

29 JUNE - 1 JULY 2015

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Paradigm Global Events

Orphan Drugs & Rare Diseases Global Congress 2015 Europe

Steering the Growth & Sustainability of the Industry

Featuring Key Industry Experts

Dr Ségolène Aymé, Emeritus Research Director, INSERM, FRANCE

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

Dr. Chia Wen Lee, Head of Emerging Markets - Corporate Market Access, BOEHRINGER INGELHEIM, GERMANY

Dr. David King, Global Regulatory Intelligence Director - Regulatory Policies & Intelligence, NOVO NORDISK, DENMARK

Barbara McLaughlan, Head of External Affairs – Oncology, NOVARTIS, UK

Dr. Carlos R. Camozzi, Chief Medical Officer, ORPHAZYME, DENMARK

Dr. John ZL Gong, Chief Executive Officer, 3D MEDICINES, CHINA

Josie Godfrey, Associate Director - Highly Specialised Technologies, NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE (NICE), UK

M. (Ken) Kengatharan, PhD. MBA., President & Chief Operating Officer, ARMETHEON, INC., USA

Dr. Hartwig Gajek, Managing Director, HESYRA-MEDICAL GMBH, SWITZERLAND

Dr Joanna Cox, Research and Business Development Manager, ROYAL HOLLOWAY, UNIVERSITY OF LONDON, UK

Prof George Dickson, Chair of Molecular Cell Biology, School of Biological Sciences, ROYAL HOLLOWAY, UNIVERSITY OF LONDON, UK

Miriam Gargesi, EuropaBio Healthcare Director, EUROPABIO, BELGIUM

Prof Bobby Gaspar, Professor of Paediatrics & Immunology, INSTITUTE OF CHILD HEALTH - U.C.L., UK

Christian Girard, Chief Editor, ORPHAN DRUGS INDUSTRY INSIDER & CEO, ABCROWDFUNDING ADVISORS, FRANCE

Christine Lavary, Chief Executive, MPS SOCIETY, UK

Didier Caizergues, Head of regulatory Affairs Department, GENETHON, FRANCE

Mark Corbett, Vice President, Clinigen Global Access Programs (GAP) – Clinigen

Dr Nicolas Sireau, Chairman and CEO, AKU Society

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ORPHAN DRUGS & RARE DISEASES GLOBAL CONGRESS 2015 EUROPE

STEERING THE GROWTH & SUSTAINABILITY OF THE INDUSTRY

29 June - 1 July 2015 • Hilton London Kensington Hotel • London • UK •

Over 7,000 different types of rare diseases and disorders exist, with more being discovered each day, and large pharmaceutical and biotech companies are structuring to participate and leverage the growing orphan market. "Orphan drug sales will make up 19% of the total share of prescription drug sales by 2020, totaling \$176 billion. And they'll grow at an annual rate of nearly 11% per year through the end of the decade, compared with about 4% for drugs treating larger populations." (EvaluatePharma's Orphan Drug Report 2014)

Orphan Drugs & Rare Diseases Global Congress 2015 Europe provides a unique platform for business knowledge sharing and convergence of top tier government, hospitals, pharmaceuticals, biopharmaceuticals, non-profit organisations, orphan drugs developers as well as regional and local manufacturers to discuss the driving macroeconomic factors, policies and issues that will steer the development of orphan drugs globally.

INDUSTRY FACTS

- There are approximately 7,000 different types of rare diseases and disorders, with more being discovered each day
- Approximately 50% of the people affected by rare diseases are children
- 30% of children with rare disease will not live to see their 5th birthday
- Rare diseases are responsible for 35% of deaths in the first year of life
- Approximately 50% of rare diseases do not have a disease specific foundation supporting or researching their rare disease

WHO WILL YOU MEET

Presidents, Heads/Chiefs, Directors, VPs and Managers of:

- Commercialisation
- Regulatory Affairs
- Market Access
- Sales & Marketing
- Research & Development

