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Orphan Drugs and Rare Diseases

Discuss the latest regulatory developments, explore how to reduce costs and learn from the latest innovations in the orphan drug landscape

HOLIDAY INN KENSINGTON FORUM, LONDON, UK

WORKSHOP: 18TH
CONFERENCE: 19TH - 20TH
OCT 2016

CHAIR FOR 2016:

- **Tim Miller,** President & CEO, **Abeona Therapeutics Inc**

FEATURED SPEAKERS:

- **Sheela Upadhyaya,** Associate Director Highly Specialised Technologies, **National Institute for Health and Care Excellence**
- **Cécile De Coster,** Associate Director, Regulatory Affairs, **Alexion Pharma GmbH**
- **Tony Hall,** Therapeutic Area Head Orphan Drugs, **Mereo BioPharma**
- **Alex Bloom,** Director of Regulatory Affairs, **Cell Medica**
- **James McArthur,** Chief Scientific Officer and Co-Founder, **Cydan Development Inc**
- **Anders Waas,** Chief Executive Officer, **Tikomed**

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KEY SESSIONS:

- Regulatory challenges for **orphan products:** Focus on **emerging markets**
- Development of orphan drugs to prevent, diagnose and **treat rare diseases**
- Challenges with **paediatric orphan drug development**
- Orphan drug industry and venture capital – key factors for a successful partnership

PLUS TWO INTERACTIVE HALF-DAY PRE-CONFERENCE WORKSHOPS
Tuesday 18th October 2016, Holiday Inn Kensington Forum, London, UK

WORKSHOP A

Planning for success: Developing the optimal orphan drug development strategy

08.30 – 12.30

Workshop Leaders:
Alex Bloom, Director of Regulatory Affairs, **Cell Medica**
Diego Ardigò, Project Leader Advanced Therapies, **Chiesi Farmaceutici S.p.A.**

WORKSHOP B

Paving the way for achieving orphan drug market access

13.30 – 17.30

Workshop Leaders:
Ad Rietveld, Director, **RJW & Partners**
John Spoors, Senior Consultant, **RJW & Partners**

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Orphan Drugs

Day One | Wednesday 19th October 2016

08.30 **Registration & Coffee**

09.00 **Chairman's Opening Remarks**
Tim Miller, President & CEO, **Abeona Therapeutics Inc**

REGULATORY UPDATES

09.10 **OPENING ADDRESS**
Market access for orphan drugs
- A brief outline of the NICE Highly Specialised Technology (HST) Evaluation process
- Topic selection and NICE HST prioritisation criteria
- Working with stakeholders, including patient support organisations

Sheela Upadhyaya, Associate Director Highly Specialised Technologies, **National Institute for Health and Care Excellence**

09.50 **Regulatory challenges for orphan products: Focus on emerging markets**
- Measuring the differences in regulations cross border and how the impact this has
- Analysing how these differences influence patient's access to drugs
- What can be done to ensure the integrity of these standards?

Alex Bloom, Director of Regulatory Affairs, **Cell Medica**

10.30 **Morning Coffee**

DEVELOPMENT OF ORPHAN DRUGS

10.50 **A new business model for developing rare diseases treatments**
- The Mereo BioPharma business model
  - acquisition of assets
  - funding the programs
  - what has been achieved to date
- Case study: Osteogenesis imperfecta
  - the need for an effective therapy in OI
  - anti-sclerostin antibody as a potential treatment for OI
  - the pathway to registration

Tony Hall, Therapeutic Area Head Orphan Drugs, **Mereo BioPharma**

11.30 **Development of orphan drugs to prevent, diagnose and treat rare diseases**
- Reviewing the challenges of orphan drugs development and creating workable solutions of to overcome them
- How to establish early access to orphan drugs
- Analysing what regulatory bodies can do to support companies investing in these drugs

