Kære medlemmer og stedfortrædere af Sundhedsudvalget.

Sjældne Diagnoser er med i et europæisk samarbejde, EPPOSI, mellem patientforeninger, industri og akademia, hvor vi i fællesskab forsøger at fremme forskning i sjældne sygdomme og at bedre forholdene for mennesker med sjældne sygdomme.

EPPOSI afholder hvert år en konference et sted i Europa, og i år afholdes konferencen i København, nærmere bestmt d. 18.-19. oktober 2007 i Landstingssalen på Christiansborg.

Vedlagt er invitation til konferencen, som jeg fremsender på vegne af EPPOSI.

Mange hilsner

Lene Jensen,
Sjældne Diagnoser

EPPOSI
European Platform for Patients' Organisations, Science and Industry

September 24, 2007

8th WORKSHOP ON PARTNERING FOR RARE DISEASE THERAPY DEVELOPMENT
Copenhagen, Danish Parliament, October 18-19, 2007

Dear parlamentarian,

We are most pleased to invite you to attend the **8th EPPOSI Workshop on Rare Disease Therapy Development. EPPOSI** is the European Platform for Patients' Organisations, Science and Industry which, in a high level workshop in a different European capital city, brings together representatives from patients, regulators, the academic and clinical community, industry and health care providers from across Europe. The Workshop will discuss the reality of orphan medicines today, especially concentrating on the discussion of their value and on models for the future, ending with a session on awareness and communication.

The European orphan medicinal products Regulation has proven to be effective in boosting the development of new therapies, but payers are concerned about the costs. This Workshop aims to bring stakeholders together to discuss solutions for the future based on a realistic forecast.

Please find herewith the full programme of the Workshop enclosed. The Workshop will take place at the Parliament in Copenhagen. As a government representative, your registration fee will be waived, but please don't forget to register at www.epposi.org.

We look forward welcoming you there.

Yours sincerely,

Ms. Birthe B. Holm
Co-Chair for Patient Organisations
Rare Disorders Denmark - EURORDIS
Tel. + 45 33 14 00 10

Dr. Ségolène Aymé
Co-Chair for Science
Orphanet - INSERM
Tel. + 33 1 5653 8137

Dr. Erik Tambuyzer
Co-Chair for Industry
Genzyme
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Attachment : Programme

EPPOSI
European Platform for Patients' Organisations, Science and Industry

8th WORKSHOP ON PARTNERING FOR RARE DISEASE THERAPY DEVELOPMENT

"The Reality of Orphan Medicines"

Danish Parliament, Copenhagen, 18-19 October 2007

Folketinget, Christiansborg 1240 København K.

With the participation of

Her Royal Highness Crown Princess Mary of Denmark

With the kind support of the

Danish Parliament

PRELIMINARY PROGRAMME

Workshop co-chaired by:

Birthe B. Holm
Rare Disorders Denmark / EURORDIS
representing Patients
Vice-Chair COMP

Segolène Aymé
INSERM / Orphanet
representing Science

Erik Tambuyzer
Genzyme Europe
representing Industry

DAY 1 – Thursday, October 18, 2007

11:30 **Delegate registration**

12:30 **Lunch**

13:30 **Opening**
Alastair Kent, EGAN, Chair of EPPOSI
Birthe B. Holm, Rare Disorders Denmark / EURORDIS, Vice-Chair COMP, Chair of the Workshop
Lars Løkke Rasmussen, Minister for Interior and Health (TBC)

SESSION 1 – How to Estimate The Value of an Orphan Drug?

Chairs: ***Alastair Kent*** *(EGAN) and* ***Andrea Rappagliosi*** *(Merck-Serono)*

Patients with rare diseases have been historically under-served in commercial medicine development. In the 80s and 90s, a consensus emerged in several countries to address this disparity by means of orphan medicines legislation, enacted to encourage the development of medicines to treat those affected by life-threatening and/or serious rare diseases.

Existing orphan medicines policies have public support, as evidenced by several wide-ranging surveys. However, increasing cost pressures risk to result in a rationing system to control healthcare costs in general, creating additional access barriers for novel treatments,

which may in particular hamper access for orphan medicines. At this time, patients' access to orphan medicines seems variable while they also are not available to patients within the legal time limit after approval across the EU. Where available, they reach patients more slowly and sometimes with tighter conditions of access than other medicines.