Orphan Drugs Manufacturers - 20%

Big Pharmas - 20%

Biopharmaceuticals - 20%

Governments (Ministries of Health/Drug Authorities) - 10%

Regional & Local Contract Manufacturers - 10%

Non Governmental Organisations (NGOs) - 5%

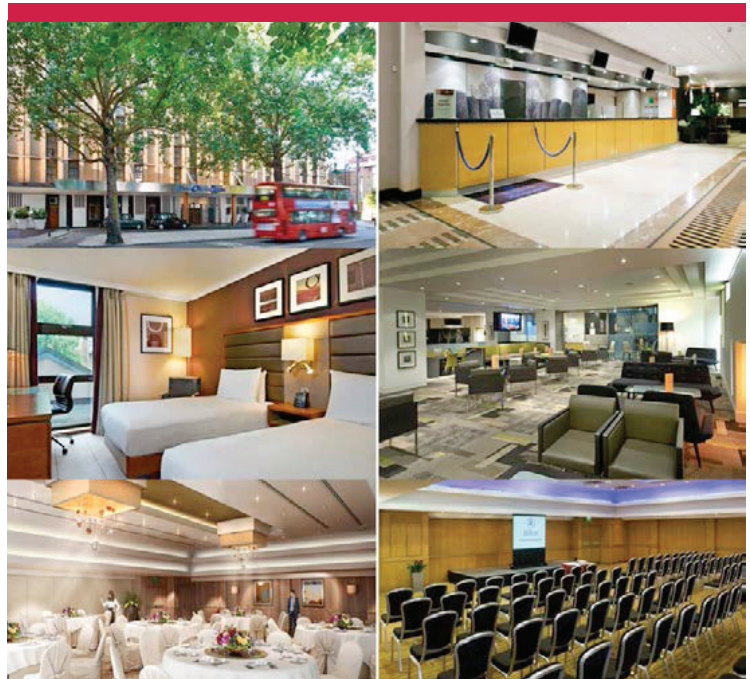
Hospitals & Patient Group Members - 5%

Others - 10%

The two-day Congress will provide an interactive and intimate discussion and networking format led by key industry speakers with intimate knowledge in the industry. Gain practical strategies and best practices on challenges, innovations, technologies and concepts surrounding orphan drugs and rare diseases.

GAIN LATEST INSIGHTS ON

- **Worldwide Development for Orphan Drugs:** Growth and Sustainability for the Industry
- **Global Partnership:** Governments, Orphan Drug Developers and CROs
- **Financing Orphan Drug Developments:** What Are the Options Available?
- **Role of Academic Institutions**
- **Case Studies:** Orphan Drug Growth in Asia and Biotech Companies
- **Regulatory Matters:** IP Strategy, Licensing and Integrity
- **Patient Access, Pricing and Reimbursements:** Challenges and Strategies
- **Future of Orphan Drugs:** What Is In Store?



Hilton London Kensington

179-199 Holland Park Ave, London

W11 4UL, United Kingdom

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SPEAKERS


Barbara McLaughlan, Head of External Affairs – Oncology, NOVARTIS, UK

Barbara McLaughlan is Head of External Affairs and Acting Head of Market Access at Novartis Oncology. In both roles Barbara's focus is on shaping the external environment to ensure that all eligible patients in England, Scotland and Wales are able to access the innovative treatments for rare cancers and rare diseases developed by Novartis. Patients benefiting from the company's commitment to rare diseases include those with Chronic Myeloid Leukaemia, myelofibrosis, pancreatic neuroendocrine tumours, sickle cell, thalassaemia and tuberous sclerosis complex. Before joining Novartis in 2011, Barbara worked as Eye Health Campaigns Manager at RNIB and previously in Government Affairs, education and communications roles in Germany, Belgium and the UK.



Chia Wen Lee, Head of Emerging Markets - Corporate Market Access, BOEHRINGER INGELHEIM, GERMANY

Dr. Chia Wen Lee is a trained epidemiologist and statistician; she has significant scientific experience across various delivery systems (clinical, pharmaceutical/ biotechnology, government, and academia) in Europe, Asia and the US.

Dr. Lee joined Boehringer Ingelheim in May 2014 as the Head of Emerging Markets Access, responsible for the entire prescription medicine portfolio.

She began her career in the Pfizer UK office and with technical competencies of evidence based medicine, statistical analysis, population and clinical epidemiology and Health Technology Assessments (HTA); she was highly effective in providing technical insights into operational planning supporting ophthalmology, pain and CNS therapeutic areas. Being recognized as a talented leader with both academic kudos and business sense, Dr. Lee was assigned the task of setting up and leading the Market Access function (including HEOR&HTA) in China during the year of National Reimbursement Drug List (NRDL), Essential Drug List (EDL) review and the publication of highly anticipated Healthcare Reform Bill in 2009. After accomplishing her assignment securing optimal pricing and access of the portfolio and coaching and developing local talent, Dr. Lee was promoted to the HQs supporting HTA activities for both clinical development assets and in-line products. She was the Head of Emerging Markets Access in Biogen Idec responsible for creating the vision of Emerging Markets Access network, setting up the organization, strategies and details for implantation.

Prior to the industry experience, Dr. Lee had extensive experience in Public Health and healthcare policy making and clinical guideline development including consulting to the Department of Health in Taiwan on maternal and child health, to leading a cross-functional team developing clinical guidelines for National Institute of Health and Clinical Excellence (NICE).

Dr. Chia Wen Lee was born and raised in Taiwan and received her PhD in Epidemiology and Biostatistics from London School of Hygiene and Tropical Medicine.



Christian Girard, Chief Editor, ORPHAN DRUGS INDUSTRY INSIDER & CEO, ABCROWDFUNDING ADVISORS, FRANCE

Christian Girard, is currently the CEO of ABCrowdFunding Advisors, a global consulting firm with a focus on equity crowdfunding and orphan drugs companies. Concurrently, he is the founder and CEO of Orphan Drugs Industry Insider, a competitive intelligence information provider in the rare diseases area. His entry in life sciences dates back in 1994, when he joined the newly formed French subsidiary of Deknatel, a Pfizer's medtech spin-off, which was later acquired by Genzyme. He is also a co-founder of gmp-orphan, a biotech repurposing small molecules in orphan indications, and an advisor to research institutions and start-up companies.



