

## **Dutch Steering Committee on Orphan Drugs: A five-year perspective**

The Dutch Minister of Health, Welfare and Sport (VWS) installed the Dutch Steering Committee on Orphan Drugs in 2001 for at least four years, in accordance with Article 9 of the European Regulation No. 141/2000 on Orphan Medicinal Products, which states that individual member states of the EU must implement measures to encourage the development of orphan medicinal products.

The construction of a national structure such as the steering committee was one of the suggestions made in 1998 by the Dutch Advisory Council on Health Research (RGO). The VWS Minister asked this council for advice on the coordination, priority and stimulation of research in The Netherlands in respect to orphan drugs.

Based on a positive evaluation of the steering committee activities, the Minister decided at the end of 2004 that the committee could continue for at least three more years (2005-2007).



### **Composition**

The Steering Committee on Orphan Drugs is a multidisciplinary group consisting of several rare disease and orphan drug stakeholders. The committee currently has eleven members and two observers. The members include representatives of umbrella organisations for patients and pharmaceutical companies, physicians, a hospital pharmacist, scientists, and representatives of the Dutch medicine evaluation board, the Dutch health care insurance board and health insurers. The observers are representatives of the Ministry of Health (and also the current Dutch COMP/EMA member from 2000-2006) and the Dutch medicine evaluation board (the new Dutch member of the COMP from May 2006 onwards). The chairperson of the steering committee is Professor Bert Leufkens of the University of Utrecht. The committee meets six to eight times per year.

The steering committee is an independent organisation. The secretariat consists of two scientific officers and a secretary and is situated at the Netherlands Organisation for Health Research and Development (ZonMw) in The Hague. The Ministry of Health has made available an annual budget of €450,000 for the secretariat, travel and functioning costs.

## Philosophy

The mission of the steering committee is to encourage the development of orphan drugs and improve the situation of rare disease patients, especially by strengthening the information on rare diseases. The philosophy of the committee is that the different stakeholders should meet and share their perspectives on issues related to rare diseases and orphan drugs, collaborating wherever possible. The members much appreciate the multidisciplinary character of the steering committee. The steering committee implements the networking of stakeholders (scientists, industry, patients, clinicians, regulators) in many of its activities, e.g. by choosing the subjects and inviting participants for working groups and meetings.

## Activities

The steering committee organised an invitational conference with fifty dedicated participants in November 2001 to discuss the main problems concerning development of orphan drugs and care for rare disease patients. Subsequently, the committee finalised its plans and introduced them at a symposium in January 2002. The activities of the steering committee are summarised in four strategies:

### *Information desk*

The committee collects and disseminates information on rare diseases and orphan drugs in the Netherlands. The dissemination takes place via a website [www.orphandrugs.nl](http://www.orphandrugs.nl), brochures, articles in magazines for different stakeholders, lectures and presentations, and telephone and e-mail services that respond to questions. The steering committee functions as a contact point for EU-bodies on orphan drugs.

### *Booster*

The steering committee has almost finished an inventory of national basic, translational and clinical research. This inventory will be used for the development of a Dutch research programme on rare diseases and orphan drugs. The steering committee encourages the industrial activities on orphan drugs and has established the position of a so-called orphan product developer, liaising between Dutch academic, industry and regulatory environments.

The steering committee participates in several European initiatives, such as the European Rare Diseases Therapeutic Initiative (ERDITI) (see [OrphaNews Europe, December 2005](#)) and the European ERA-NET CA project E-Rare. The committee collaborates with the ERA-Net project *Priority Medicines for Children* and has written a background chapter on orphan diseases for the WHO report *Priority Medicines (2004)*.

### *Architect*

The committee facilitates and stimulates patient group activities that have an impact on rare diseases in general, by funding projects, for example. Furthermore, working groups have been set up for issues such as rare disease registries (resulting in a 2004 report) and the availability of registered and non-registered drugs for patients with a rare disease (resulting in a brochure in the second half of 2006). The steering committee monitors the availability of orphan drugs and reimbursement in the Netherlands.

### *Brainpower/think-tank*

The steering committee constantly fuels societal dialogue through public debates (Orphan Cafés), invited debates (Orphan Topics), seeding projects, et cetera, with subjects such as '*Orphan drug development by small enterprises*', '*Reimbursement of and access to orphan drugs*' and '*Pricing of orphan drugs*'.

The committee gives advice upon request or on its own initiative to the Minister of VWS and other authorities. The steering committee expressed sharp concern over plans to finance orphan drug

therapies in academic hospitals in collaboration with other stakeholders. As a consequence of this, a new policy rule has been issued to finance specific orphan drugs.

## **Evaluation**

The activities of the steering committee were externally evaluated in 2004. It was concluded that, *“The overall impression that the steering committee makes, its activities during the past years and its effects on relevant target groups are good. The steering committee has, within its remit and resources, raised the quality and quantity of available information on orphan medicines and rare disorders, not only for patients, but for other relevant stakeholders as well. Undoubtedly, the steering committee has succeeded in putting rare disorders and its problems higher on the agendas of relevant parties. From an international viewpoint, the steering committee has fulfilled its remit and is well known among relevant organizations”*.

## **Other steering committees on orphan drugs?**

Due to its positive experiences, the Dutch steering committee likes to encourage and support initiatives of stakeholders in other European member states to establish similar national structures. The steering committee would very much appreciate ‘sister’ organisations with whom to discuss international issues on rare diseases and orphan drugs.

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