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SMi present their inaugural conference on...

Orphan Drugs & Rare Diseases

Innovative strategies for getting orphan drugs to where they are needed...

Monday 8th & Tuesday 9th October, 2012
The Copthorne Tara Hotel, London, UK

KEY SPEAKERS INCLUDE:

Gary J. Clements Ph.D
Senior Director, Business Development
Shire Pharmaceuticals

Dr. Elisa Muscianisi
Bone, Inflammation & Rheumatology Areas
Novartis

Richard Philipson
Disease Area Head
GSK Rare Diseases

Karen Aiach
CEO / Founder - Director Research and Development Programs
Lysogene

Tony Hall,
Founder
OneOrphan

Géraldine Honnet
Head of Clinical
Genethon

Celine Plisson
Associate Medical Director
Orphan Europe Recordati Group

Thomas Meier
Chief Executive Officer
Santhera Therapeutics

Celine Plisson
Associate Medical Director
Orphan Europe Recordati Group

Josie Godfrey
Head of Policy and Coordination, National Specialised Commissioning Team
Advisory Group for National Specialised Services

Dr. Ségolène Aymé
Chair
EUCERD, INSERM, EU Commission

Martine Zimmermann
Executive Director Global Regulatory Affairs
Alexion Pharma

KEY TOPICS:

- **Innovations in gene therapy**
- **Partnerships across sectors to develop and implement orphan medicines**
- **Successful business strategies and economic models for orphan drugs**
- **Sources of funding and financing for orphan drug research and development**
- **The global regulatory environment and health technology assessments**

PLUS ONE INTERACTIVE FULL-DAY POST-CONFERENCE WORKSHOP
Wednesday 10th October 2012, The Copthorne Tara Hotel, London, UK

Orphan Drugs - From Application to Market Access

Workshop Leader: **Dr Tony Hall,** Chief Medical Officer, **PSR: the orphan drug experts**
8.30am - 4.45pm

To attend, contact Fateja Begum on Tel +44 (0) 20 7827 6184, Fax +44 (0) 20 7827 6185, email fbegum@smi-online.co.uk or visit www.smi-online.co.uk/ts05.asp to register online

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Orphan Drugs & Rare Diseases

Day One | Monday 8th October 2012

www.smi-online.co.uk/ts05.asp

8.30 Registration and Coffee

9.00 Chairman's opening remarks
Olivier Menzel, Founder & President, **Blackswan Foundation**

RECENT ORPHAN DRUGS DEVELOPMENTS AND REGULATIONS

OPENING ADDRESS – KEYNOTE SPEAKER

9.10 **Shire's orphan drug philosophy: Creating Stakeholder value**
- The past: The early years of orphan drugs and Shire's emerging leadership
- The present: Delivering value, growth and consolidation
- The future: Embracing innovation – imagining the possibilities: leading through the challenges

Gary J. Clements Ph.D, Senior Director, Business Development, **Shire Pharmaceuticals**

9.50 **Orphan drugs and rare disease policy in the UK: Perspectives from a Patient Group**
- Towards a successful patient-focused national strategy
- Impact of policy and regulations on access to orphan drugs
- What are the obstacles and limitations for access to treatment?
- Partnership working to develop personalised treatment and care

Stephen Nutt, Executive Officer, **Rare Disease UK**

10.30 Morning Coffee

10.50 **The BLACKSWAN Foundation: a unique organisation supporting orphan disease research**
- Promoting and supporting research into rare disease
- Research priorities
- The RE(ACT) Congress: Bringing together researchers and their knowledge
- The RE(ACT) Community: an online platform to strengthen synergies between people involved in rare and orphan disease research

Olivier Menzel, President and Founder, **Blackswan Foundation**

11.30 **Specialised Drugs and Technology: Appraisals and Selection**
- Health Technology Assessment requirements
- Assessing the effectiveness, costs and impact of new treatments
- How to demonstrate comparative value
- Developing a productive relationship between the health authority and industry

Josie Godfrey, Head of Policy and Coordination, National Specialised Commissioning Team, **Advisory Group for National Specialised Services (AGNSS), UK**

12.10 Networking Lunch

1.30 **Innovative Models for Transferable R&D**
- Combining complex scientific expertise to improve rare disease therapy
- Achieving Business & Research relationships to progress orphan drug discovery
- What are the financial parameters for rare disease treatment and orphan drug development?
- Partnership with patients' families, communities and patient groups: the scientific and ethical commitments

Karen Aiach, CEO / Founder - Director Research and Development Programs, **Lysogene**

2.10 **Building a Sustainable Pipeline in Rare Diseases: from Discovery to Commercialisation**
- Rare disease prioritization: how to maximize R&D potential
- Fuelling the R&D machine: internal discovery engines and external partnerships
- Maximising the opportunities for new technologies in rare diseases
- From discovery to treatment and commercialization
- Case study: an innovative model for developing treatments

Richard Philipson, Disease Area Head, **GSK Rare Diseases**

2.50 Afternoon Tea

3.10 **Orphan Drugs Clinical Trials**
- Global Clinical Development
- Specific gene therapy trials challenges:
  - Regulatory submissions
  - Design
  - Recruitment
  - Statistics