Dr Didier Caizergues, Head of regulatory Affairs Department, GENETHON, FRANCE

A Doctor in Pharmacy, who holds a degree in Health law. He enjoyed a long career in the field of Regulatory Affairs. He began his career as a consultant with International Drug Development (IDD), but soon joined the company SYNTHELABO in its Research and Development center where he worked for nine years. In this office Dr Didier CAIZERGUES oversaw the registration of new molecules that had evolved from cardiovascular, urological and anti-depressive research.

He later headed up the international registration department for the Pierre Fabre group and for the next six years he practices to the fields of anti-cancer drugs, anti-depressants and orphan diseases.

In 2001 he joined the GENETHON as Director of International Regulatory Affairs where he set up a regulatory affairs department specialized in gene and cell therapies in the fields of rare diseases. He obtained several clinical trials authorization in European countries and US for gene therapy products with lentivirus and AAV in the fields of muscular dystrophies and paediatric immunodeficiencies. He is also in charge of the assessment of new molecules or molecules already marketed still in the field of rare diseases in different working groups in European projects (Treat-NMD).

**Christine Lavery**, Chief Executive, SOCIETY FOR MUCOPOLYSACCHARIDE DISEASES, UK

Christine Lavery was appointed Chief Executive of the Society for Mucopolysaccharide and Related Diseases (MPS) in 1993. During her time at MPS she has taken the charity to new heights, managing a substantial research budget and a unique UK-wide advocacy service providing needs led support to nearly 1300 children and adult sufferers, their families and professionals in areas of home adaptations, special educational needs, access to new therapies, respite care, palliative care and pre- and post-bereavement support. Christine has served on the Department of health Advisory Board on Genetic Testing and is currently a patient representative on the NHS England LSD Specialised Commissioning Advisory Group.

Christine was awarded the Member of the British Empire for her services to Metabolic diseases by the Queen in the New Year's Honours List for 2002 and at the 2006 International Symposium on Mucopolysaccharide and related diseases received 'A Life Time Award' from the International MPS Community. Christine continues to promote the needs of MPS, Fabry and related Lysosomal Storage Diseases across nations and working closely with the MPS International Network.

**David C. King**, Global Regulatory Intelligence Director - Regulatory Policies & Intelligence, NOVO NORDISK, DENMARK

David originally trained as a pharmacologist receiving an MSc in Neuropharmacology University of Toronto and a PhD in Cardiovascular Pharmacology from the University of Western Ontario in Canada. Since completing his PhD, David has worked in the pharmaceutical industry for the last 25 years both in Canada and globally based in Europe. During this period he has various roles including clinical research; pharmacovigilance; product development; in-licensing and regulatory affairs. He is currently working in a global regulatory intelligence role with a focus on the Asia Pacific region. David has worked for the last 14 years with the global development and registration of Orphan Medicinal Products. Prior to joining Novo Nordisk, David was the acting medical director working for Swedish Orphan International which specialised in the development of older compounds for rare diseases. He is currently an active member on the EBE/EuropaBio Joint Rare Diseases / Orphan Medicinal Products Task Force.

**David Boothe**, Global Commercial Leader - GSK Rare Diseases, GSK, UK

David Boothe is the Global Commercial Lead for Inherited and Metabolic Disorders within the Rare Diseases Unit at GSK. He has commercial responsibility for a broad franchise that has spanned amyloidoses, lysosomal storage disorders (LSDs) and pulmonary arterial hypertension (PAH) from pre-clinical development through to maturity. David has a wealth of experience in the rare and orphan diseases field having joined GSK from Shire Human Genetics Therapies in Switzerland where he served as EMEA Product Director in Fabry Disease. Prior to Shire, David worked in international marketing roles for BioMarin and before that Oxford GlycoSciences, in MPS VI and Gaucher disease respectively.

**Dr Joanna Cox**, Research and Business Development Manager, ROYAL HOLLOWAY, UNIVERSITY OF LONDON, UK

Dr Joanna Cox is the Research and Business Development Manager for Life Sciences at Royal Holloway, University of London. She has divided her career between academia and industry, previously working in both academic research at the University of Oxford and in early stage biotechnology companies. She has co-founded two companies, both of which continue to be successful. She is currently responsible for research and innovation development in the life sciences at Royal Holloway, University of London. She has formed numerous successful collaborations with industrial partners from multinationals through to mid-cap and SME companies. In technology development she has taken ideas through from early stage proof-of-concept to out-licensing and clinical trials. Her work has often focussed on utilising public/private sector funding initiatives and she has been instrumental in securing over £12million in development funds. In the orphan drugs area her work has focussed on the development of gene therapy technology, with 5 patents currently out-licensed to industry partners and in clinical development.

**Dr. Carlos R. Camozzi**, Chief Medical Officer, ORPHAZYME, DENMARK

Dr. Camozzi has more than 25 years' experience in the biopharmaceutical industry, most recently within the orphan drugs development and paediatric therapeutic options, and successful achievements of products approvals at the European Medicines Agency (EMA) and U.S. Food and Drug Administration (FDA). Prior to Orphazyme, Dr. Camozzi held roles of increasing responsibility at Lederle/ American Cyanamid, F Hoffmann La- Roche and Mepha Ltd. He was the Medical Director of Orphan Europe-Recordati and VP-Chief Medical Officer of uniQure BV (ex-AMT) for the development of Genetherapy for rare diseases.