Tim Miller, President & CEO, **Abeona Therapeutics Inc**

12.10 **Networking Lunch**

13.30 **KEYNOTE ADDRESS**
Challenges with paediatric orphan drug development
- Complex Issues in developing drugs and biological products for rare diseases
- How to accelerate the development of therapies for paediatric rare diseases
- Methods to overcome the challenges of trial design

Cécile De Coster, Associate Director, Regulatory Affairs, **Alexion Pharma GmbH**

14.10 **Orphan drug industry and venture capital – key factors for a successful partnership**
- Investment in orphan drugs industry is a very attractive fit for venture capital
- What venture capital is looking for in orphan drug Industry
- Key factors you should to consider when searching for venture capital

Robert Karl, Partner, **RBV Capital**

14.50 **Afternoon Tea**

15.20 **A case study of patient foundations and industry working together**
- Education is key and it's a two way street
- Working together at all stages, ensuring effective collaboration
- Providing consistent and accurate information

Michelle Berg, Vice President, Patient Advocacy, **Abeona Therapeutics, Inc.**

16.00 **A patient group's perspective on patient recruitment and retention**
- Patient recruitment in Europe
- How to maximise patient retention
- Why patient groups can be an effective may to manage the patient input of clinical trials

Oliver Timmis, CEO, **AKU Society**

16.40 **Chairman's Closing Remarks and Close of Day One**
Tim Miller, President & CEO, **Abeona Therapeutics Inc**

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08.30 **Registration & Coffee**

09.00 **Chairman's Opening Remarks**
Tim Miller, President & CEO, **Abeona Therapeutics Inc**

09.10 **OPENING ADDRESS**
Building a patient centric model for rare disease drug discovery
- Role of patient groups in drug development
- Applying artificial intelligence to drug repurposing
- Supporting non-for-profit drug discovery

Tim Guilliams, Chief Executive, **Healx**

THE PRICING MECHANISM

09.50 **Rare disease clinical research: Collaboration the key to success**
- Focus on the patient experience and early engagement of patients and their advocates drives data quality and access to novel therapies
- Partnership with quality and experienced sites, leveraging their clinical expertise while providing operational support ensures quality despite complexity
- groups, specialised sites, medical and scientific leaders, and operational experts in rare diseases accelerates drug development and patient access to medicines in rare diseases

Judith Ng-Cashin, Chief Scientific Officer, **INC Research**

10.30 **Morning Coffee**

10.50 **The challenges in defending orphan products pricing**
- Pricing models for orphan drugs
- The changing environment for contracting options
- Increasing the likelihood of acceptance of contracting options by payers

Ad Rietveld, Director, **RJW & Partners**

11.30 **Setting reimbursement strategies - How to establish a foundation for reimbursement**
- Analysing how payers are considering reimbursing drugs that are coming on the market for orphan drugs and rare diseases
- Can workable changes be made to improve current reimbursement strategies?
- Creating a collaborative reimbursement strategy that satisfies and benefits all parties involved
- Taking a look at regulatory frameworks and how they influence the reimbursement process

Nigel Nicholls, Country Director UK/Ireland, **BioMarin**

12.10 **Networking Lunch**

A CLOSER LOOK AT RARE DISEASES

13.30 **Advancing therapies for Duchenne Muscular Dystrophy (DMD)**
- Utrophin modulation has the potential to treat all boys and young men with DMD, regardless of their underlying dystrophin gene mutation
- PhaseOut DMD, a Phase 2 trial of lead utrophin modulator, ezutromid, expected to report 24 week biopsy data from initial group of patients in January 2017 – could provide first signs of proof of mechanism
- Mechanism of action and results to date

A senior representative from Summit Therapeutics

14.10 **The role of patient associations and how they are helping to fund the rare diseases field**
- How do patient associations contribute to the funding of orphan drugs companies today?
- What are the pros and cos of patient organisations directly contributing to the funding of orphan drugs companies
- The role of patient organiSations in promoting drug development for their disease
- What lessons can be learnt?

