Orphan Drugs Summit 2011

14th – 16th September, 2011, Radisson Blu Royal Hotel, Copenhagen, Denmark

THE INTERNATIONAL EXCHANGE OF KNOWLEDGE FOR TREATING RARE DISEASES

HIGHLIGHT TOPICS COVERED IN 2011

- Join other leaders in the forefront of Orphan Drug development
- Meet head of leading companies responsible for following health policies
- Review the role of large Pharma in the Orphan Drugs' sector
- Accelerate orphan drug commercialisation from manufacturing to market
- Understand patient access, affordability and issues affecting reimbursement

EXPERT PANELISTS:

Ad Schuurman, Head of the Business Contact Center & International Affairs, **Dutch Health Care Insurance Board (CVZ)**

Omar Ali, Formulary Development Pharmacist, **Surrey & Sussex NHS Trust**. Omar is also member of the External Reference Group on Cost Impact Modelling for **NICE**.

Tom Bols, VP Health Policy and Market Access, **Merck Serono**

Pierrick Rollet, VP, Global Market Access/ Advocacy/Communications, **GlaxoSmithKline Rare Diseases**

Adam Heathfield, Director of Policy, **Pfizer**

INDUSTRY SPEAKERS:

TopoTarget A/S
François Martelet, CEO

Swedish Orphan Biovitrum AB
Bo Jesper Hansen, Chairman of the Board

AMT
Jörn Aldag, CEO

Zymenex
Jens Fogh, CEO

Actelion
Otto Schwarz, President Business Strategy & Commercial Operations

Genzyme
Wills Hughes-Wilson, Senior Director & Health Policy Europe

Kakkis EveryLife Foundation
Emil Kakkis President

GlaxoSmithKline
Andrea Rappagliosi, VP European Government Affairs and Head of Brussels Office

Shire,
Gary Clements Director, Senior Director Business Development (Invited speaker)

Sjældne Diagnoser
Birthe Byskov Holm, President

Media Partners:

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rare, orphan and neglected diseases

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Orphan Drugs Summit 2011

Dear Colleagues

Over 55 million people are estimated to suffer from a rare disease in Europe and in the US. Global estimates are between 5000 to 7000 rare diseases. New rare diseases are discovered every week and many have no treatments available. * For the pharma industry, the cost of developing a rare-disease medicinal product to the market would not be covered by the expected sales of the product, if it was not for economic and regulatory incentives in place Europe and the US to encourage drug companies to develop and market medicines for the rare disease patients.

The results of regulatory initiatives are clear: 344 drugs have become approved products in the US during the past 25 years and 69 new medicinal orphan products came up to the European market, whilst before EC 141/2000, there were only 8 products in Europe.** Estimates indicate that the market earned revenues for orphan drugs will reach USD 27.09 billion in 2015**.

Therefore it is no surprise that this niche market is now attracting an even higher interest from not only small and medium enterprises, but from big pharmaceutical companies, which are looking at adding orphan drugs products to their blockbuster portfolios.

If you are looking forward to being part of the orphan drugs business and policy of tomorrow, attend the Orphan Drugs Summit 2011.

Join us to get the latest on:

- ***Market incentives for Orphan Drug designation***, *steps to market access and approval process and successful partnerships that were built to effectively distribute and safeguard patient access to orphan drugs globally.*
- ***Best practices*** *from EU Member States such as the Netherlands, UK and France regarding issues on pricing and reimbursement approaches*
- ***Dynamics in the Orphan Drugs market involving large pharma alliance***, *new partnership and business models expansion and the impact to the existing orphan drugs market practice.*
- ***Improved clinical trials***, *diagnosis and assessment to ensure safety and quality to speed up patient access to orphan drugs.*
- ***Regulations affecting the EU & US orphan drugs industry***, *funding mechanisms, collaboration initiatives and processes to support orphan drug development.*

I look forward to welcoming you to the Orphan Drugs Summit 2011.

