9th EPPOSI Workshop on Partnering for Rare Disease Therapy Development
Sharing strategies and tools for access to diagnosis and treatment
Assemblée Nationale, Paris, France
16-17 October 2008

An official event of
The French Presidency of the EU Council (TBC)

Under the High Patronage of
The Co-Chairs of the French Parliament’s Working Group on Rare Diseases
Dr. Marc Laffineur
Dr. Dominique Orliac

With the support of
The French Ministry for Health, Youth and Sports
The French Ministry for Culture and Communication

PROGRAMME

Organising Committee co-chaired by:

Yann Le Cam
EURORDIS representing Patients

Kerstin Westermark
COMP Chairperson representing Science

Wills Hughes-Wilson
Genzyme Europe representing Industry

Thursday 16 October 2008

8.30 - Registration opens

NATIONAL SESSION

9.00 - Opening
Dominique Orliac, Member of the French National Assembly, French Parliament’s Working Group on Rare Disorders, France

Objectives and topics to be finalized in collaboration with the Alliance Maladies Rares and the LEEM

12.30 – Lunch (TBC)

OPENING

13.30 - Opening: Setting the scene
Marc Laffineur, Member of the French National Assembly, French Parliament’s Working Group on Rare Disorders, France

13.50- Welcome note
Alastair Kent, Chairman of EPPOSI

14.00-Case study of a crisis: Hunter’s syndrome in Sweden – steering lessons one year later?
Veronica Hübnette, Swedish Mucopolysaccharidosis Society, Sweden (TBC)

14.10- Case study of a public policy: is France the benchmark for Europe on access to orphan therapies?
François Meyer, HAS, France
SESSION 1.1

14.30 - Improving early diagnosis and access to care: Is population screening an avenue to explore when a treatment exists?

In rare diseases, the success of treatment often depends on early diagnosis. The earlier the diagnosis, the more likely a successful treatment outcome. But how can we best achieve that early diagnosis? When and how should early diagnosis targeted programmes be developed? Should population screening programmes be used and, if so, how and when? This session will aim to explore the range of different approaches currently in place or in development in different European countries to facilitate early diagnosis with the objective of identifying measures to improve the outcomes for patients.

Chair: Ségolène Aymé, INSERM, Orphanet, ESHG, France
Speaker: Martina Cornell, VU University Medical Center, The Netherlands
Rapporteur: (TBI)

14.50-Panel debate
Adrian Quartel, BioMarin Europe, United Kingdom
Representative of a Patient Group for Sickle Cell Anaemia (TBI)
Olaf A. Bodamer, Austrian Neonatal Screening Programme, Division of Biochemical and Pediatric Genetics, University Children's Hospital Vienna, Austria

15.30- Discussion with the audience: Questions and concrete recommendations
➡ When and how should we assess screening strategies when a treatment exists? Which measures to improve the orientation of patients through the healthcare services? Which measures to improve early diagnosis in national plan? When and how to pool expertise and experience at the European level?

16.30 - Coffee break

SESSION 1.2

16.50 - Improving early diagnosis and access to care: How to optimise access to appropriate care?

It is not just rare disease patients who are rare. So are expertise and experts. Such expertise is scattered across Europe and successful diagnosis and treatment too often depends on a lucky accident of geography. How can we build access to the best expertise in diagnosis and treatment via Centres of Expertise and European Reference Networks for rare diseases? And how can such Centres and Networks facilitate access to the best treatment?

Chair: Alexandra Fourcade, French Ministry for Health, Youth and Sports, France
Speaker: Edmund Jessop, National Health Service, United Kingdom
Rapporteur: (TBI)

17.10 – Panel discussion
Alain Fischer, Hôpital Necker – Enfants Malades, Department of Immunology, Haematology and Rheumatology, France
Thomas Wagner, J.W.Goethe Universität, European Centres of Reference Network for Cystic Fibrosis, Germany
Christel Nourrissier, Prader-Willi France, EURORDIS, France
Dennis Jackman, CSL Behring, U.S.A.

17.50 - Discussion with the audience: Questions and concrete recommendations
➡ How to develop standard of diagnosis and care at national level? How to collaborate at European level to compare and upgrade standard of diagnosis and care? Which measures to optimize pre-marketing and post-marketing studies on orphan drugs through Centres of Expertise and European Reference Networks for rare diseases?

18.50 - End

20.30 - Networking Dinner
Welcome note: Alastair Kent, Chairman of EPPOSI, United Kingdom
SESSION 2.1

9.00 – Improving Access to Orphan Drugs: How to assess clinical added value of orphan medicinal products?

