

Optimising Orphan Drug Development

Tailor a specific approach for orphan drug product development across various rare disease classifications and optimise the commercial aspects

Brussels, Belgium

15th & 16th November 2010

Effectively navigate

the **regulatory** and **reimbursement**

landscapes to gain **better market access**

while optimising the **clinical development**

of your orphan drug



Attending This Premier **marcus evans** Conference Will Enable You to:

- **Explore** the regulatory landscape for orphan drug development
- **Review** the different reimbursement processes in the EU to achieve better market access
- **Overcome** developmental challenges in orphan drugs
- **Examine** the post phase 4 and market authorisation challenges
- **Assess** the opportunities for orphan drug development for big pharma

Hear Cutting-Edge Industry Case Studies:

- **GlaxoSmithKline** explores the opportunities for big pharma to venture into orphan drug development
- **Alaxia** overcomes developmental challenges in developing their cystic fibrosis treatment
- **Orphan Europe** examines the challenges post phase 4 and market authorisation for Carbaglu
- **Shire HGT** discusses how they overcome the delays in reaching the market for greater market access

marcus evans Expert Speaker Panel:

Dr. Hartwig Gajek M.D.
Medical Director Europe, TA
BioTherapeutics
Baxter Innovations

Sonja von Weely
Scientific Officer
Dutch Steering Committee on Orphan Drugs

Philippe Monteyne MD, PhD
Senior Vice President,
Head of Rare Diseases Medicines
Development
GlaxoSmithKline

Erik Tambuyzer
Senior Vice President, Public Policy
Zenzyme

Katia Finck
Director, Market Access and Public
Affairs, Europe
Shire Human Genetics Therapies

Philippe Bordeau
Chief Executive Officer
Alaxia

Carlos Camozzi
Chief Medical Officer
Orphan Europe

Gerard Loeber
Head, Laboratory for Infectious Diseases
and Perinatal Screening
**Dutch National Institute for Public
Health and the Environment (RIVM)**

Annie-Claude Benichou
General Manager
Stragen Services

Jean-Louis Abitbol
Chief Medical Officer
Trophos

Gerard Picot
Head of Global Regulatory Affairs
Ipsen

Jean-Jacques Cassiman
Professor of Forensic Medicine
Center for Human Genetics
University of Leuven

Ed Mascioli, MD
Head of the Orphan and Genetic
Diseases Research Unit
Pfizer

Corinne Duguet
Medical Director
Addmedica

Segolene Ayme
Director of Research
Orphanet

Taha Keilani
Vice President, Clinical Development
Sigma Tau

Harald Fricke
Chief Medical Officer
Apogenix

Media Partner:



Day 1

15th November 2010

08:30 Registration and Coffee

09:00 Opening address from the Chair

EXPLORING THE REIMBURSEMENT ASPECTS FOR GREATER MARKET ACCESS IN ORPHAN DRUGS

09:10 **Overview of different reimbursement processes in the EU to improve the access of orphan drugs for rare diseases patients**

- Understanding different pricing and reimbursement processes for orphan drugs in several EU member states
- Gain an insight into the example of The Netherlands concerning the considerations of the government on reimbursement of orphan drugs

Sonja von Weely

Scientific Officer

Dutch Steering Committee on Orphan Drugs

09:50 **Assessing the different reimbursement processes in the EU to gain better market access for small sized pharma**

- Evaluating the different assessment criteria and procedures for rewarding reimbursement in different countries such as Greece, Belgium, France, UK, Netherlands and Germany
- Discussing the repurposing of existing drugs for orphan indication and the price setting of these
- Analysing the safety and risk management in place

Corinne Duguet

Medical Director

Addmedica

10:30 Morning Coffee

11:00 **How to overcome the delays in reaching the market for greater market access**

- Examining how to secure better patient access
- How can we make the market access process shorter and more efficient?
- How can we generate evidence to convince payers?

Katia Finck

Director, Market Access and Public Affairs, Europe

Shire Human Genetics Therapies

PRACTICAL IMPLEMENTATION TO FURTHER CLINICAL DEVELOPMENT OF ORPHAN DRUGS

11:40 **Reviewing the importance of genetic testing in orphan drug development**

- Assessing the role genetic testing takes in early diagnosis
- Examining the need for pre- and post-natal screening of rare diseases
- Profiling the latest modern techniques in genetic testing

Gerard Loeber

Head, Laboratory for Infectious Diseases and Perinatal Screening

Dutch National Institute for Public Health and the Environment (RIVM)

Business Development Opportunities:

Does your company have solutions or technologies that the conference delegates would benefit from knowing? If so, you can find out more about the exhibiting, networking and branding opportunities available by contacting:

Lysithea Sazon, Sponsorship Manager, **marcus evans** London

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Booking Line

Sean Creech

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12:20 **Case Study:**

Overcoming developmental challenges in developing your Cystic Fibrosis treatment

- Outlining the difficulties in understanding disease pathways to create effective drugs
- Exploring the need for benchmarks in orphan drugs to gain a better understanding of the costs and development times
- Is there scope for targeting orphan drugs even more closely to specific patient populations?