Beatriz Viellas
Senior Project Manager
Pharma Division

* Heemstra, H.E., et al., Orphan drug development across Europé: Bottlenecks and opportunities, Drug Discov Today (2008), doi10.1016/j.drudis.2008.05.001
** Pharmaceutical Starting Materials, Market News Services (Bi-monthly Edition)
*** European Commission Public Health Rare Disease, The orphan drugs strategy.
http://ec.europa.eu/health/rare_diseases/orphan_drugs/strategy/index_en.htm

WHO WILL ATTEND?

Orphan Drugs and Rare Diseases Stakeholders Attending:

- **Pharmaceutical companies in the fields of oncology, haemophilia, genetic therapies, paediatrics and other rare diseases fields.**
- **Biotech organisations involved with orphan drugs**
- **Patient Organisations and Patient Access Institutions**
- **European and Member States Regulatory Bodies**
- **Payers & Reimbursement Authorities, HTAs**
- **Academics, Research Centres, Public Sector entities and Hospitals**

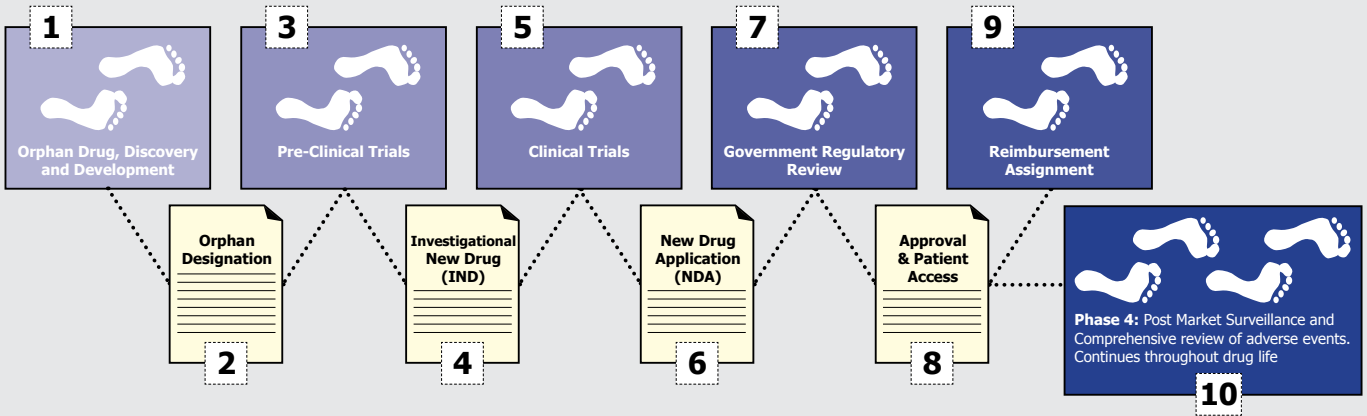
Job Titles:

- **CEOs, CFOs and CTOs**
- **VP, Heads of and Directors of: Orphan Drugs Business Development, Rare Diseases Business Development Unit, Clinical Trials & Therapeutics, Market Access and Regulatory and Public Policy**

P.S. Attend the Orphan Drugs Summit 2011 to influence the orphan drugs business and policy of tomorrow.

Book now!

Your summit experience will cover the life cycle of orphan drug development. Join the Experts discussing 18 Topics over three days



CONFERENCE-AT-A-GLANCE

Day 1:
17.00 Welcome Address
18.00 Evening Networking & Drinks

Day 2:
08.10 Registration
08.40 Plenum conference starts
12.20 Networking lunch
13.20 Plenum conference continues
17.20 – End of day 1 & Dinner Reception

Day 3:
08.30 Coffee & Networking
08.40 Conference program
10.30 – 12.00 Round tables
12.20 – End of conference

Day 2:

