

SMi presents its 4th annual conference on...

Orphan Drugs and Rare Diseases

Reviewing strategies to increase rare disease research
and enhance the development of new treatments

Holiday Inn Regents Park, London UK

19th - 20th
OCT
2015

Benefits of attending:

- Learn about **key strategies** and collaborations to accelerate **rare disease clinical drug development**
- **Understand** how patients and families are **key team players** in improving therapy development
- **Discover** how gene therapy is a very real factor in clinical trials
- Review how **big pharma** is positioning itself in the emerging orphan drug industry
- **Discuss academic** and **big pharma** perspectives on how to overcome the challenges of rare diseases

Chairs for 2015:

- **Dr Alastair Kent OBE**, Director, **Genetic Alliance UK**, Chair, **Rare Disease UK (RDUK)**
- **Dr Nicolas Sireau**, Chairman & CEO, **AKU Society**



Key speakers include:

- **Dr Tim Miller**, President & CEO, **Abeona Therapeutics**
- **Dr Anne Marquet**, Principal Clinical Scientist, Rare Diseases, **Roche Pharma Research and Early Development**
- **Dr Carlos R. Camozzi**, Chief Medical Officer, **Orphazyme**
- **Michael Skynner**, Head of Rare Disease External Alliances, **Pfizer**
- **David Boothe**, Global Commercial Leader – GSK Rare Diseases, **GSK**

PLUS TWO INTERACTIVE HALF-DAY POST-CONFERENCE WORKSHOPS Wednesday 21st October 2015, Holiday Inn Regents Park, London UK

A: Market access to orphan drugs: Controversies; trends and solutions

Workshop Leader:
Colette Hamilton, Managing Director, **ATP Market Access**
8.30am – 12.30pm

B: The rare disease patient perspective – from regulatory to clinical execution

Workshop Leaders:
Christa van Kan, Team Lead Clinical Execution, **PSR Orphan Experts**
Steve Pinder, PhD Director, **Investia Ltd**
Nicolas Sireau, PhD, Chairman and CEO, **AKU Society**
1.30pm – 5.30pm

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8.30 Registration & Coffee

9.00 Chair's Opening Remarks

Dr Nicolas Sireau, Chairman & CEO, AKU Society

PRICING, REIMBURSEMENT & COSTS

OPENING ADDRESS

9.10 Challenges for reimbursement of orphan and ultra orphan and the shifting UK payer landscape

- Routes to reimbursement
 - NICE Highly Specialised Technologies programme
- Josie Godfrey**, Associate Director, Highly Specialised Technologies, **National Institute for Health and Care Excellence (NICE)**, UK

KEYNOTE ADDRESS

9.50 Patient organisations working collaboratively with the challenges of reimbursement from a patient perspective

- A collaborative approach
- Working in partnership with industry
- Reimbursement and access to high cost therapies

Christine Lavary, Chief Executive, **MPS Society UK**

10.30 Morning Coffee

11.00 Cost of illness analysis in rare diseases: cystic fibrosis as a model

Professor Milan Macek, Head of Department, Department of Biology and Medical Genetics, National Coordination Centre for Rare Diseases, National Center for Cystic Fibrosis, **Charles University Prague**

MARKET ACCESS

11.40 DevelopAKUre - a patient-led clinical trial

- How to work closely with patients to develop a clinical trial programme
- Strategies for recruiting patients for successful trials
- Using social media in clinical trials
- How to put together successful collaborations

Dr Nicolas Sireau, Chairman and CEO, **AKU Society**

12.20 Networking Lunch

1.20 Meeting the challenges of rare diseases - a big pharma perspective

- Listening to patients, care givers and patient groups
- Creative development program design
- Open and early engagement with regulators

David Boothe, Global Commercial Leader – GSK Rare Diseases, **GSK**

2.00 The academics' perspectives

- Engaging academic institutions to contribute external expertise to overcome development hurdles
- Drivers for academics to participate in development programs
- Key elements of partnering

