From: gea.spierings@orfagen.com on behalf of
patrick.dupuy@pierre-fabre.com
Sent: mardi 15 janvier 2008 16:53
To: SANCO RAREDISEASES CONSULTATION
Subject: Consultation Publique

PUBLIC CONSULTATION

Orfagen is a small pharmaceutical laboratory, based in Toulouse, France. It is dedicated to the development of orphan drugs, including biotechnological products, in the field of dermatology. The company has extensive research and development activities both in Europe and the USA (investment in R&D of about 1.5 millions Euros/year).

We would like to take the opportunity of the public consultation on rare diseases, organised by the Health and Consumer Protection Directorate General, to express some recommendations that appear to be important to us.

Question 4.
We totally agree with the implementation of reference centers for rare diseases.

Question 5.
We support the extension of electronic tools such as oOrphanet, as valuable source of information both for professionals and patients.

Question 8.
We strongly advocate for a common pricing policy within the European Union. We also recognize that a more transparent argumentation form the pharmaceutical industry is to be settled. For this purpose, we propose that the global sales turn-over within the European Community can be the basis of the discussion between regulators and the pharmaceutical companies; along with the ex-factory price of the products, this financial marker will allow the regulators to appreciate the real benefit margin European-wide. We consider that this will be a valuable basis for determining the price of orphan products.

Question 10.
As a counterpart, we call for accessible public funds at the European level, in the field of therapeutic research in rare diseases. The framework programmes are not adapted to the research that needs be done in this field. Indeed, large cooperation between research bodies in at least 3 countries is not appropriate because competent centers in a given rare disease are often too few and not disseminated throughout the European territory. Furthermore, application forms of the framework programmes are too bureaucratic and too time-consuming for small enterprises or even public institutions.
There is an excellent model, which is the orphan product development grant programme (OPD), directed by the FDA (USA) in association with the NIH. As a European-based small company (8 persons ; no sales turn-over, yet), we could benefit from this OPD, whereas our two applications in the FP6 (one STREP, one MARIE-CURIE) were unsuccessful.

We propose to simplify the application process, with a specific grant programme for orphan products. Revision of the application by the European Commission may be based on the protocols only, associated with some practical information on the logistics and financial aspects of the

research. Such application may be only reserved to clinical studies, involving the cooperation between the private sector (e.g. pharmaceutical laboratories) and the public sector (universities, research, institutions), irrespective of their territories.

Question 11.
We support the setting-up of patient registries. This will be a valuable tool to enhance research on rare disorders (epidemiology, clinics, therapeutics, genetics, etc.).

Hoping that these comments will be taken into consideration, we remain,

Yours sincerely,

P. DUPUY, MD
President Orfagen

ORFAGEN
Parc Technologique du Canal
4 Rue Marie Curie
BP 22132
31521 Ramonville St Agne - FRANCE
Tel : 33 (0)5 62 24 76 78 / Fax : 33 (0)5 62 24 76 86
Portable : 06 25 31 26 47

Email : patrick.dupuy@orfagen.com