

## OrphaNews Europe: 23 September 2005

**Interview with Yann Le Cam, member of the EMEA's Committee for Orphan Medicinal Products (COMP) and Chief Executive Officer of EURORDIS (European Organisation for Rare Diseases)**

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**What has been the impact of the patient groups involvement in the decision-making of the COMP?**

**YLC:** The COMP was pioneering in that patients were involved for the first time in a decision-making committee at European level. Their role has become progressively more important over time by providing a special focus on the public health aspects of the committee's work. For the first time, candidate drugs were looked at for their potential use in day-to-day life and in a therapeutic strategy and not only from a regulatory and scientific point of view.

One of the key achievements has been that increased participation by patients has enhanced the transparency of the COMP and the EMEA. More information has become available, such as the public summary of opinions for designated drugs, and an on-going dialogue has been established through a series of workshops. Patient participation has also contributed to the recognition of the specific expertise that patient groups can bring to scientific and regulatory debate. Over the five years, 45 different patient representatives have participated in the COMP's activities.

Having patient representatives at the EMEA has also helped to structure the rare diseases patient movement in Europe and to promote the dialogue with all interested parties, particularly industry and national authorities.

**Orphanews Europe:** Is there still more that can be done to increase transparency?

**YLC:** The development of each orphan drug needs to be followed and this information made available to patients and professionals. We would like to see more information made available from the studies that take place after orphan drug designation up until the marketing authorisation, including the final results of studies, either positive or negative. This lack of transparency is found in clinical research generally but its impact is greater in rare diseases, where unmet medical needs are high and resources are very limited. There is a risk, however, of ending up with too much information and not being able to extract what is useful.

**OrphaNews Europe :** What effect has five years of orphan drug regulation in Europe had on the availability of orphan drugs to patients and how can availability be improved?

**YLC :** After five years of implementation of the regulation the delay between the marketing authorisation and the real availability of orphan drugs is still too long – certainly longer than the 180 day legal limit. Several studies, conducted annually by Eurordis, and by [Alcimed](#) in 2004, for example, demonstrate that some medical products approved in the EU are not available in all member states and in some cases the delays in availability exceed 2 years.

One option is for the European Commission to make member states more aware of the need to take action on a national basis. Going further than this we can also imagine creating a European group of volunteer member states who would co-ordinate their reviews for therapeutic assessment value and agree on a European catalogue price with the drug's sponsor which could be used as a reference document by all member states. This would not require a new EU regulation nor a transfer of national competence; it would not be compulsory but would provide a reference point.

Price is a crucial issue for sponsors. Market exclusivity is important but they also need to know what price they can get. One of the pitfalls of the orphan drug regulation is that it only applies up to the point of market authorisation – price is the remit of the member states.

**OrphaNews Europe:** How can member states be encouraged to put in place national incentives for orphan drug development?

**YLC:** The COMP considers that the regulation is a good one and that its implementation has been successful. However, looking to the future, the issue is how to promote national policies with adequate incentives to stimulate rare disease research and clinical development and to promote better co-ordination on national policies in research, industry and health in collaboration with stakeholders. The experience gained in member states with the most comprehensive policies, such as France and the Netherlands, or with active policy in one of the areas mentioned, such as the UK, Germany, Italy, Denmark and Sweden, should be shared and lessons learnt.

The European Commission has a role in encouraging member states to do more – it can't go any further. The most effective way is to demonstrate existing best practice and encourage emulation. Member states need to recognise the benefits that come from having incentives in place in terms of competitiveness and economic development, as well as unmet public health needs.

**OrphaNews Europe:** How can international collaboration in the field of orphan drug development be reinforced?

**YLC:** Since the beginning there has been on-going collaboration between the COMP/EMA and the FDA's Office of Orphan Products Development to discuss key issues in orphan drug development, such as assessment of prevalence and methodology in clinical trials for small populations. That said, it is now time to envisage a more formal and closer co-operation in order to facilitate parallel applications for orphan drug designation in the US, Europe and maybe Japan. It will also be important to reinforce collaboration on protocol assistance by proactively proposing to sponsors a joint EMA/FDA review to ensure converging, consistent opinions and to save limited resources in terms of patients, funding and time, to the benefit of all, and primarily of patients.