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
Shire’s orphan drug philosophy: Creating Stakeholder value

The BLACKSWAN Foundation: a unique organisation supporting Specialised Drugs and Technology: Appraisals and Selection

Global Developments in Rare Diseases and Orphan Drugs: www.smi-online.co.uk/ts05.asp

Chairman’s Closing Remarks and Close of Day One

Day One | Monday 8th October 2012

Orphan Drugs & Rare Diseases

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

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

10.50 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 Aicah, 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

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

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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The Benefits of Patient Groups, Pharmaceutical Companies and Consortium Cooperation in Rare Disease Research and Orphan Drug Gene Therapy: an overview and selected clinical studies

Orphan drug policy & its results in Asian countries

Regulatory challenges and strategies in drug development for orphan diseases – a case study

Achieving Orphan Medicine Status and Regulation of Specialised Technologies, Incentives and Technology Assessments for Gene Therapy in Rare diseases

Is the business model for orphan drug development sustainable and are there any alternatives?

MARKET ACCESS AND BUSINESS STRATEGIES

Achieving Orphan Drug Status and impact on collaborations and business development: An Industry Perspective

Regulatory challenges and strategies in drug development for orphan diseases – a case study

NATIONAL PLANS AND PARTNERSHIPS

OPENING ADDRESS

9.10 The Benefits of Patient Groups, Pharmaceutical Companies and Health Institutions Working Together

 KEYNOTE SPEAKER

9.50 Working with Regulators: A Market Overview

10.50 Achieving Orphan Drug Status and impact on collaborations and business development: An Industry Perspective

11.30 Regulatory challenges and strategies in drug development for orphan diseases – a case study

12.10 Networking Lunch

2.10 Orphan drug policy & its results in Asian countries

2.50 Is the business model for orphan drug development sustainable and are there any alternatives?

3.30 Afternoon Tea

3.50 Achieving Orphan Medicine Status and Regulation of Specialised Technologies: An Industry Perspective

4.30 Consortium Cooperation in Rare Disease Research and Orphan Drug Development: Two Case Studies

5.00 Gene Therapy: an overview and selected clinical studies

5.30 Chairman’s Closing Remarks and Close of Day Two

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Supported by
# 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

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
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<tbody>
<tr>
<td>8.30</td>
<td>Registration &amp; Coffee</td>
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<tr>
<td>9.00</td>
<td>Welcome – Introduction to orphan drugs</td>
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<tr>
<td>9.45</td>
<td>Guide to writing an orphan drug application (EU&amp;US)</td>
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<td>10.45</td>
<td>Coffee Break</td>
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<tr>
<td>11.15</td>
<td>Designing clinical trials for orphan indications</td>
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<td>12.15</td>
<td>Registries for rare diseases</td>
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<td>1.00</td>
<td>Lunch</td>
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<td>2.00</td>
<td>Rare diseases from the patient’s perspective</td>
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<td>Patient advocacy groups</td>
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<td>Afternoon Tea</td>
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<td>4.00</td>
<td>Market access and reimbursement for orphan drugs</td>
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<tr>
<td>4.45</td>
<td>Close of Workshop</td>
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## 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.
**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 Therapeutics and 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
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Workshop: Wednesday 10th October 2012, London, UK

4 WAYS TO REGISTER

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

Fax your booking form to +44 (0) 20 7827 6185
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EARLY BIRD DISCOUNT

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PROMOTIONAL LITERATURE DISTRIBUTION

- Distribution of your company’s promotional literature to all conference attendees

£999.00 + VAT £1198.80

GROUP DISCOUNTS AVAILABLE

The conference fee includes refreshments, lunch, conference papers and access to the Document Portal containing all of the presentations.

VENUE
Copthorne Tara Hotel, Scarsdale Place, Kensington, London W9 5SR

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- Substitutions/Name Changes: If you are unable to attend you may nominate, in writing, another delegate to take your place at any time prior to the start of the event. Two or more delegates may not share a place at an event. Please make separate bookings for each delegate.
- Cancellation: If you wish to cancel your attendance at an event and you are unable to send a substitute, then we will refund/credit 50% of the due fee less a £50 administration charge, providing that cancellation is made in writing and received at least 28 days prior to the start of the event. If you wish to cancel the event for any reason, then we will make a full refund immediately, but disclaim any liability. We cannot accept cancellations of orders placed for the conference documentation via the Document Portal to any delegate who has paid but is unable to attend the event. Regrettably cancellation after this time cannot be accepted. We will however provide the conference documentation via the Document Portal containing all of the presentations.
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