John Zhaolong Gong, Chief Executive Officer, 3D MEDICINES, CHINA

3DMed is a platform-based, biomarker-driven biopharmaceutical company in China, its mission is to develop safe and efficacious oncology products to fulfill unmet medical needs in China through collaborations with hospitals and clinicians. 3DMed's platforms include the world largest PDC bank for liver cancer, Next-Generation DNA Sequencing, and bioinformatics for large genome data analysis. 3DMed is capable of conducting "clinical trials" in large number of human tumor celllines with clear genome information, thus identifying effective new compounds and discovering new biomarkers.

Prior to 3DMed, Dr. Gong served as VP of Drug Development and Regulatory Affairs of Beigene, CEO of BL Pharma, and CTO of JOINN Laboratories. From 1998 to 2008, Dr. Gong worked at US FDA as Reviewer, he conducted primary and critical reviews of numerous IND and NDA applications for both small molecules and biologics. Dr. Gong is member of the Drug Safety Evaluation Committee of the Chinese Pharmaceutical Association, Council member of AAALAC, and member of editorial board of Chinese Journal of New Drugs. Dr. Gong received his Ph.D. in Toxicology from New York University in 1996 and a medical degree from Beijing Medical College in 1984.



M. (Ken) Kengatharan, PhD, MBA., President & Chief Operating Officer, ARMETHEON, INC., USA

Dr. M. (Ken) Kengatharan is a co-founder and President & COO of Armetheon, Inc. a co-founder and former President & CSO of Altheos, Inc., and a co-founder and former Vice President, Pre-Clinical R&D of Athenagen, Inc. (re-named CoMentis, Inc), all three biopharmaceutical companies are based in San Francisco bay area. He is also a founder and General Partner at Atheneos Capital, a San Francisco bay area based evergreen healthcare incubator fund.

During the last 14 years, Dr. Kengatharan has been critical to the development of multiple drug candidates (NCEs and re-purposed drugs) from concept to Phase II. Since setting-up his first company in the late 1990s, Dr. Kengatharan has been critical to raising more than \$130 MM in private equity for five start-up companies.

In 2004, Dr. Kengatharan co-founded Athenagen, Inc. (re-named CoMentis, Inc.) which in 2008 completed licensing and drug development collaboration deal with Astellas Pharmaceutical worth \$760+ MM with an upfront payment of \$100MM. He joined the company as its Vice-President of Pre-Clinical Research and Development and then became Vice-President of Translational Research and Scientific Affairs. His responsibilities included overseeing discovery research and pre-clinical drug development in cognition enhancement, inflammation, and angiogenesis, in addition to managing pre-clinical R&D operations in South San Francisco. Prior to joining CoMentis, he was a staff scientist in the Division of Cardiovascular Medicine at Stanford University, where he continued his affiliation as a visiting scientist until June 2013. Dr. Kengatharan has also served as Director of Corporate Development, and later, Vice President of Pre-Clinical R&D at OxoN Medica Inc., where he spearheaded pre-clinical drug development as well as R&D operations. He is currently a member of the board of directors at Armetheon, Inc., and previously a board observer at CoMentis, Inc., and Altheos, Inc. He has also served on the boards of EPI3, Athenagen, Inc., and Altheos, Inc., and as a member of the advisory board of Cardinal Free Clinics at Stanford University School of Medicine. In addition, he has been an advisor to Stanford's SPARK program, a technology translation project for biotech ideas, since it was founded in 2006. Dr. Kengatharan is an author of several research articles and reviews in the vascular biology, inflammation, and ocular pharmacology areas, and has been invited to present at local and international scientific meetings covering topics in angiogenesis, inflammation, ocular drug delivery and biotech financing and start-ups.

Dr. Kengatharan obtained his PhD in pharmacology from the University of London at the William Harvey Research Institute with Nobel laureate Sir John Vane and his MBA (with Distinction) from Durham University in England, where he focused on Biotech Finance and Entrepreneurship. Thereafter, he held a post-doctoral position at the same institute as a recipient of a British Heart Foundation Fellowship.



Prof Bobby Gaspar, Professor of Paediatrics & Immunology, INSTITUTE OF CHILD HEALTH - U.C.L., UK

Bobby Gaspar is a physician/scientist working in paediatric immunology at the Institute of Child Health (ICH) and Great Ormond Street London. He initially trained in paediatrics and then became interested in primary immunodeficiencies at an early stage in his career and undertook a PhD at the Molecular Immunology Unit at ICH. From there he has continued his academic and clinical career. His interests are in many different aspects of primary immunodeficiency including understanding the molecular and cellular defects and disease pathogenesis, bone marrow transplantation for severe immunodeficiencies and the development of gene and cell therapy for these conditions. Over the last decade, his team have conducted clinical trials that have shown that gene therapy can successfully correct the immune defect in specific immunodeficiency conditions. He is leading UK and European initiatives for newborn screening for severe combined immunodeficiency. He is also Director of the Centre for Research in Rare Diseases in Children (CRRDC) a new academic and clinical facility that will open at Great Ormond Street in 2018.