Géraldine Honnet, Head of Clinical, **Genethon**

PERSPECTIVES FROM PATIENT GROUPS AND ORPHAN DRUG SPECIFIC COMPANIES

3.50 **Global Developments in Rare Diseases and Orphan Drugs: Partnerships and Business Strategies**
- The current state of rare diseases and orphan drugs in the world
- Is there an orphan drug business development model, focusing on orphan drugs for very rare diseases?
- Partnerships in research, development and treatment for rare diseases

Celine Plisson, Associate Medical Director, **Orphan Europe Recordati Group**

4.30 **Ilaris case study: launch of an orphan drug**
From clinical development to launch: the orphan drug experience
- Fasten patient identification during the clinical development of Ilaris
- Increase disease awareness:
  - Local advocacy plan
  - Campaign for facilitating networking/mapping
  - Involvement of the patients association
- Assure patient access to the drug
- Ensure fast local access to Ilaris:
- Key centers mapping and KOL mapping
- Mapping of regional orphan diseases related listing process
- Ensure all relevant data for local dossier submissions

Elisa Muscianisi, Bone, Inflammation & Rheumatology Areas, **Novartis**

5.10 **Chairman's Closing Remarks and Close of Day One**

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Who should attend this conference:

Chief Executive Officers, Chief Scientific Officers, Chief Operating Officers, Chief Medical Officers, Vice Presidents, Directors, Professors, Heads, and Managers in::

- Orphan Medicines & Rare Diseases
- Medical Affairs
- Medical Technology & Medical Devices
- Health Technology Assessment
- Clinical Trials
- Clinical Development
- Product Development
- Research & Development
- Drug Discovery
- Inflammation
- Operations
- Regulatory Affairs
- Market Access
- Policy & Public Affairs
- Partnering & Strategic Alliances
- Business Development

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8.30 Re-registration and Coffee

9.00 Chairman's opening remarks
Donald Macarthur, Independent Consultant and Principal, **Justpharmareports**

NATIONAL PLANS AND PARTNERSHIPS

OPENING ADDRESS

9.10 **The Benefits of Patient Groups, Pharmaceutical Companies and Health Institutions Working Together**
- Learning from each other
- Political and practical achievements so far due to this partnership
- Points on the agenda for the years to come
- Areas for public/private partnership

Ségolène Aymé, Chair, **EUCERD, INSERM, EU Commission**

KEYNOTE SPEAKER

9.50 **Working with Regulators: A Market Overview**
- The current European and Global regulatory environment for orphan drugs
- An industry overview on Achieving global Orphan designation Status
- Comparisons between international country regulations

Martine Zimmermann, Executive Director Global Regulatory Affairs, **Alexion Pharma**

10.30 Morning Coffee

MARKET ACCESS AND BUSINESS STRATEGIES

10.50 **Achieving Orphan Drug Status and impact on collaborations and business development: An Industry Perspective**
- Incentives for industry to develop rare disease medicines and technologies
- Working with partners to enable regulatory and clinical development
- Orphan status and how to approach intellectual property issues
- Developing innovative and entrepreneurial strategies in the orphan drug business

Anders Waas, Chief Executive Officer, **Tikomed**

11.30 **Regulatory challenges and strategies in drug development for orphan diseases – a case study**
- What are the challenges in conducting a first-ever controlled clinical study in an ultra rare indication?
- How and when should regulatory bodies be approached for scientific advice?
- How to secure the continued support from health authorities in preparing for MAA filing?

Thomas Meier, CEO, **Santhera Therapeutics**

12.10 Networking Lunch

1.30 **Regulations, Incentives and Technology Assessments for Gene Therapy in Rare diseases**
- Research and development far ahead from regulations adaptation
- Value of scientific advice and protocol assistance
- Fees, grants and tax reduction
- How to assess innovative, breakthrough technology with past knowledge

Carlos R. Camozzi, Vice President - Chief Medical Officer, **uniQure B.V.**

2.10 **Orphan drug policy & its results in Asian countries**
- Japan, Korea, Taiwan
- Incentives to orphan drug sponsors
- Role of HTA and risk sharing in reimbursement
- Special funding for orphan drugs
- Case studies

Donald Macarthur, Independent Consultant & Principal, **Justpharmareports**

2.50 **Is the business model for orphan drug development sustainable and are there any alternatives?**
- Efficacy of the incentives for orphan drugs
- Pricing & reimbursement issues
- The sustainability of the current business models
- Social enterprise – an alternative to the traditional approach?