Diana Ribeiro, CEO, **Action Duchenne**

14.50 **Afternoon Tea**

15.20 **How can partnerships improve the Orphan Drug field?**
- How to make a multi-stakeholder partnership successful?
- What role does each stakeholder play?
- How can collaboration/partnership improve orphan drugs access to market?

Panelists:
Tim Miller, President & CEO, **Abeona Therapeutics Inc**
Nigel Nicholls, Country Director UK/Ireland, **BioMarin**
Robert Karl, Partner, **RBV Capital**
Michelle Berg, Vice President, Patient Advocacy, **Abeona Therapeutics, Inc.**

16.00 **Opportunities and challenges for biotech company to develop orphan drugs**
- Strategy and tactics on the way – building a pipeline
- Effective drug development and research
- Partnering focus and timing of deals

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

16.40 **Chairman's Closing Remarks and Close of Day Two**
Tim Miller, President & CEO, **Abeona Therapeutics Inc**

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HALF-DAY PRE-CONFERENCE WORKSHOP
Tuesday 18th October 2016
08.30 – 12.30
Holiday Inn Kensington Forum, London, UK

Planning for success: Developing the optimal orphan drug development strategy

Workshop Leaders:
Alex Bloom, Director of Regulatory Affairs, **Cell Medica**
Diego Ardigò, Project Leader Advanced Therapies,
Chiesi Farmaceutici S.p.A.

Overview of Workshop:

With an estimated 8,000 rare diseases identified and worldwide sales expected to exceed $175bn by 2020, commercialising new orphan medicines remains highly attractive to the pharmaceutical industry, although greater regulatory and market access challenges are to be overcome for a product to be successful.

Developing medicines for rare diseases give rise to unique challenges, which often require creative solutions and innovative development strategies to overcome.

This workshop will look at best practices for the development of orphan medicines, how to avoid common pitfalls and how to "plan for success".

Programme:

08.30 **Registration and Coffee**

09.00 **Strategy and profiling: laying the foundations for success**
- The importance of a target product profile
- Understanding your regulatory environment
- Knowing your patient population: the value of natural history studies and patient registries

11.00 **Morning Coffee**

11.30 **Development, approval and marketing**
- Clinical development in small populations: opportunities and limitations
- Expedited development pathways and early access schemes
- Developing a market access strategy and the value of real world data

12.30 **End of workshop**

About the Workshop Leaders:

Dr Bloom acts as Director of Regulatory Affairs at Cell Medica (UK), a niche biopharmaceutical company specialising in the development of cell therapies to treat rare diseases. He also acts as Principal Consultant for Aceso Consulting Ltd, offering strategic and regulatory support to the biopharmaceutical industry. In the past ten years, he has worked on multiple rare disease programs, including the oversight of more than a dozen orphan drug applications in the EU and US.

Dr. Ardigò received his degree in Medicine and the specialization in Internal Medicine at the University of Parma (Italy). After a post-doctoral fellowship at Stanford University (California, US) he obtained a PhD degree in Cardiovascular Pathophysiology at the University of Parma. He joined Chiesi in 2010 where acted as Clinical Lead in the development and registration of the first stem cell therapy in the EU (Holoclar®) and is leading the cross-company team (in alliance with uniQure BV) that led to the treatment of the first commercial patient with a gene therapy in EU.

About the Organisation:

Cell Medica is committed to improving patients' lives through the significant therapeutic potential of cellular immunotherapy. Our approach is to apply innovative technologies with the aim of improving the treatment of cancer and immune reconstitution following hematopoietic stem cell transplant. We have developed a business platform that has positioned Cell Medica as a leader in the manufacture, development and commercialization of cellular products.

Chiesi Farmaceutici (www.chiesi.com) is a research-focused international group, headquartered in Parma (Italy). Chiesi researches, develops and commercializes innovative pharmaceutical solutions in the respiratory, specialist medicine, and rare diseases areas. In 2008, Chiesi launched the spin-off company Holostem, dedicated to the development of tissue engineered and gene therapy products. In 2014, the first advanced therapy candidate was approved by EMA, becoming the first medicinal product containing stem cells in EU.