Josie Godfrey, Associate Director - Highly Specialised Technologies, NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE (NICE), UK

Josie Godfrey is Associate Director at NICE with responsibility for the new Highly Specialised Technologies programme. She is leading work to establish programme which will make recommendation to the NHS in England about the use of new highly specialised technologies.

Before joining NICE, Josie led work to establish the new Advisory Group for National Specialised Services (AGNSS) She developed a decision-making framework to support AGNSS in making these recommendations and developed the process for considering highly specialised services, drugs and technologies.

Josie has worked in health policy development and implementation for the NHS in England and national and local government.



Miriam Gargesi, EuropaBio Healthcare Director, EUROPABIO, BELGIUM

Miriam Gargesi is currently Director for Healthcare Biotechnology at EuropaBio, the European Association for Bioindustries. In this role, she leads the advocacy and public affairs of EuropaBio's healthcare department, in close coordination with the association's Healthcare Council and Working Groups, and maintains a strong network of contacts with policymakers and other stakeholders. Mrs Gargesi has longstanding experience in the field of European healthcare policies, spanning across major pillars of the sector such as biotechnologies, pharmaceuticals and diagnostics alike. Prior to joining EuropaBio, she served for several years as Director for Public Affairs and Communications at EDMA, the European Diagnostic Manufacturers Association, and she worked for the healthcare practice of a frontrunner international consultancy. She also gained extensive insights into the work of political institutions through her work at the House of Commons in London and the Italian Chamber of Deputies in Rome. Mrs Gargesi holds an MSc in European Political Economy from the London School of Economics and Political Science (LSE), as well as a BSc in International Relations from the University of Wales, Aberystwyth, and McGill University. An Italian national, she is fluent in English and French, and has a working level of Spanish.



George Dickson, Chair of Molecular Cell Biology, School of Biological Sciences, ROYAL HOLLOWAY, UNIVERSITY OF LONDON, UK

George Dickson is Professor of Molecular Cell Biology at Royal Holloway - University of London (RHUL). He has spent most of his career studying neuromuscular disease and muscle cell biology, including the first cloning of an intact dystrophin gene, the discovery of the role of cell adhesion molecules in muscle stem cell fusion, the first identification of utrophin, and the first description of exon skipping in Duchenne muscular dystrophy (DMD). Professor Dickson has also conducted notable research into gene therapy for atherosclerosis, and genetic vaccination against HIV/AIDS. He is a member of the UK MDEX Consortium, UK HIV-Vaccine Consortium, and the EU-SKIP-NMD Project, and has been a platform leader in the EU Clinigene Network of Excellence. He is a past President of the European Society of Gene & Cell Therapy, and a past Secretary and founder member of the British Society for Gene Therapy. He has been a member of the European Medicine Agency Committee for Advanced Therapeutics (Gene and Cell Therapies). Professor Dickson is also a Royal Holloway Research Theme Champion for 'Health, the Human Body and Behaviour'.



Hartwig GAJEK, Managing Director, HESYRA-MEDICAL GMBH, SWITZERLAND

Hartwig is Managing Director of Hesyra Medical GmbH in Binningen/Switzerland. For approx. 25 years, Hartwig held various positions in international clinical development and medical affairs, mainly in the biopharmaceutical industry. His scope included clinical development phase I-IV in Europe, US and Asia, product launch and post-launch programmes, scientific communication, negotiations with regulatory authorities, health and industry policy, supporting market access, and developing defence strategies for loss-of-exclusivity. Hartwig has developed a special interest in rare congenital diseases, and has experience in developing international registries, as well as working with advocacy groups. Before joining the pharmaceutical industry, Hartwig spent 10 years as a practising physician in anaesthesia and intensive care.



Ségolène Aymé, Emeritus Research Director, INSERM, FRANCE

Ségolène Aymé is Emeritus Director of Research at the French Institute of Health and Medical Research (INSERM). She was the founder of Orphanet in 1997 and its Executive Manager up to 2011. She chaired the EU Committee of Experts on rare Diseases (EUCERD 2010-2013) and is now a member of the Commission Expert Group on Rare diseases. She also chairs the WHO Topic Advisory Group for Rare Diseases and serves as Editor-in-Chief of the Orphanet Journal of Rare Diseases (www.ojrd.com). She is the project leader of "Support IRDiRC", which provides the services of a scientific secretariat to the International Rare Diseases Research Consortium (www.irdirc.org).



Mark Corbett, Vice President, Clinigen Global Access Programs (GAP) – Clinigen

Mark Corbett joined Clinigen in mid 2010 as Vice President Clinigen Global Access Programs with overall responsibility for Clinigen's Global Access Programs Division which specialises in the consultancy, development, set up and implementation of access programs on behalf of the Biotech and Pharmaceutical industries.