The use of health economics for orphan medicines is still evolving and only limited attention has been paid to how it fits with the societal values expressed in the OMP regulation. This session will focus on these issues with the aim of:

1. *Promoting dialogue with multiple stakeholders on the role of health economics in creating a stable and sustainable access to and marketplace for orphan medicines.*
2. *Evaluating the current impact of applying health economics to the field of orphan medicine policy and research.*
3. *Discussing the basis for differentiating health technology and economic assessments of orphan medicines from the assessments of common disease medicines with specific consideration to rarity, innovation, and social values.*
4. *Identifying areas of further research to help identify and address shortcomings of current health economic methodology and develop criteria for an informed application of health economic evaluation to inform resource allocation decisions.*
5. *Involving all stakeholders to advance health policy and funding for orphan medicines.*

14:00 **Introduction**
***Andrea Rappagliosi**, Merck Serono*

14:05 **The patient's view on health technology assessment for Orphan Medicines: are there models?**
***Christine Lavery**, MPS Society / EURORDIS*

14:25 **Is HTA an appropriate tool to promote access to Orphan Drugs? –**
***Finn Borlum Kristensen**, Danish Center for Evaluation and HTA (DACEHTA), EUnetHTA*

14:50 **Access in a real-life setting: Member States experiences go live**
- France: ***Francois Meyer**, HAS*
- The Netherlands: ***Wim Goettsch**, CVZ*
- UK: ***Carole Longson**, NICE*

15:30 **Questions & Answers**

16:00 **Coffee break**

16:30 **A case study: industry experience**
***Jens Grueger**, Novartis*

16:50 **Can cost-effectiveness alone determine the value of Orphan Drugs?**
***Rod S. Taylor**, Associate Professor in Health Services Research, Peninsula Medical School - Universities of Exeter & Plymouth, United Kingdom*

17:10 **The patients' view.**
***Cees Smit**, Dutch Genetic Alliance - VSOP*

17:30 **Interactive session with audience participation**

17:55 **Concluding remarks**
***Alastair Kent**, EGAN*

18.00 **Session ends**

20:00 **Dinner Debate: the Communication from the European Commission on the Consultation regarding the European Action in the Field of Rare Diseases**
Location TBC

DAY 2 – Friday, October 19, 2007

08:30 **Delegate arrival, tea & coffee**

SESSION 2 – How Many Orphan Drugs – For How Many Patients?

Assessing Treatable Rare Diseases And The Proportion Of Patients Eligible For Treatment
Chairs: ***Ségolène Aymé*** *(INSERM / Orphanet) and* ***Erik Tambuyzer*** *(Genzyme Europe)*

The Orphan Drug regulation proves to be effective in boosting the development of therapeutic solutions. The time when there was limited rare disease research with no therapeutic options at all is over. This constitutes an era of optimism and constructive partnering, but the process is so effective that payers are concerned about costs in the future, as they anticipate that there may be a day when every rare disease could be treated with an orphan drug. Health care managers need to be provided with a more realistic forecast of the orphan medicinal products which may be granted a marketing authorisation in the coming ten years, as well as an estimate of the number of patients who may benefit of these products.
The Session on Epidemiology of orphan drugs "How many drugs for how many patients ?" will focus on these issues with the aim of:

1. *Presenting the data currently available*
2. *Discussing the predictive model based on FDA and EMEA data*
3. *Agreeing on the conclusions*
4. *Involving all stakeholders in a constructive dialogue with health authorities and third-party payers on this basis*

09:00 **Epidemiology and Literature Systematic review**
Ségolène Aymé, *INSERM / Orphanet*

09:20 **Forecast of Orphan Drugs based on the US experience**
Tan Nguyen, *FDA*

09:40 **Forecast of Orphan Drugs based on the European Experience**
Jordi Llinares, *EMEA*

10:00 **The Patients' view**
Birthe B.Holm, *EURORDIS or Pauline Evers, VSOP*

10:20 **An industry point of view**
Christian Klaus, *Novartis or* ***Geoff McDonough***, *Genzyme (TBC)*

10:40 **Interactive session with audience participation**

12:30 **Lunch**

SESSION 3 – How to Communicate about Orphan Drugs in the Real Life Setting?

14:00 **Patients' Access to Orphan Drugs in the EU: an EURORDIS survey**
Yann Le Cam, *EURORDIS*

14:20 **Orphan Drugs today: what are the communication challenges?**
Bert Leufkens, *Dutch Steering Committee Orphan Drugs - WGM, Utrecht Institute for Pharmaceutical Sciences, Division of Pharmacoepidemiology & Pharmacotherapy*

14:40 **Industry's communication about orphan drugs**

Erik Tambuyzer *(TBC), Chair EBE/EuropaBio orphan drugs task force, Genzyme Europe*

15:00 **Interactive discussion**

15:45 **Conclusion of the Workshop**
Birthe B. Holm*, Rare Disorders Denmark / EURORDIS, Vice-Chair COMP, Chair of the Workshop*

16:00 **Workshop ends**

For additional information:
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