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Press release

Thalidomide: patients denounce the withdrawal of the marketing authorisation application for multiple myeloma treatment.

Multiple myeloma patients' representatives opposed to Pharmion Corporation's decision. EMEA/patients dialogue still inadequate.

Paris, June 8th 2004: Despite its dramatic history, thalidomide should now be evaluated exactly on the same basis as other medicinal products. Facts from 1957-1962 should not obliterate scientific data and medical needs in 2004.

- Patients with multiple myeloma and their organisations strongly denounce the absence of transparency in Pharmion Corporation's decision of May 19th 2004 to withdraw their marketing authorisation application for thalidomide to treat refractory multiple myeloma. A new marketing authorisation application could be submitted but not before 2005/2006.
- The availability of the product in the coming months and years is no longer guaranteed for European patients. Pharmion, by acquiring Laphal in 2003, has created a distribution monopole for thalidomide in Europe. EU based manufacturers have stopped producing thalidomide. All other European pharmaceutical companies that submitted a similar application have since withdrawn it.
- A well-regulated marketing authorisation could have clarified the dispensation of thalidomide, this withdrawal confines thalidomide to a regulatory no man's land, creating de facto a situation wherein the pharmaceutical company can continue distributing the product on its own conditions. These conditions are clearly rejected by patients, health care professionals and many national authorities. Patients are dissatisfied by the absence of adequate discussion with the European Medicinal Evaluation Agency (EMA) and criticise the absence of transparency in this decision, as no official explanation is available from EMA.
- This process has a negative impact on patients as it creates scientific uncertainties on thalidomide use, confuses medical practices, and opens the door to parallel trade and grey market. "To me this is simply blackmail, I don't have the choice, or I continue taking thalidomide under Pharmion's conditions or I stop it. There is no consideration for patients, only for financial strategy", says Ilse Hein, patient representative from Austria.

**For immediate release
June 8th 2004 4.00 pm**

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Patients refuse to meet Pharmion's unreasonable demands.

There is no provision for patients' organisations to appeal to the company or to the European Commission in case of such an inopportune decision. This loophole in regulatory procedure would have no consequences if the right level of dialogue existed between all parties on all subjects.

Pharmion declares itself committed to the continuation of the compassionate access programme to deliver thalidomide. But to Pharmion, compassion is conditional to the acceptance of its risk management programme says Judith Hemberger, Pharmion's executive vice president and chief operating officer. Only countries that will implement Pharmion's programme will continue to receive the product.

However, from January 2003 to March 2004, a consultation process between the EMEA, multiple myeloma patients' organisations, and thalidomide victims, clearly rejected the terms and conditions of the proposed risk management programme. Alternative proposals were made, supported by the medical community. A communiqué* reflecting the common position of 12 organisations out of 17 was sent to the Committee for Proprietary Medicinal Products (CPMP) at EMEA.

This lack of transparency in the evaluation of the risk/benefits ratio does not serve public health policy.

EMEA/patients dialogue still inadequate

Multiple myeloma patients regret the absence of direct dialogue with CPMP experts who consistently refused to address their concerns on thalidomide efficacy with patients, and the consequent need for additional studies.

The CPMP has requested new studies from Pharmion, however these studies can not be satisfactory for the following reasons:

1. To compare two different doses in 400 treatment refractory patients is not likely to be conclusive. A much large population would be needed to reach statistical power.
2. Products officially designated as orphan medicinal products are, by definition, products for rare diseases, and clinical trials can not enrol large populations. The requested studies are not taking into account the specificities of rare diseases.
3. With the adoption of the new European Directive 2004/27/EC on medicinal products for human use, published in the Official Journal on 29 April 2004, a conditional marketing authorisation under exceptional circumstances can now be granted. A temporary marketing authorisation could have been granted, provided that additional and confirmatory results would be obtained for a full approval by 2005/2006. As an alternative to an outright withdrawal, this option was unfortunately not considered, indicating inadequate discussion between CPMP and Pharmion.

Multiple myeloma

Multiple myeloma accounts for approximately 1% of all cancers, and 10% of haematological cancers. Recent statistics indicate increasing incidence. The European Cancer Registries estimate that approximately 21 420 new cases of myeloma are diagnosed each year in Europe and just under 15 000 deaths. Only 30% of multiple myeloma patients survive longer than five years.

In 1965, the first report of a slowing of multiple myeloma progression in a patient treated with thalidomide was published.

Multiple myeloma is incurable with conventional chemotherapy. High-dose chemotherapy with auto-transplant increases the rate of complete remission and extends event-free and overall survival after failure of initial first-line treatment.

Thalidomide is the first product for multiple myeloma with a relatively high response rate and which stabilises the disease in many patients. It is successfully used as monotherapy and/or in combination with other drugs for first-line, maintenance and refractory therapy. Thalidomide has proved to be one of the most efficient drugs in multiple myeloma treatment and will continue to be very useful for many patients whatever the stage of the disease.

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*: communiqué available upon request to Eurordis