Over the last 6 years Mark has specialised in the area of Named Patient / Early Access and Compassionate Use Programs. Prior to joining Clinigen, Mark was the Head of European Business Development and a Global Account Director at the market leader in the field of Named Patient Programs. Within this role Mark was responsible for overseeing all new business opportunities for the provision of services to the pharmaceutical industry originating through the global headquarters.

Mark has gained extensive specialist knowledge and operational expertise in implementation of over 100 Global Early Access / Named Patient Programs for a variety of companies ranging from niche Biotech to Large Top 10 Pharma.

Mark is widely recognised as a thought leader on the subject of Early Access / Compassionate Use / Named Patient Programs having spoken at a number of industry congresses and workshops over the last 6 years.

Prior to specialising in the area of Early Access and Named Patient Programmes, Mark spent 13 years within the pharmaceutical industry in a number of sales, training and marketing roles with Servier Laboratories Ltd. Latterly, he had overriding responsibility for the launch of an innovative novel product to the market during which he developed his interest in and experience in the complexities of market access to innovative medicines across Europe.



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).

The AKU Society is a fast-growing patient movement, with formal patient groups in the UK, France, Italy, Germany, the Netherlands, Jordan, Belgium, Slovakia, North America and India. It is spearheading an international consortium of biotechs, pharma companies, universities, hospitals and clinical trial centres across Europe, the Middle East, Asia and North America. It works closely with the hospital and university in Liverpool.

Dr Sireau is Co-founder and Chairman of Findacure (www.findacure.org.uk), a new social enterprise that raises awareness into and helps patients with fundamental diseases: extreme and exceptional diseases that advance our understanding of medicine and help us discover potential new treatments (www.findacure.org.uk).

He is a committee member of Rare Disease UK, the national alliance of rare disease groups in the UK, and of the International Rare Disease Research Consortium (IRDiRC). He is also a founding member of the Cambridge Rare Disease Network and a trustee of DNAdigest, a social enterprise that works on collaborative data cures for genetic diseases.

He is a fellow of the Ashoka fellowship of social entrepreneurs and of the Royal Society of Arts. He is the editor of 'Rare Diseases: Challenges and Opportunities for Social Entrepreneurs' (Greenleaf, 2013) and co-editor of 'The Rare Diseases Patient Group Handbook' (forthcoming). He is co-founder of the Vincent Foundation for brain injury and a former non-executive Director of GenSeq, a bioinformatics company.

Dr Sireau's previous career was in international development, where he was CEO of SolarAid and Sunny Money, two award-winning non-profit social enterprises that he set up. Sunny Money is now the largest distributor of solar lanterns in Africa. He has also written several books on international aid.

Dr Sireau has a PhD in Social Psychology. His hobbies include writing novels and producing electronic music.



CONGRESS DAY 1 | Monday, 29 June 2015

08.00	Registration
08.45	Chairperson's Opening Remarks

GLOBAL OUTLOOK - OVERVIEW

09.00 <Presentation>	<p>Global Paradigm Shift for Orphan Drugs: How Has the Industry Evolved and What Must Be Done to Ensure Its Sustainability?</p> <ul style="list-style-type: none">• Review the growth of orphan drugs market which includes exclusivity options for multiple orphan indications, off-label usage, expansion of orphan indications, and freedom from generic competition• What will continue to be the key game changers altering the dynamic of the orphan drug industry for developers?• How can the industry and payors work together to ensure long term sustainability of bringing rare disease treatments to patients in need?
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09.40 <Panel Discussion>	<p>Financing Drug Discovery for Orphan Diseases: What Are the Options Available?</p> <ul style="list-style-type: none">• How can megafunding address the lack of in traditional sources of financing for orphan drugs?• How are orphan drugs suited for portfolio financing?• Why orphan drug companies are good investments and what risks are associated with investing in orphan drugs?• What role do mergers and acquisitions play in the orphan disease investment story? <p>PANELLISTS:</p> <p>Miriam Gargesi, EuropaBio Healthcare Director, EUROPABIO, BELGIUM</p> <p>Christian Girard, Chief Editor, ORPHAN DRUGS INDUSTRY INSIDER & CEO, ABCROWDFUNDING ADVISORS, FRANCE</p>
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10.20 <Presentation>	<p>Ultra Orphan Drugs: What is the Long Term Strategy to Support Ultra Orphan Drugs and How Commercially Viable Are These Drugs to Stakeholders?</p> <ul style="list-style-type: none">• With orphan drugs pricing being questioned, how can companies develop ultra orphan drugs for patients with ultra rare diseases and what can governments do to support these companies?• What are investors take on the value for money for pharmaceutical companies seeking to boost their top line sales via M&A in ultra orphan drugs and should regional manufacturers look at developing ultra orphan drugs?• Will the classification of some drugs as "ultra-orphan" and others just "orphan" artificially divide services and access to life-saving drugs? <p>Christian Girard, Chief Editor, ORPHAN DRUGS INDUSTRY INSIDER & CEO, ABCROWDFUNDING ADVISORS, FRANCE</p>
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10.50	10.50 Morning Break & Networking
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11.30 <Shared Platform>	<p>Academic Platform: From the Perspectives of Academics</p> <p>The Role of Academic Institutions in Contributing to the Development of Orphan Drugs</p> <p>Dr Joanna Cox, Research and Business Development Manager, ROYAL HOLLOWAY, UNIVERSITY OF LONDON, UK</p> <p>Case Study on the Development of a Gene Therapy ATMP Orphan Drug for Duchenne Muscular Dystrophy</p> <p>Prof George Dickson, Chair of Molecular Cell Biology, School of Biological Sciences, ROYAL HOLLOWAY, UNIVERSITY OF LONDON, UK</p>
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12.10 <Presentation>	<p>The Emerging Markets Perspective in Orphan Drug Development, Market Access And Pricing</p> <ul style="list-style-type: none"> • All eyes on Asia: Japan and China - Considerations for clinical development in trial design and launch planning - Construct relevant value stories for Japan and China : neighbouring countries with very different approach - Pricing and access in Japan and China • Orphan Drug Designation in key markets (including China, Japan, Mexico, Turkey, Argentina, S Korea, Taiwan Brazil and Russia) - What is the implication on global launch sequencing? <p>Dr. Chia Wen Lee, Head of Emerging Markets, Corporate Market Access, BOEHRINGER INGELHEIM, GERMANY</p>
12.50 <Presentation>	<p>GSK's Adaptive and Flexible Approach to Rare Diseases</p> <ul style="list-style-type: none"> • How GSK continues to adapt to the external environment to ensure patients/HCPs/carers are at the centre of everything we do • How GSK's operating model aims to bring benefit to patients with Rare Diseases • How GSK has applied adaptive licensing to help shorten the period to approval and broadening access to medicines: a case study <p>David Boothe, Global Commercial Leader – GSK Rare Diseases, GSK, UK</p>