Dr Joanna Cox, Head of Business Partnerships, **Cranfield University**

2.40 Importance of collaboration in rare disease research

- Working in rare disease research presents specific challenges
- One approach to address these challenges is through collaboration
- Pfizer's collaborative approaches in the area of drug discovery

Michael Skynner, Head of Rare Disease Alliances, Rare Disease Research Unit, **Pfizer**

3.10 Afternoon Tea

GENE THERAPIES

3.40 Developing gene therapy for orphan disease indications - a perspective from academia

- Gene therapy shows significant efficacy in a number of inherited bone marrow disorders
- These are possible curative genetic medicines for otherwise untreatable diseases
- The move beyond academic clinical trials is still difficult and not yet well charted
- There is increasing interest from industry but the commercial case for rare diseases remains difficult

Professor Bobby Gaspar, Professor of Paediatrics and Immunology, Consultant in Paediatric Immunology, Head, Rare Diseases Theme Head, **University College London, Institute of Child Health**

4.20 Gene therapy for rare diseases – what we can expect in 2016

- Multiple groups moving rare disease gene therapies into Phase I and II clinical trials
- The gene therapy bubble – 2013-2015 saw large investments in gene therapy companies
- Manufacturing – the next big hurdle on the path to commercialization

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

5.00 Is global collaboration the answer to reaching all potential patients?

- Is this the way forward for reaching patients and developing and new treatments?
- What resources would be required?
- How would this be coordinated?

Dr Nicolas Sireau, Chairman & CEO, **AKU Society**

Professor Bobby Gaspar, Professor of Paediatrics and Immunology, Consultant in Paediatric Immunology, Head, Rare Diseases Theme Head, **University College London, Institute of Child Health**

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

Dr Robert Ryan, President & Chief Executive Officer, **Scioderm, Inc.**



5.40 Chairman's Closing Remarks and Close of Day One

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8.30 Registration & Coffee

9.00 Chairman's Opening Remarks

Alastair Kent OBE, Director, Genetic Alliance UK, Chair, Rare Disease UK (RDUK)

9.10 Putting patients on the team to improve therapy development

- Adaptive Pathways and other innovations will require real world evidence collection
- Patients and families have views on what matters that need to be included
- The benefit: Risk estimation cannot be undertaken effectively without patient input
- Demonstrating value and clinical benefit relies on patient and family experiences being systematically incorporated into MA and HTA processes

Alastair Kent OBE, Director, Genetic Alliance UK, Chair, Rare Disease UK (RDUK)

KEYNOTE ADDRESS

DRUG DEVELOPMENTS & COMMERCIALISATION

9.50 Big pharma and the orphan drug industry

- Analysing the role of big pharma in the emerging orphan drug industry
- Where is their focus going and what does this mean for the industry long term
- What is their current perspective and what is the next step?

Dr Carlos R. Camozzi, Chief Medical Officer, Orphazyme

10.30 Morning Coffee

11.00 Development of an orally available SMN2 splicing modifier for the treatment of SMA: An example of a development program in rare diseases

- An accelerated preclinical and development program
- Coping with heterogeneity in rare diseases
- A unique alliance with a focus on patients

Dr Anne Marquet, Principal Clinical Scientist, Rare Diseases, Roche Pharma Research and Early Development

11.40 The complexity and peculiarities of developing and commercialising advance therapies (ATMPs) for the treatment of rare diseases

- The complexity of translating "traditional" pharma development into ATMP's...
 - when pharmacology (primary, secondary, safety), toxicology, DMPK, and carcinogenicity are "not applicable" and phase I simply not existing
 - when late stage clinical studies inevitably carry over development risks from untraditional CMC, pre-clinical, and early clinical stages
- Understanding the complexity ... of logistics in ATMPs with short shelf-life, requiring delicate transportation conditions, or manufactured/ packaged for an individual patient
- The complexity ... of market access and pricing for treatments to be administered once in a lifetime
- The return-of-investment in the orphan ATMP space

Dr Diego Ardigo, ATMP Project Leader, Corporate Drug Development, R&D, Chiesi Farmaceutici S.p.A.