8.10 Registration & Coffee
8.35 Chairman Opening Address

1-5

08.40 Financing and creating a sustainable pipeline of orphan drugs in the portfolio

Zymenex A/S focuses on the development of therapies for rare, genetic diseases and specifically the development of Recombinant Enzyme Replacement Therapy (ERT) for lysosomal diseases. Zymenex, a company that has managed to keep a sustainable pipeline of orphan drugs in development, has minimized costs by outsourcing development tasks and has experience in achieving EU grants and working with international partners to develop orphan drugs. Learn from their experiences with:

- *Developing ERT for lysosomal diseases through project management and outsourcing*
- *Putting together international scientific and clinical collaborations*
- *Raising research funds through the EU 5th, EURAMAN and 6th, HUE-MAN, framework programs*
- *Implementing clinical trials by raising EU Grant ALPHA-MAN via the 7th framework program*

Jens Fogh
CEO
Zymenex

1-5

09.20 Success factors to build an efficient clinical development plan and ensure market access to orphan drugs

Hear from TopoTarget about its dedicated business to develop and market improved oncology therapies and innovative drugs, such as belinostat, which is a De A Cetylase Inhibitor (HDACi). With blockbuster potential, Belinostat is currently in its first pivotal trial. Topotarget has an agreement for the development and commercialization of Belinostat in North America with a US partner Spectrum Pharmaceuticals. Learn from them on how to:

- *Move towards late stage clinical development to determine significant medical benefit and meet regulatory thresholds for orphan drugs*
- *Research collaboration, out-licensing and partnership agreements as alternatives to ensure the effective handling of clinical development and advance product-to-market*

François Martelet
CEO
TopoTarget A/S

topotarget

09.50 Coffee & Networking Break

UNIQUE NETWORKING & INTER-ACTIVITY:

2 extra panel debates on: (a) pricing, technology and financial standards to Orphan Drugs and (b)

Large pharma's role in the orphan drugs sector

5 Round tables: the networking zone will provide you unique face-to-face contact, extensive informal discussions to gain insights on crucial issues

Not just a congress: **drinks reception, dinner & activities** included to give you extra networking and time with your peers.

8-9

10.20 Panel: Pricing and regulations issues in European Member States: Discussions covering Payers' criteria, fast track approvals and initiatives to support orphan drug development

Developments of orphan drugs have been considerable over the past years, although challenges still remain at Member State level to support patient access and orphan drugs development. Life expectancy of patients suffering from rare diseases will only be affected once patients gain access to drugs. At this panel, best practice from key European Member States will be discussed concerning key areas such as pricing, reimbursement & fast track approvals:

- *France fast track system, early market access and reimbursement approaches*
- *The Netherlands dedicated orphan drug reimbursement regulations, planned re-assessment of affordability and financial standards to orphan drugs*
- *UK orphan price-setting issues & reimbursement approaches*

Panelists:

Ad Schuurman MBA, Head of International Relations, **CVZ** - Dutch Health Care Insurance Board

Omar Ali, Formulary Development Pharmacist, **Surrey & Sussex NHS Trust**. Omar is also member of the External Reference Group on Cost Impact Modelling for **NICE**.

Invited speaker: **HAS- Santé**

2-9

11.00 EU future & current policy developments affecting funding, patient access & sustainability of orphan drugs development

- *Regulation EC 141/2000 criteria for orphan drug designation status: "Unique" or "Significant Clinical Benefit"*
- *Issues and consequences arising from conditional pricing & reimbursement decisions: market access and financial support*
- *Clinical Added Value of Orphan Drugs & EU Member States collaboration status: issues impacting disease awareness, patient access and affordability of orphan drugs*
- *Latest Policy and regulatory developments to be aware of: DG Enterprise – CSR in Pharmaceuticals, Centres of Expertise and the EMA Road Map 2015: accelerated assessment scheme and other incentives to orphan drugs development.*

Wills Hughes-Wilson
Senior Director, Health Policy Europe
Genzyme

genzyme

3-5

11.40 Overcome clinical trials challenges and safety issues - a gene therapy case study

- *Building internal processes and manufacturing platforms to in-house develop AAVs and ensure quality clinical trials*
- *Fostering relationship and early engagement with authorities to mitigate risks and speed market authorization*
- *Providing sufficient info on therapy benefits with the right quality, safety and efficacy from a reduced patient numbers*

