of various industry representatives on the proposed initiatives and how to address the challenges. The AGNS method will be presented as well as the positioning of orphan drugs in healthcare systems. This session will end with a presentation on a specific case and a panel discussion.



The workshop will conclude with a session on the innovative approaches being considered by the European Medicines Agency. The concept of progressive approval will be described and its application to speeding up approval and access of medicinal products. The application of progressive approval to orphan drugs will be discussed.