Orphan drugs, by their very nature, are intended to treat only a small number of patients. This means that gathering clinical outcomes is only possible based on a small number of patients. When healthcare systems are used to dealing with dossiers based on thousands of patients, how can we tailor them to also meet the needs of dossiers based on just tens of patients? Bundling the fragmented know-how between different EU-level committees and the Member states would allow timely production of shared information reports. This session aims to identify methods for building information on clinical added value of orphan medicinal products.

Chair: Andrea Rappagliosi, Merck-Serono, joint task force EBE-Europabio, Switzerland
Speaker: Kerstin Westermark, COMP-EMEA, Sweden
Rapporteur: (TBI)

9.20 - Panel debate

Finn Børlum Kristensen, DACEHTA, EUnetHTA, Denmark
Francois Meyer, HAS, France
Birthe Holm, Rare Disorders Denmark, EURORDIS, COMP-EMEA, Denmark
Patrick Le Courtois, EMEA, France
Jens Grueger, Novartis, Switzerland

10.00 - Discussion with the audience: Questions and concrete recommendations

When, where and how to implement European collaboration for common scientific assessment report on the clinical added value of orphan medicines? Which measure in national plans for rare diseases?

11.00 - Coffee break

SESSION 2.2

11.20 - Improving Access to Orphan Drugs: How should reimbursement policies support access to treatment?

Rare disease patients deserve the same access to care as those suffering from more common conditions. But Member States are under pressure to manage healthcare budgets effectively. This means paying only for "things that work". Established methodologies are not tailored to suit the orphan medicinal products model. There is often a lack of understanding of rare diseases, orphan products and the process by which they are authorised. Do Member States' decision-makers have the tools they need to make decisions for rare disease treatments? If not, what needs to be changed or developed to help them evaluate this special sub-set of treatments?

Chair: Andrea Rappagliosi, Merck-Serono, joint task force EBE-Europabio, Switzerland
Speaker: Erik Tambuyzer, Genzyme, joint task force EBE-EuropaBio, Belgium
Rapporteur: (TBI)

11.40 - Panel debate

Stefaan Van der Spiegel, WG Pricing, Pharmaceutical Forum, DG Enterprise, European Commission, Belgium (TBC)
Kristin Raudsepp, Heads of Medicines Agency, Task Force Access, Estonia
Christine Lavery, Mucopolysaccharidosis Society, United Kingdom
Noreen Quinn, Irish representative, WG Pricing, Pharmaceutical Forum, Ireland

12.20 - Discussion with the audience: Questions and concrete recommendations

How to share common information to base pricing and reimbursement decision making by Member states? How to implement parallel requests for pricing and reimbursement to all Member states? How to fast track decision making procedures for orphan drugs? How to improve the reimbursement system for orphan medicinal products in the current regulatory framework?

13.20 – Lunch
SESSION 3

14.30 - Awareness and solidarity: How to deliver equitable access and social justice for rare diseases patients and their families?
Care for rare disease patients is delivered at a national level. But rare diseases often need special attention and a specific approach. France implemented a tailored approach for rare diseases over the past 4 years. And multi-stakeholder, expertise-based working groups in several Member States are developing their own strategies and action plans. The Commission Communication on Rare Diseases is providing a policy framework covering the wide spectrum of needed actions, including national plans for rare diseases as a cornerstone. So which measures should Member States deliver in their National Plans for Rare Diseases to deliver equitable access and social justice?

Chair: Bert Leufkens, WGM - Dutch Steering Committee on Rare Diseases and Orphan Drugs, The Netherlands
Speaker: Yann Le Cam, EURORDIS, COMP-EMEA, France
Rapporteur: (TBI)

14.50 – Panel debate
Domenica Taruscio, ISS, EuroPlan, Italy
Miroslav Zielinski, Orphan Forum - Krajowe Forum na rzecz terapii chorob rzadkich, Poland
Yves Juillet, LEEM, France
William Gunnarsson, Orphan Europe, France

15.30 - Discussion with the audience: Questions and concrete recommendations
How can stakeholders partner best to raise public awareness on rare diseases? Which measures in National Plans for Rare Diseases would we expect to deliver equitable access and social justice?

CLOSING SESSION

16.30 – Key messages & recommendations
Wills Hughes-Wilson, Genzyme, Co-Chair of the Workshop, Belgium

16.50 – Closing remarks
Roselyne Bachelot, Minister for Health, Youth and Sports, France (TBC)

For additional information about our activities:
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