6th Edition

Orphan Drugs Summit

21st - 23rd September, Novotel Amsterdam Schiphol Airport

#orphandrugssummit

FOSTERING THE RIGHT RELATIONS AND BRINGING THE RIGHT DISCUSSIONS TO COLLABORATIVELY SHAPE THE FUTURE OF ORPHAN DRUGS

Partner

Medicom Innovation Partner

Ad Schuurman, Head of Business Contact Centre and International Affairs National Health Care Institute

Martine Zimmermann, VP Global Regulatory Affairs, Alexion Pharma International

Egbert Biesheuvel, Senior Researcher, ASTERIX Project

Alan Thomas, Founder, Ataxia and Me

Steinar Madsen, Medical Director, Norwegian Medicines Agency

Luisa-Maria Botella, Research Scientist, Centro de Investigaciones Biológicas CSIC

Robert Derham, Founder & President, CheckOrphan

Jean Nordstrom, CEO, Sixera Pharma

Anders Waas, CEO, Tikomed

Elizabeth Vroom, Founder and President, Duchenne Parent Project

Marlene E. Haffner, MD, MPH, CEO, Haffner Associates, LLC (former Director of OOPD at US FDA)

Dr. Hoss A Dowlat, Vice President Regulatory Affairs, Global Strategy PharmaBio Consulting

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Orphan Drugs Executive Platform
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3 REASONS TO ATTEND

1 **Structured networking** - Meet over 100 of your peers during our structured networking breaks, informal dinner and cocktail receptions

2 **Strategic learning** - Our programme offers choice! Select and tailor the event according to your needs.

3 **Niched and solution oriented** - Our optimally scaled event allows for in depth learning and discussion resulting in a solution oriented approach for your burning questions

EDITOR'S NOTE

The Orphan Drugs Summit is back for the 6th edition and after 5 successful years in Copenhagen we are happy to announce that we are moving to Amsterdam! What has not changed is that orphan drugs are highly challenging from discovery to postmarket surveillance and comprehensive review of adverse events. That is why we are continuing to create THE platform for payers, hospital representatives, patient organisations, venture capitalists, regulatory bodies, drug developers, researchers and industry associations to come together to overcome major challenges.

2016 is an important year as the global orphan drugs market is expected to reach a $176 billion mark in only 4 years from now. With a 10.5% compound annual growth rate, the prescription sales in the orphan sector is growing at twice the rate of the overall prescription market.

However, there are major factors like high initial investment, regulatory obstacles and clinical hurdles that constrain the run of the orphan drugs market. The regulatory framework and standards vary from country to country, making it difficult for companies to operate on a global level, especially in Europe.

If you are looking forward to being part of the orphan drugs business and policy of tomorrow, attend the Orphan Drugs Summit 2016.

Join us to get the latest on:

- *Fast changing national and regional regulations*
- *Clinical trial design*
- *Patient Registries & stakeholder engagement*
- *Partnering and establishing financing for future development*
- *Establishing a balanced and sustainable pricing and reimbursement foundation*
- *Achieving an efficient and timely access to market with equal access for patients around the world*

Welcome to the 6th annual Orphan Drugs Summit.
Sincerely,

Maaike Gerritse
Editorial Director
Orphan Drugs Summit

OPENING KEYNOTE JOHN F. CROWLEY

John F. Crowley is the Chairman and CEO of Amicus Therapeutics, Inc. John's involvement with biotechnology stems from the 1998 diagnosis of two of his children with Pompe disease-a severe and often fatal neuromuscular disorder. In his drive to find a cure for them, he left his position at Bristol-Myers Squibb and became an entrepreneur as the Co-founder, President and CEO of Novazyme Pharmaceuticals, a biotech start-up conducting research on a new experimental treatment for Pompe disease (which he credits as ultimately saving his children's lives). In 2001, Novazyme was acquired by Genzyme Corporation and John continued to play a lead role in the development of a drug for Pompe disease as Senior Vice President, Genzyme Therapeutics.

John and his family have been profiled on the front page of The Wall Street Journal and are the subjects of a book by Pulitzer prize-winning journalist Geeta Anand, "The Cure: How a Father Raised $100 Million-And Bucked the Medical Establishment-In a Quest to Save His Children." The major motion picture, Extraordinary Measures, starring Brendan Fraser and Harrison Ford, is inspired by the Crowley family journey. John is the author of a personal memoir: Chasing Miracles: The Crowley Family Journey of Strength, Hope and Joy.

John is also a commissioned officer in the U.S. Navy Reserve, assigned to the United States Special Operations Command and is a veteran of the global war on terrorism, with service in Afghanistan. He graduated with a B.S. in Foreign Service from Georgetown University, and earned a J.D. from the University of Notre Dame Law School and an M.B.A. from Harvard. The Crowley family was the recipient of the 2011 Family Exemplar Award from the University of Notre Dame. He is also a member of the University Council on Science & Technology at Notre Dame. He serves on the Executive Committee as National Chairman of the National Board of Directors of the Make A Wish Foundation of America and is a founding Board member of the Global Genes Project. John is a Henry Crown Fellow at the Aspen Institute.