12.20 Networking Lunch

1.20 Zorblisa™ (SD-101): A topical therapy in phase 3 for treating the skin effects associated with epidermolysis bullosa (eb)

CASE STUDY

- Epidermolysis bullosa (EB) is a rare genetic disease that typically manifests at birth or early childhood leading to fragile skin susceptible to blistering and tearing
- There are many genetic and symptomatic variations of EB, but all forms share the common symptom of fragile skin that blisters and tears from the slightest friction or trauma
- EB is chronic, debilitating and potentially disfiguring, and patients with EB can have painful wounds and blisters affecting a substantial percentage of their bodies
- There is currently no effective or approved treatment for EB. Current standard of care consists solely of bandaging and wound management to prevent infection and manage patients' pain
- The Pivotal Phase 3 registration trial is ongoing in the US and EU to evaluate the safety and efficacy of Zorblisa (SD-101), a proprietary and novel topical therapy for the treatment of patients across all subtypes of Epidermolysis Bullosa (EB)

Dr Robert Ryan, President & Chief Executive Officer, Scioderm, Inc.

2.00 The Future of Orphan Drugs: Creating an orphan market for a sustainable future

PANEL DISCUSSION

- Discussing whether the market sustainable
- How can we prepare for the future, what future steps are needed

Josie Godfrey, Associate Director Highly Specialised Technologies and Topic Selection, National Institute for Health and Care Excellence (NICE)

Dr Tim Miller, President & CEO, Abeona Therapeutics



2.40 Afternoon Tea

3.10 Development of a drug for GM1-gangliosidosis by a non-profit foundation

- Discovery phase: Working with academic laboratories and spin-off of a commercial entity
- Preclinical development: Outsourcing to commercial service providers vs. academic laboratories
- Clinical development: Selecting the right partners for a micro-company
- Financing the development of a drug for a rare disease

Dr Stephane Demotz, Founder, Dorphan SA, Switzerland

ULTRA-RARE DISEASE

3.50 Patient-empowered pharmaceutical R & D. Building an orphan and ultra-orphan gene therapy pipeline, from research to patient access or marketing authorisation

- The role of patients association in the rare disease field
- The development of an ATPM (gene therapy) as an orphan drug

Dr Didier Caizergues, Head of regulatory Affairs Department, International Regulatory Affairs Department, Genethon

4.30 The real story about rare and ultra-rare disease

CASE STUDY

- A story that needs to be told by people who are living the nightmare of rare and ultra-rare diseases
- A story of how and why the Foundation was put in place

Terence Hoey, CEO, The Ultra Rare Diseases, Disorder & Disabilities Foundation

5.10 Chairman's Closing Remarks and Close of Day Two

Supported by



Market access to orphan drugs: controversies; trends and solutions

Overview of workshop:

Working through case studies and drawing on the experience of participants, the workshop will explore trends in policy and the reimbursement of orphan drugs and the implications for industry; doctors and patients. The emphasis will be on generating solutions that can both address market conditions and drive patient access to effective orphan drugs.

Benefits of attending:

Keeping people affected by rare diseases at the heart, the workshop will promote sharing of up to the minute information, market trends, payer insights and best practise to support your effective cross functional market entry and strategic market planning. The content will be kept iterative in order to address the specific requirements of participants. It will include new insights on pricing for access and on how to develop a stronger payer value story.

Programme:

8.30 Registration and Coffee

9.00 Opening remarks and introductions

9.10 The Pricing Controversy

- Price vs cost – payer perspectives
- Label creep
- The relationship between pricing policy and patient access
- Case studies
- Attaining WIN-WIN

9.50 Payor Trends and Implications

- Trends in payer policy and coverage
- Trends in HTA evaluation
- Understanding conditions for reimbursement
- Implications for industry

10.30 Coffee

11.00 Redefining Value

- Evolving understanding of benefit versus cost
- QALY limitations and alternatives
- Adoption of multi-criteria decision analysis
- Solutions that work for patients, doctors, industry and payers