Philippe Bordeau

Chief Executive Officer

Alaxia

13:00 Luncheon

14:10 **Removing barriers in orphan drug development**

- Counteracting the issues of low patient recruitment spread geographically to set up randomised control trials for registration
- Analysing the considerations for safety and efficacy in orphan drug development
- Understanding the need for significant clinical benefit and medical plausibility

Annie-Claude Benichou

General Manager

Stragen Services

14:50 **Case Study:**

Examining the challenges post phase 4 and market authorisation for Carbaglu: Treatment for metabolic diseases

- Discussing the heterogeneity of rare diseases with few patients to carry out epidemiological analysis and how to overcome this
- Outlining the level of commitment and investment required post marketing for better planning

Carlos Camozzi

Chief Medical Officer

Orphan Europe

15:30 Afternoon Tea

PRACTICAL DISEASE CASE STUDIES

16:00 **Case Study:**

Primary immunodeficiency diseases

- Outlining the safety considerations in developing orphan drugs
- Understanding the role of patient organisations from a pharma perspective
- Profiling the post approval investment in registries

Dr. Hartwig Gajek M.D.

Medical Director Europe, TA BioTherapeutics

Baxter Innovations

16:40 **Case Study:**

Eye diseases: Cystinosis

- Profiling the latest clinical developments in orphan drugs for the treatment of these diseases
- Outlining examples of successful drugs in this area
- Evaluating the business model for drug development in this rare disease area

Taha Keilani

Vice President, Clinical Development

Sigma Tau

17:20 Closing Comments from the Chair

17:25 End of Day One

Day 2

16th November 2010

08:30 Registration and Coffee

09:00 Opening Address from the Chair

ASSESSING THE REGULATORY AND POLICY LANDSCAPE FOR ORPHAN DRUGS

- 09:10 **Exploring the regulatory landscape for orphan drug development to anticipate future changes**
- Reviewing the changes in requirement of running small trials and accepted end points
 - Analysing the need for more clinical data to file for designation
 - Examining the scope for simplifying the process to reduce time and costs

Gerard Picot

Head of Global Regulatory Affairs

Ipsen

- 09:50 **Examining European public policy strategies for rare diseases and orphan medicines – Now and the future**
- Profiling the changes in the last 10 years since the introduction of the EU Orphan Medicinal Products Regulation
 - Outlining the national plans under review with a time limit until 2013 and the opportunity for public policy changes
 - Addressing misconceptions about rare diseases and orphan drugs and their regulations
 - Evaluating possible public policy strategies to build on the success of orphan medicines development and what to anticipate

Erik Tambuyzer

Senior Vice President, Public Policy

Genzyme

10:30 Morning Coffee

- 11:00 **Panel Discussion:**
- Evaluating the possibility of a global registry database**
- Reviewing the potential of setting up a global database to speed up patient recruitment and further understanding of these rare diseases
 - Examining the challenges of co-ordination, costs and responsibilities to maintain it
 - Discussing the different possibilities on achieving this

EXPLORING THE ROLE OF GENETICS IN ORPHAN DISEASES

- 11:40 **Evaluating the role of genetics in rare diseases**
- Will the new technologies allow us find more/new targets for genetic tests for many rare diseases
 - The importance of understanding signaling pathways when developing orphan drugs
 - The importance of individual and regional genetic diversity for orphan drugs to act efficiently (personalised medicine)

Jean-Jacques Cassiman

Professor of Forensic Medicine

Center for Human Genetics

University of Leuven

Media Partner:



CheckOrphan is a dynamic and interactive platform dedicated to people affected by rare, orphan and neglected diseases. The goals of CheckOrphan are to educate, unite and empower people affected by rare diseases, to increase communication and understanding, and to hasten the development of treatments and cures.

www.checkorphan.org

Booking Line

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EVALUATING PRIORITY SETTING FOR DEVELOPING ORPHAN DRUGS

12:20 **Panel Discussion:**

Priority setting: develop what orphan drugs for which diseases?

- Discussing the factors behind developing orphan drugs for one disease over another
- Evaluating the issues to consider when priority setting

Erik Tambuyzer

Senior Vice President, Public Policy

Genzyme

Segolene Ayme

Director of Research

Orphanet

Philippe Monteyne MD, PhD

Senior Vice President,

Head of Rare Diseases Medicines Development

GlaxoSmithKline

Philippe Bordeau

Chief Executive Officer

Alaxia

13:00 Luncheon

FUTURE OPPORTUNITIES TO EXPLOIT WITHIN ORPHAN DRUGS

14:00 **Case Study:**

Identifying the best practices in developing an orphan drug portfolio and pipeline

- Profiling Pfizer's current orphan drug portfolio and pipeline
- Outlining the Orphan and Genetic Diseases Research Unit's strategy for adding assets to Pfizer's pipeline
- Discussing the cultural changes within pharma required to accommodate orphan therapeutics

Ed Mascioli, MD

Head of the Orphan and Genetic Diseases Research Unit

Pfizer

14:40 **Case Study:**

Exploring the opportunities for big pharma to venture into orphan drug development

- Reviewing the reasons behind the recent interest from big pharma to develop orphan drugs
- Outlining the progress and developments in big pharma
- What to expect in the future?

Philippe Monteyne MD, PhD

Senior Vice President,

Head of Rare Diseases Medicines Development

GlaxoSmithKline

15:20 **Case Study:**

Assessing how to maximise protection of your drug by applying for orphan drug designation

- Examining how to identify your drug as having an orphan indication and the modality of action
- Analysing the pros and cons of applying for orphan drug designation
- Profiling Apogenix's three successful designations and the progress so far

Harald Fricke

Chief Medical Officer

Apogenix

16:00 Closing Comments from the Chair

16:05 End of Conference

Optimising Orphan Drug Development

LS100

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PAYMENT IS REQUIRED WITHIN 5 WORKING DAYS.

A 24% service charge has been added to all conference fees prior to the event and is inclusive of programme materials, luncheon and refreshments. Credit card payment will be processed in SEK.

Registration Details

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DATES: 15TH & 16TH NOVEMBER 2010

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