Jörn Aldag
CEO
AMT Biopharma

2-9

14.00 The role of Health Technology Assessments

- *Will coordinated mechanisms improve patients' access to orphan medicinal products?*
- *The role of HTA networks in assessing the value of orphan drugs*
- *Stakeholders involvement in access decisions as a key enabler for patients suffering from rare diseases*

Andrea Rappagliosi
VP European Government Affairs & Head of Brussels Office
GlaxoSmithKline

Andrea is currently chairing the Board of EuropaBio and co-chairing the EFPIA Task Force on HTA

12.20 Lunch Break

14.40 Coffee & Networking Break

6-10

13.20 Value-Based Pricing & Technology appraisals: UK & Global policy developments and how they affect pricing and reimbursement processes

25 % of pharmaceutical global market sales use the UK as a price referencing benchmark. At this presentation, you will hear from the External Reference Group on Cost Impact Modelling for NICE, which is setting up the Roadmap for Drug Pricing in the UK by 2014, including on:

- *Pricing and reimbursement process changes to consider when setting your orphan drug's price: how to demonstrate the value of your innovation*
- *How thresholds will be set to guide NHS reimbursement*
- *Price thresholds affecting maximum price for orphan drugs*

Omar Ali, Formulary Development Pharmacist, **Surrey & Sussex NHS Trust**. Omar is also member of the External Reference Group on Cost Impact Modelling for **NICE**.

6-10

13.40 Panel: Large Pharma role in the Orphan Drugs' Sector: New business models to enhance orphan drug portfolio development and the impact on the market

- *Licensing, partnerships, joint-ventures: alternatives to expand your orphan drugs' portfolio*
- *Shifting to orphan drugs to enhance your competitive edge at new and existing markets globally*
- *Assessing the rare disease and therapeutic areas of focus*

Panelists:

Tom Bols, VP Health Policy and Market Access, **Merck Serono**

Pierrick Rollet, VP, Global market access/advocacy/ communications, **GlaxoSmithKline Rare Diseases**

Adam Heathfield, Director of Policy, **Pfizer**

1-2

15.10 Orphan vs. Blockbuster: Issues & Opportunities arising from New Users and Expansion of Therapeutic Uses to a Designated Orphan Drug

During the life-cycle of a novel drug, new medical potential, product uses or patient groups can be identified due to the better understanding of the disease mechanisms, making it possible to expand the use or application of the original drug. This can take blockbuster drugs and redevelop them for an orphan use, or take orphan drugs and redevelop them for additional orphan indications or even in relatively common disorders. At this session, you will hear more about the impact, benefits and issues arising when multiple indications are possible that cross the orphan and non-orphan boundary.

Adam Heathfield
Director of Policy
Pfizer

1-10

15.50 Success Factors to Orphan Drug Development, Product-to-Market & Distribution

- *Early understanding of issues affecting product development of orphan drugs*
- *Business alternatives to maximize your orphan drug distribution reach following post-phase 4 trials*
- *Building an infrastructure to support development, commercialization and distribution of orphan drugs*

Invited Speaker: **SHIRE**

1-2

16.30 30 US vs. European government incentives. Balancing the risks and evaluating your options: The benefits for orphan drug' developers

Listen to Swedish Orphan International AB's journey from a research company to a niche specialty pharma leader in the field of rare diseases, including:

- *The role of US and EU incentives to the continuous development of rare diseases' products commercial portfolio*
- *The impact of orphan drugs determination in the US and Europe*
- *US local pricing & reimbursement procedures vs. European approach: challenges ahead*
- *The Danish and Swedish perspectives towards fast track assessment, market exclusivity, research grants and tax reduction.*

Bo Jesper Hansen
Chairman of the Board
Swedish Orphan Biovitrum AB

17.10 Chairman Closing Remarks
17.20 Dinner Reception

Day 3:

