

10th EPPOSI WORKSHOP ON PARTNERING FOR RARE DISEASE THERAPY DEVELOPMENT

*10 years after the Adoption of the EU Orphan Medicines Regulation:
Where do we go to?*

-
Belgian Federal Parliament - Brussels, Belgium
-

Monday 26- Tuesday 27 October 2009

MONDAY 26 OCTOBER

Opening - In the presence of H.R.H. Princess Astrid of Belgium

10 years of Partnering for rare disease therapy development

Alastair Kent, Chair EPPOSI – United Kingdom

Yolande Avontroodt, Chair Committee Science and Technology of Parliament - Belgium

SESSION 1 – IMPACT OF THE ECONOMIC CRISIS ON THE FIELD OF RARE DISEASES :

WHAT IS THE IMPACT ON R&D, DIAGNOSIS AND ACCESS?

Sessions Moderators: Alastair Kent, Genetic Interest Group/ European Genetic Alliances' Network, Chair EPPOSI – United Kingdom
Erik Tambuyzer, Genzyme, Vice Chair EPPOSI – Belgium

Panel Discussion

Birthe Holm, EURORDIS and COMP – Denmark

Ségolène Aymé, Chair of the Rare Diseases Task Force, DG SANCO – France

Jo De Cock, INAMI/RIZIV – Belgium

Willem van Weperen, to-BBB – The Netherlands

Timothy M. Cox, University of Cambridge – United Kingdom

Heinrich Schulte, ESCI – Germany

Discussion with the audience

TUESDAY 27 OCTOBER

SESSION 2 – BUILDING ON THE POLICY BASE OF THE LAST 10 YEARS TO ADVANCE POLICY IN THE NEXT 5 YEARS

Sessions Moderators : Yann Le Cam, EURORDIS, EPPOSI – France
Giulia Del Brena, European Commission, DG Enterprise & Industry – Belgium

Introduction/Overview

Yann Le Cam, EURORDIS – France

How to increase the number of Rare disease therapies under Research & Development to cover unmet medical needs?

Kerstin Westermark, COMP – Sweden

Ségolène Aymé, Orphanet – France

Panel: Manuel Hallen, European Commission, DG Research – Belgium

Pauline Evers, Nederlandse Federatie van Kankerpatientenorganisaties – the Netherlands

Discussion with the audience

How to improve the success rate of OD Development between Orphan Drug Designation (ODD) and Marketing Authorisation?

Katerina Kubackova, University Hospital of Motol – Czech Republic

Jordi Llinares, European Medicines Agency – United Kingdom

Panel: Kathy Needleman, Director of Orphan Products Grants Program, Office of Orphan Products Development Food and Drug Administration – USA
Marie-Christine Fortun, Orphan-Europe – France

Discussion with the audience

How to provide sustainable access to OMP at national level?

Wills Hughes-Wilson, Genzyme – Belgium
Andrea Rappagliosi, GlaxoSmithKline – Switzerland

Panel : Mattias Neyt, Centre Fédéral d'expertise des soins de santé / Kenniscentrum voor de Gezondheidszorg – Belgium
Larry Warren, ALFA Europe – Ireland

Discussion with the audience

SESSION 3 – RARE CANCERS

Session Moderators : Paolo Casali, European Society for Medical Oncology – Italy
Jean-Jacques Cassiman, European Society for Human Genetics, EPPOSI – Belgium

Introduction

Jeanne-Marie Bréchet, Institut National du Cancer – France
Jean-Jacques Cassiman, ESHG – Belgium

How to address the needs of Patients with rare cancers? – Commonalities and differences with rare diseases.

Paolo Casali, ESMO – Italy
Kerstin Westermark, COMP – Sweden
Markus Wartenberg, Das Lebenshaus – Germany
Andras Fehervary, Novartis – Switzerland

Questions and Answers

Clinical research, therapy development and regulatory considerations

Jean-Yves Blay, Conticanet – Belgium
Jan Geissler, European Cancer Patient Coalition – Germany
Katrin Rupalla, Celgene – Switzerland

Questions and Answers

Access (to information, timely and correct diagnosis, therapies including centres of excellence/HTA/reimbursement/multidisciplinary teams, patient mobility, etc)

Garth Cruickshank, Queen Elizabeth Hospital, Birmingham – United Kingdom
Kathy Oliver, International Brain Tumour Alliance – United Kingdom
Karl Freese, European Commission, DG Health and Consumer Affairs – Belgium
Adam Heathfield, Pfizer, United Kingdom

Questions and Answers

Discussion with the audience

Where are we? What have we achieved on the RT conference recommendations? What should we jointly do as next actions?

Chairs: Paolo Casali, ESMO – Italy
Jean-Jacques Cassiman, ESHG – Belgium

CLOSING SESSION

Conclusions and Next Steps

Alastair Kent, Chair EPPOSI – United Kingdom
Erik Tambuyzer, Vice Chair EPPOSI – Belgium