



FOURTH WORKSHOP ON PARTNERING FOR RARE DISEASE THERAPY DEVELOPMENT

**Orphan Therapies : from Clinical Development to Equitable Access**

The Hague, 13-14 November 2003

Under the auspices of the Dutch Steering Committee Orphan Drugs,  
established in 2001 by the Minister of Health, Welfare and Sport

## PROGRAMME

Venue: "Sociëteit De Witte"  
Plein 24 - P.O. Box 11589  
2502 AN The Hague  
The Netherlands

*Thursday 13 November 2003*

11:30-12:30 **Registration**  
**Light Lunch**

**Workshop Chairman:** Bert Leufkens  
*Chair Dutch Steering Committee Orphan Drugs, The Netherlands*

12:30 **Introductory remarks**  
*Ysbrand Poortman, Chairman, EPPOSI*

12:45 **Welcome by Minister H. Hoogervorst, Dutch Ministry of Health, Welfare and Sport**

13:00 **SESSION 1 - ORPHAN THERAPIES: WHERE ARE WE WITH POLICIES IN EUROPE ?**

Chaired by: Alastair Kent  
*Director, Genetic Interests Group (GIG);  
Patient representative member, Committee for Orphan Medicinal Products COMP/EMEA*

13:10 **The EU contribution to the development of orphan therapies ?**  
*Jules Maaten, Member of the European Parliament, Group of the European Liberal,  
Democrat and Reform Party*

13:30 **The EU Orphan Drug Regulation: how successful has it been in stimulating R&D in rare diseases?**  
*Josep Torrent y Farnell, Chairman, Committee for Orphan Medicinal Products, COMP/EMEA*

13:50 **Orphan therapies & public health priorities in the EU**  
*Fernand Sauer, Director of DG Health and Consumer Protection, European Commission*

14:10 **Overview of political developments in rare diseases**  
*Yann Le Cam, Chief Executive Officer, EURORDIS; Vice Chairman, COMP/EMEA*

- 14:30 **Interactive panel and audience discussion**  
- Ysbrand Poortman, *Chairman EPPOSI*  
- Erik Tambuyzer, *Chairman Healthcare Board EuropaBio, Sr Vice-President Corporate Affairs, Genzyme Europe*  
- Ségolène Aymé, *President Orphanet, Director of Research INSERM*

15:10 Coffee break

15:40 **SESSION 2 – EQUITY IN ACCESS: TOWARDS A PAN-EUROPEAN POLICY ?**

Chaired by: Harrie Seeverens  
*Ministry of Health, Welfare and Sport, Member COMP/EMEA*

15:50 **Methodology issues: how to assess the value of orphan drugs**  
Rod Taylor, *Department of Public Health & Epidemiology, University of Birmingham*

16:10 **Compassionate use: a way to improve patient access?**  
François Meyer, *Agence Française de Sécurité Sanitaire des Produits de Santé (AFSSAPS), Member COMP/ EMEA*

16:30 **The hurdles to patient access: the case of orphan drugs**  
Andrea Rappagliosi, *Vice President, Corporate Health Policy & Government Relations, Serono International*

16:50 **Patient access : the clinical point of view**  
Hans-Georg Eichler, *Department of Clinical Pharmacology, University of Vienna, former member COMP/EMEA*

17:10 **Patient access: the individual vs Society**  
Hélène Tack, *EURORDIS*

17:30 **Interactive panel and audience disussion**  
- Hartwig Gajek, *Medical Director Europe, Baxter Biopharmaceuticals*  
- Torben Grønnebaek, *Board member, EURORDIS*  
- Hans Büller, *Head of Department Pediatric Gastroenterology and Nutrition, ErasmusMC Rotterdam*

18:15 **Closing remarks of Day 1**  
Bert Leufkens, *Workshop Chairman*

20.00 **Dinner**  
*Offered by the Dutch Steering Committee on Orphan Drugs*

Friday 14 November 2003

9:00 **SESSION 3- CLINICAL DEVELOPMENT : PRE & POST MARKETING METHODOLOGY  
ISSUES RELATED TO RARE DISEASE THERAPY**

Chaired by: Daniel Brasseur  
*Chairman, Committee for Proprietary Medicinal Products CPMP/EMEA*

9:10 **Partnering value: participation of patient groups in clinical trials**  
*Peter Streng, Director, Muscular Dystrophy Association, The Netherlands*

9:30 **Orphan Therapies and EU Clinical Trials Directive**  
*Francis Crawley, Secretary General, European Forum for Good Clinical Practice*

9:50 **Methodology issues : a sponsor's perspective**  
*Carlo Incerti, Co-Chairman European Management Board, CEO Scientific Development, Senior Vice President Biomedical & Regulatory Affairs, Genzyme Europe*

10:10 **Methodology issues : continuity in data gathering before and after market approval.  
A regulatory perspective.**  
*Barbara van Zwieten-Boot, Clinical Guidelines Committee CPMP/EMEA*

10:30 **The new Protocol Assistance & Scientific Advice procedure:  
opportunity or hurdle for clinical development?**  
*Agnès Saint-Raymond, Head of Sector, EMEA*

10.50 Coffee Break

11.20 **The U.S. experience: how to overcome methodology issues**  
*Marlene E. Haffner, Director, Office of Orphan Products Development, FDA, USA*

11.45 **Interactive panel and audience discussion**  
- *Catarina Edfjäll, Director, Global Regulatory Liaison, Actelion Pharmaceuticals*  
- *Mary Baker, President, European Parkinson's Disease Association & President, European Federation of Neurological Associations, UK*  
- *Carla Hollak, Department of Internal Medicine, Academic Medical Center of Amsterdam*

12:30 **Conclusions** and take-home messages  
*Bert Leufkens, Workshop Chairman*

13.00 Workshop ends