8.30 Coffee & Networking
8.35 Chairman Opening Remark Day 2

1-2

08.40 Identifying opportunities in rare diseases fields to build, shape and grow the orphan drug market: A Case Study

Actelion is a successful biotech group which focuses on treatment for pulmonary arterial hypertension and on the development of therapies for rare diseases. It has executed a global commercial strategy in the orphan drug space including Japan, US and Europe. The commercial strategy is driven by the science based on responsive R&D. Its development pipeline is sound and targeted to build a diverse portfolio development for global markets. Learn how to:

- *Build, shape and grow orphan drug markets: PAH as a case study*
- *Screen the scientific and the technology gaps in rare diseases to identify commercial opportunities*
- *Gain an early understanding of diagnosis, buying processes, competition and other aspects affecting the orphan drug market*
- *Flexible approach to ongoing changes in patient numbers, market prognosis, patient access and clinical trials requirements which affect your drug development and commercial strategies*

Otto Schwarz
President Business Strategy & Operations
Actelion

2-9

09.20 US policy developments and the Brownback/ Brown Amendment and how they impact sponsorship funding

Hear the latest US developments impacting on funding and development of orphan drugs, including:

- *The possible impacts to market exclusivity, patent extensions, patient counts thresholds*
- *The shift in application standards and processes which you need to consider when applying for orphan drug designation and approval*
- *Are the approval procedures going to be simplified?*
- *US developments affecting patient access to orphan drugs*

Emil Kakkis
MD, PhD. & President
Kakkis EveryLife Foundation

10.00 Coffee & Networking Break

10.30 Round Table Discussions

A

Patient-driven development and distribution of orphan drugs: engaging stakeholders to enhance benefits

- *Tackling rare disease challenges through partnering with patent organizations, health care professionals (HCPs), academia and industry*
- *Patient programs: structuring a successful patient program which accounts for and goes beyond individual rare patient needs*

B

Marketing strategies to map and reach the universe of beneficiaries of orphan drugs

- *Setting up effective patient orphan drugs patient and group patient registries to maximize the market reach of your orphan drugs*
- *Maximizing patent network to strengthen your marketing strategies*

C

New approaches to clinical trials to compensate & overcome challenges to market access

- *Building patient partnership schemes to sustain your drug development process and ensure safety*
- *Designing clinical trials to collect measurable data, statistics & evidence-based info to support early market approval*

D

Beyond international initiatives: engaging patient organisations to overcome challenges

- *Understanding the US & European Policy Convergence and Collaboration Initiatives*
- *Establishing platform of collaboration and stakeholder relations with patient organisations to reach the beneficiaries*

Moderator: Sjældne Diagnoser, Birthe Byskov Holm, President

E

Securing capital & investments to orphan drugs development

- *Existing incentives for SMEs companies to invest in drugs for rare and neglected diseases*
- *Comparative analysis of orphan drug designation benefits in the US & Europe*

Orphan Drugs
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BENEFITS OF ATTENDING: KEY ORPHAN DRUGS ISSUES NOT TO MISS

- **Approval processes, pricing and value demonstration to support reimbursement and commercialization of your orphan drugs in the most favorable European markets.**
- **Market access, marketing approval and partnerships to safeguard patient access to orphan drugs globally**
- **Orphan drugs market dynamics: big pharma alliance, partnership and business models to gain new markets and the impact to the existing orphan drugs market praxis.**
- **Improved clinical trials, diagnosis and assessment to ensure safety and speed up patient access to orphan drugs.**
- **EU & US orphan drugs regulations, collaboration initiatives and processes for designation and approval of orphan drugs.**

INVESTMENT DETAILS

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☐ Patient Organisation*: Investment 990 Euro (excl. tax)

**Disclaimer: Discounted rates are only valid for full-time employees at Academia or NPO`s. Discounts may be denied at any time by the organizer if the above given term is not met or can be proven by the applicant/delegate.

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