HALF-DAY PRE-CONFERENCE WORKSHOP
Tuesday 18th October 2016
13.30 – 17.30
Holiday Inn Kensington Forum, London, UK

Paving the way for achieving orphan drug market access

Workshop Leaders:
Ad Rietveld, Director, **RJW & Partners**
John Spoors, Senior Consultant, **RJW & Partners**

Overview of Workshop:

Orphan products are coming to the market at an accelerating pace. Although in principle welcoming the introduction of products for diseases for which no treatments were available before, payers are still contemplating whether the influx of orphan products will lead to unsustainable cost increases and how keep these costs down. The perceived high pricing of orphan drugs is clearly a focus and payers are looking for ways to ascertain that prices paid are reasonable and sustainable. At the same time, manufacturers are seeking news ways of pricing orphan drugs so they can recoup their development costs and generate a profit whilst not creating barriers to the funding of orphan drugs by payers. The workshop will examine the current state of affairs and take a look into what the future may bring with a focus on the changing payer environment with a focus on pricing models that could be applied to pursusade payers to fund new drugs.

Programme:

13.30 **Registration and Coffee**

14.00 **Opening remarks and introductions**

14.10 **Session 1: Orphan Drugs: Why is regulatory orphan drug status still not enough to achieve market access at acceptable prices?**
- Orphan drugs – do payers think a success from a quality of healthcare perspective?
- Outline of the pricing, reimbursement and access process
- The real hurdles to orphan drug market access

14.50 **Session 2: The payer perspective: Orphan products are what we've been waiting for!**
- Growing demand and rising expenditure
- The impact of geography – Europe vs US
- Payer attitudes to orphan drugs
- Payer developments with respect to orphan drugs

15.30 **Afternoon Tea**

16.00 **Session 3: How to ensure that evidence for orphan drugs is relevant to the payer**
- Expectations of evidence in support of pricing and access
- Are orphan drugs really a special case when it comes to evidence?
- How payers look at value - it's not all about price, cost and cost-effectiveness

17.30 **End of workshop**

About the Workshop Leaders:

Ad Rietveld is a former GP with marketing experience in the industry (Solvay) and extensive consulting experience in pricing and market access (Cambridge/IMS). Ad was a former national payer in the Dutch Ministry of Health and has been a Consultant to World Bank, EU and WHO, advising countries on how to build their pricing and reimbursement systems. In 2008, Ad co-founded RJW & partners Ltd, a consultancy that provides pricing and market access services to pharmaceutical and medical device companies.

John Spoors has over 10 years experience in the industry and in consulting. He joined RJW & partners in 2012 and prior to that was with Helen Johnson Consulting Limited (HJCL) as an Account Executive and subsequently as Policy and Market Access Manager. In his consultancy career, he has worked for a number of leading pharmaceutical and patient organisations across a broad range of therapy areas including diabetes, rheumatology and oncology. By combining politics, science and economics has was able to offer his clients a tailored service that included Health Technology Appraisal (HTA) advice, tools and project management; he also designed and implemented a HTA monitoring service for a number of major pharmaceutical companies. Before joining HJCL, John was employed as Policy and Market Access Executive at Merck, Sharp and Dohme where he was responsible for project managing the company's HTA programme for diabetes, HIV and oncology, and assisting with policy and public affairs campaigns. He also managed European and Global HTA projects. John has an academic background in politics and economics/natural sciences.

About the Organisation:

RJW & partners Ltd is a UK-based Consultancy with presence in Europe, US and Australia. RJW & partners provide strategic pricing and market access advice to pharmaceutical and medical device companies of all sizes. RJW

ORPHAN DRUGS

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Workshop: Tuesday 18th October 2016, London, UK

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