13.30	Networking Lunch
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REGULATORY, RISKS AND CHALLENGES

14.30 <Presentation>	<p>Integrity of Orphan Drugs Legislations: What Benchmark for Globalised Standards Can Regulators Implement Globally and What Can Be Done to Ensure the Integrity of These Standards?</p> <ul style="list-style-type: none"> • How can orphan drug manufacturers help support governments' initiatives to drive the region's industry over the next 10 years? • What approaches can pharmaceutical companies use to get a new product through development and approval? <p>Dr. David King, Global Regulatory Intelligence Director - Regulatory Policies & Intelligence, Novo Nordisk, DENMARK</p>
15.10 <Presentation>	<p>Early access to orphan drugs: • Current environment, • Regulatory requirements, • Practical considerations</p> <p>Mark Corbett, VP, Clinigen Global Access Programs (GAP) – Clinigen </p>
15.50	Tea Break & Networking
16.30 <Presentation>	<p>IP & Licensing Strategy: How Can Patent Protection Be Used To Expand the Scope Of Protection Beyond the Particular Indication Covered By Orphan Exclusivity?</p> <ul style="list-style-type: none"> • How can patent protection extend the actual market exclusivity of a drug which enhances the incentive for investment? • How can sponsors file an investigational new drug (IND) application to study the product in order to protect exclusivity and limit competition? • How licensing affects big pharmas and biotech companies?
17.10 <Presentation>	<p>Risks & Rewards of Orphan Drugs Development</p> <p>M. (Ken) Kengatharan, PhD. MBA., President & Chief Operating Officer, ARMETHEON, INC., USA</p>
17.50	<p>Case Study: Why the Development of Orphan Drugs Attractive to Small Biotech Companies Compared to Big Pharmas and Should Others Follow Suit?</p>
18.30	Chairperson's Closing Remark & End of Day 1



CONGRESS DAY 2 | Tuesday, 30 June 2015

08.00 Registration

08.45 Chairperson's Opening Remarks

PATIENT ACCESS, PRICING & REIMBURSEMENTS

09.00 **Rising Patient Costs: How Are Payors and Drug Companies Balancing the High Costs of Orphan Drugs?**

<Presentation>

- How can manufacturers establish the value of orphan drugs therapies worldwide?
- What are the criteria that should be used to access the value of a newly approved orphan drug?

Barbara McLaughlan, Head of External Affairs - Oncology, NOVARTIS, UK

09.40 **The Ideal Partner: What Are the Criteria that Partners for Orphan Drugs Developers Look?**

<Panel Discussion>

- How can orphan drug companies partner with governments and what are some of the steps to follow in seeking partnership?
- What are orphan drug developers looking for when partnering with a CRO and how CROs can meet the needs for external expertise to overcome development hurdles?
- How to pick the right team and focus on retaining key study personnel for the length of the trial?

Dr. John ZL Gong, Chief Executive Officer, 3D MEDICINES, CHINA

10.20 **Building Long-Term Sustainability of Clinical Research Networks and Registries**

<Presentation>

- How can you prepare healthcare providers to know about the therapy and identify the right patients for your therapy?
- How can developers make research goals and use of data to be transparent and to be informed about progress or roadblocks in order to build a relationship based on trust with patients?
- What enhanced ongoing patient support beyond the clinic can be provided to encourage positive relationships between patients and healthcare providers?