Tony Hall, Founder, **OneOrphan**

3.30 Afternoon Tea

3.50 **Achieving Orphan Medicine Status and Regulation of Specialised Technologies: An Industry Perspective**
- Incentives for industry to develop rare disease medicines and technologies
- Working with global partners to increase provision of treatment
- Increasing medical and scientific knowledge of rare diseases through partnerships
- Developing innovative and entrepreneurial strategies in the orphan drug business

Marisa Jaconi, Vice Director, Principal Investigator and Maître d'Enseignement et Recherche, **Swiss Institute of Cell Therapies**

4.30 **Consortium Cooperation in Rare Disease Research and Orphan Drug Development: Two Case Studies**
- An International Consortium: Research, resources and expertise sharing
- Collaborative rather than competitive working for the end result
- An innovative model of drug developments and business strategy: - A Not-For-Profit Approach: AKU Society
- Future developments and trends in rare disease treatments

Nicolas Sireau, Chairman, **AKU Society**

5.00 **Gene Therapy: an overview and selected clinical studies**
- The importance of gene therapy for rare diseases
- Basic gene therapy tools
- Successful clinical studies
- The challenges ahead

Dr Rafael Yáñez, Lead Scientist, Royal Holloway, **University of London**

5.30 **Chairman's Closing Remarks and Close of Day Two**

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FULL-DAY POST-CONFERENCE WORKSHOP
Wednesday 10th October 2012
8.30am – 4.45pm
Copthorne Tara Hotel, London, UK

Orphan Drugs - From Application to Market Access

In association with

PSR theorphanEXPERTS

Overview of workshop

This workshop offers an ideal introduction to pharmaceutical orphan drugs through step by step analysis of the orphan drug process - from orphan drug application all the way through market access. The master class will offer an accessible and comprehensive overview, and will be of particular use to companies looking towards further involvement in orphan drug development. Lessons can also be learned for companies currently within the orphan drug sphere with analysis of the current orphan drug landscape and isolation of key orphan diseases.

Key topics include:

- Designing clinical trials for orphan indications
- Rare diseases from the patient's perspective
- Market access and reimbursement for orphan drugs
- Patient advocacy groups
- Guide to writing an orphan drug application (EU&US)
- Variations in patient access between countries
- Predictors of success or failure
- Roles & achievements of EURORDIS

Programme

8.30 **Registration & Coffee**

9.00 **Welcome – Introduction to orphan drugs**
- Background
- Regulations
- Definitions
- Incentives
- Relevant regulatory bodies

9.45 **Guide to writing an orphan drug application (EU&US)**
- Procedure
- Sections of application
- Sub-setting

10.45 **Coffee Break**

11.15 **Designing clinical trials for orphan indications**
- Choice of endpoint
- Ethical issues
- Protocol assistance
- Predictors of success or failure
- Surrogates
- Guidance documents
- Case studies

12.15 **Registries for rare diseases**

1.00 **Lunch**

2.00 **Rare diseases from the patient's perspective**
- Problems with diagnosis
- Isolation
- Lack of medical expertise

2.45 **Patient advocacy groups**
- Roles & achievements of EURORDIS
- NORD & individual patient groups

3.30 **Afternoon Tea**

4.00 **Market access and reimbursement for orphan drugs**
- Value of orphan drugs
- Variations in patient access between countries

4.45 **Close of Workshop**

About the workshop host

Dr Tony Hall, Chief Medical Officer, **PSR: the orphan drug experts**

Tony graduated with first class honours in physiology & pharmacology from King's College London and later qualified as a doctor in at the Royal Free Hospital, London. He specialised in Emergency Medicine before joining the pharmaceutical industry in 1994.

His first industry position was at Boehringer Ingelheim, where he was responsible for the strategic development of many clinical programs and he was the medic responsible globally for two of Boehringer's products. He later worked at Yamanouchi before starting his own business.

As Chief Medical Officer of PSR, Tony has developed an in-depth knowledge of the orphan drug world, the applicable regulations and procedures. He has also built trusted relationships with patient groups in order to help ensure that the patient's voice is heard in clinical trials with which PSR is involved.

Tony is able to provide advice and guidance on development plans for orphan drugs, including applying for orphan designation, protocol assistance and the design of clinical programs.

Orphan Drugs & Rare Diseases

SMi's Pharmaceutical Forward Planner 2012

JUNE

11-12 RNAi & Nanotechnology

JULY

2-3 KOL Management and MSL Best Practice in Europe (Switzerland)
9-10 ADMET
9-10 Social Media in the Pharmaceutical Industry
11-12 BioBanking

SEPTEMBER

17-18 Next Generation Sequencing
19-20 Cancer Vaccines
24-25 Biosimilars and Biobetters
26-27 KOL Management

OCTOBER

3-4 Partnerships with CROs
8-9 Pharmaceutical Orphan Drugs
22-23 COPD: Novel Therapeuticsand Management Strategies
24-25 Point of Care Diagnostics - Market Opportunities and Technology Trends
29-30 European Pharmaceutical Pricing & Reimbursement

NOVEMBER

5-6 Cell Based Assays
5-6 Clinical Trials in CNS
28-29 Diabetes

DECEMBER

3-4 Cold Chain Distribution

All conferences take place in central London, UK – unless indicated otherwise in brackets

ORPHAN DRUGS & RARE DISEASES

Conference: Monday 8th & Tuesday 9th October 2012, The Copthorne Tara Hotel, London, UK Workshop: Wednesday 10th October 2012, London, UK

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