11.40 Evidence Generation

- Addressing challenges in demonstrating safety and efficacy when working with low patient numbers
- Creative ways to assimilate data
- The opportunities and limitations of real world evidence
- Evidence generation solutions for orphan drugs

12.20 Closing remarks

12.30 End of workshop

About the workshop leader:

Colette is a global market access, reimbursement and health economics specialist, with a proven track record in extending the reach of health transforming technologies to patients. Before transitioning to healthcare industry in 2002, she was an international healthcare advisor with PwC following eleven years of health services management experience in the UK. She has successfully delivered reimbursement projects in North America; Asia Pacific; Europe and Africa. She is experienced in working with orphan drugs, in particular, with applications in immunology and cancer treatment. Colette has a clinically qualified, with an MBA and an MSc in Psychological Studies. Colette is also Vice President of Health Economics and Reimbursement for Evidence for Access, a new venture with Tech Mahindra, a multi-billion pound technology company, providing end to end real world evidence generation solutions for pharma and medical device companies

About the organisation:

ATP Market Access, the business name of ATP Consulting Ltd., is a niche consultancy practise, founded in 2005 to deliver healthcare transformation by accelerating access to value adding health technologies.

The rare disease patient perspective – from regulatory to clinical execution

Overview of workshop:

This workshop will provide insights into the orphan drug regulatory landscape towards clinical execution and its accompanying challenges, with emphasis on the rare disease patient's perspective. It includes insights into the current possibilities and challenges within the regulatory framework plus the necessary requirements to transform the regulatory strategy into feasible clinical development planning. Furthermore, the crucial role of the patient and the patient advocacy groups in all aspects of Clinical Development Planning and the organisational and practical challenges during execution of the clinical trials will be considered.

Benefits of attending:

- To get insight into the overall process from the regulatory perspective (e.g. orphan drug designation) towards running clinical studies in rare disease patients.
- To successfully transform regulatory strategies into practical clinical development planning and subsequently running successful clinical trials in rare disease indications.
- To understand how to collaborate with patient advocacy groups in the most successful way for your orphan drug development program.
- To learn the importance of potential strategies to lower patient burden and thereby increase patient recruitment and retention in rare disease studies and thus benefit overall study success rate.

Programme:

- 1.30 Registration and Coffee**
- 2.00 Opening remarks and introductions**
- 2.10 Regulatory framework & how to successfully transform regulatory strategies into practical clinical development planning (Steve Pinder)**
 - Brief outline of Orphan Drug regulations
 - Common misinterpretations of orphan drug legislations
 - Regulatory strategies vs Clinical development strategies
- 3.30 Coffee**
- 3.45 The crucial role of patient advocacy groups in a successful orphan drug development program (Nick Sireau)**
 - Public/private funding & crowd funding
 - Role of patient advocacy groups in rare disease clinical trials
- 4.30 Lowering patient burden & practical / logistical challenges to ensure successful clinical study execution (Christa van Kan)**
 - Patient recruitment & retention strategies
 - Lowering patient burden
 - Logistical / practical challenges
- 5.15 Closing remarks**
- 5.30 End of workshop**

About the workshop leaders:

Christa van Kan has more than 20 years experience in managing clinical trials in a broad range of diseases, including many orphan and paediatric indications. Christa has developed several innovative recruitment & retention strategies, specifically focussed on Orphan Diseases.

Dr Nicolas Sireau is Chairman and CEO of the AKU Society, a medical charity that works to find a cure for and support patients with AKU (also called Black Bone Disease), an ultra rare disease that affects his two sons (www.akusociety.org).

Steve Pinder In 2007 Steve founded Envestia Ltd with business partner Dr Ian Dews, a pharmaceutical physician and former full-time clinical investigator. Steve is one of the regulatory consultants for PSR Orphan Experts and as such been involved in a variety of regulatory projects for orphan drugs.

ORPHAN DRUGS AND RARE DISEASES

Conference: 19th -20th October 2015, Holiday Inn Regents Park, London UK | Workshops: 21st October 2015, London

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