Dr. Hartwig Gajek, Managing Director, HESYRA-MEDICAL GMBH, SWITZERLAND

10.50 10.50 Morning Break & Networking

11.30 **Cross-Border Health Care Services for Patients with Rare Diseases**

<Shared Platform>

- How does reimbursement work under the Directive 2011/24/EU for EU member states?
- How does the directive allow health professionals assist in the diagnosis of rare diseases and possibly accelerate the treatment for patients?

Dr Ségolène Aymé, Emeritus Research Director, INSERM, FRANCE

12.10 **Paediatric Drugs and Rare Diseases: How Are Children with Rare Diseases Treated and How Are Their Data Protected?**

<Presentation>

- What influences does the European paediatric regulation have on marketing authorisation of orphan drugs?
- What can be done by healthcare providers to be proactive in monitoring side effects that can put patient safety at risk and effectively communicate results to the developer?
- How should patients' information about their treatment experience be collected and how can this steer the evolution of orphan drugs for children with rare diseases?

Christine Lavary, Chief Executive, MPS SOCIETY, UK

12.50 **Case Study: The Role Played by the Patients Associations in the Field Of The Rare Diseases in the Development of New Medicinal Products**

<Presentation>

- The "Association Française contre les Myopathies" a patient group supporting R&D for Rare Disorders from Research to patient access.
- An expert group the TACT, supported by several patients associations that created the European program Treat-NMD, in charge to assess several developments of orphan medicinal products.

Dr. Didier Caizergues, Head of regulatory Affairs Department, GENETHON, FRANCE

13.30 **Networking Lunch**



SCIENCE, TECHNOLOGY AND THE FUTURE OF ORPHAN DRUGS

14.30 <Presentation>	<p>Supply Chain Optimisation: Timely and Equitable Access and Definition of Value of Innovation</p> <ul style="list-style-type: none">• How can governments support production and supply of orphan drugs and continue to increase access especially for medications that are hard to come by?• What major challenges will developers face when distributing orphan drugs and how can these challenges be overcome?• How can you effectively handle your supply chain to deal with orphan drug supply?
15.10 <Presentation>	<p>Strategic Corporate Social Responsibility (CSR) and Orphan Drugs Development: Why Is It Important to Integrate CSR Into the Development of Orphan Drugs and How Can This Reinforce the Orphan Market for a Sustainable Future?</p> <ul style="list-style-type: none">• How can orphan drug companies take a strategic approach to social responsibility by integrating orphan drug development and social responsibility practices with core business activities to obtain both economic and non-economic benefits for the firm and its stakeholders?• In what way can companies derive more strategic social responsibility benefits from developing orphan drugs?• How can the potential of new technological developments alleviate social responsibility in the development of orphan drugs? <p>Dr. Carlos R. Camozzi Chief Medical Officer, ORPHAZYME, DENMARK</p>
15.50	Tea Break & Networking
16.30 <Presentation>	<Sponsor Spotlight>
17.10 <Panel Discussion>	<p>What Challenges Still Remain and How Can the Orphan Drugs Marketplace Be Improved?</p> <p>Panellists:</p> <p>Josie Godfrey, Associate Director, Highly Specialised Technologies, NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE (NICE), UK</p> <p>Prof Bobby Gaspar, Professor of Paediatrics & Immunology, INSTITUTE OF CHILD HEALTH - U.C.L., UK</p> <p>Dr. Carlos R. Camozzi, Chief Medical Officer, ORPHAZYME, DENMARK</p>
17.50	<p>The Future of Orphan Drugs in Science and Leveraging Technology</p> <ul style="list-style-type: none">• What technology platforms can spur the orphan drug development?
18.30	Chairperson's Closing Remark & End of Conference



POST-CONFERENCE WORKSHOP DAY 3 | Wednesday, 1 July 2015

WORKSHOP A: EQUITY CROWDFUNDING FOR ORPHAN DRUGS – PATIENT-CENTRIC INVESTORS

- How do we fund an orphan drug candidate in the Death Valley?
- Can the crowd fund the rare?
- Why and how did patients associations contribute to the funding of orphan drugs companies (the Kalydeco case)?
- How do patients associations contribute to the funding of orphan drugs companies, today?
- Can patients, families and relatives directly contribute to the funding of orphan drugs companies, and what are the pros and cons?
- How to reconcile donation and ownership?

Led By:

Christian Girard, Chief Editor, ORPHAN DRUGS INDUSTRY INSIDER & CEO, ABCROWDFUNDING ADVISORS, FRANCE

WORKSHOP B: PATIENT ADVOCACY ENGAGEMENT & COLLABORATION

- How can manufacturers build strong relationships with patients?
- How can we bridge power and knowledge with patients to bring them as partners into the drug development process which continue to remain complicated for rare diseases?
- How can secured patient registries infrastructure provide assured confidentiality and privacy protections for patients contributing their information?
- How can patient advocacy groups influence regulation and corporate strategy?
- How can developers make transparent the research goals and use of data to patient and continue to gain patients' trust?

Led By:

Dr Nicolas Sireau, Chairman and CEO, AKU Society

ORPHAN DRUGS & RARE DISEASES

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