*For the full list of speaker, please see page 14

1ST GLANCE

DAY 1
September 21st

DAY 2
September 22nd

09:00 - 09:45
Keynote: Chasing Miracles: When Drug Research is Personal

09:45 - 10:30
Keynote: Case study: 2 recent approval for ultra metabolic diseases

LAB: CLINICAL TRIALS

11:00 - 11:20
Promoting clinical development for patients with rare diseases: an overview of new methodology by the ASTERIX project

11:20 - 11:40
Development of a drug for a rare and orphan disease through a foundation

11:40 - 12:00
Challenges in Prioritization and Clinical Development of Orphan Drugs

12:00 - 12:30
Clinical trials panel: Trial design: How to make it regulatory acceptable and cost effective?

LAB

13:30 - 15:00
Shark Tank

LAB: PATIENT SPOTLIGHT

13:30 - 13:50
I have a Rare disease and I am Crossing Borders

13:50 - 14:10
The wisdom of a small crowd: Waihonapedia

14:10 - 14:30
The patient as a partner

14:30 - 15:00
Patient spotlight panel: What is the role of the patient?

15:30 - 16:15
Keynote: Listening to the Voice of the Patient and Integrating that Voice into the Regulatory Decision Making Process: Benefit and Risk Studies in Duchenne Muscular Dystrophy

16:15 - 17:00
Keynote: Cost-effectiveness of Orphan Drugs

1ST GLANCE

DAY 3
September 23rd

08:45 - 09:30
Keynote: Value Based Assessment of Orphan Drugs - How do we value Innovation with rare Diseases? The Payer's Perspective

09:30 - 10:15
Keynote: "VIB's impact from Science to Patients – Oncurious as a case"

10:15 - 10:45
Networking break

LAB: PRICING & REIMBURSEMENT	LAB: MARKET ACCESS
10:45 - 11:05 Dutch Orphan Drugs Policy - EU collaboration regarding assessment and reimbursement	**10:45 - 11:05** Drug Repurposal as an alternative way for orphan drug research
11:05 - 11:25 Affordability & maintainability: socially responsible pricing and ensuring patient access to treatments	**11:05 - 11:25** Is global collaboration the answer to reaching all potential patients?
11:25 - 11:45 Pricing of orphan drugs: Establishing a reimbursement foundation	**11:25 - 11:45** Strategies for regulatory fast tracks and global market access
11:45 - 12:15 Pricing & Reimbursement panel: What is the right price?	**11:45 - 12:15** Market Access panel: Orphan drugs in emerging markets: how to ensure market access?

12:15 - 13:00
Keynote: Data & Innovation

13:00
End of event, one for the road

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AGENDA

DAY 2
September 22nd

09:00 - 09:45

KEYNOTE: CHASING MIRACLES: WHEN DRUG RESEARCH IS PERSONAL

John Crowley has never quit. When two of his young children were diagnosed with Pompe disease, a rare and fatal neuromuscular disorder that affects only several thousand children worldwide, Crowley defied the odds of survival and embarked on a journey to find a cure, parlaying his personal struggle into a professional juggernaut. The Crowley family personal memoir, chasing miracles, is an inspiring story of what it means to fight and overcome phenomenal odds for your children. It is a journey about hope, learning, life and love.

JOHN F. CROWLEY
Chairman & Chief Executive Officer, Amicus Therapeutics

09:00 - 09:45

CASE STUDY: 2 RECENT APPROVAL FOR ULTRA METABOLIC DISEASES

In this session we will share our recent experience with the regulatory review of 2 enzyme replacement therapies for 2 ultra-rare life threatening disease. Key Success factors and pitfalls from both submissions will be described. Moreover, details regarding the role of natural history data in the regulatory decision will be discussed.

LEARN

- *Accelerate assessment*
- *Approval under exceptional circumstances*
- *Natural history studies*

MARTINE ZIMMERMANN
VP Global Regulatory Affairs, Alexion Pharma International

10:30 - 11:00

NETWORKING BREAK

11:00 - 12:30

CLINICAL TRIALS

11:00 - 11:20

PROMOTING CLINICAL DEVELOPMENT FOR PATIENTS WITH RARE DISEASES: AN OVERVIEW OF NEW METHODOLOGY BY THE ASTERIX PROJECT

ASTERIX is a novel EU funded research project specifically designed to optimize methodology for clinical trials in small populations to achieve more reliable and cost efficient clinical development of treatments for rare diseases. The main objectives include to

- develop design and analysis methods for single trials and series of trials in small populations,
- propose a framework to cluster clinical conditions based on key characteristics of disease to guide design and analysis
- include patient level information and perspectives in design and decision making throughout the clinical trial process.
- validate new methods and propose improvements for regulatory purposes

Key learning points:

- Innovation in clinical trials is possible
- reconsideration of level of evidence is needed in combination of new models of decision making
- patient involvement is essential.

JOHANNA H. (HANNEKE) VAN DER LEE
MD, PhD

AGENDA

DAY 2
September 22nd

11:20 - 11:40

DEVELOPMENT OF A DRUG FOR A RARE AND ORPHAN DISEASE THROUGH A FOUNDATION AND COMPANY PARTNERSHIP

The presentation will describe and highlight the difficulties and challenges for a foundation to move a drug development project for a rare and orphan disease from the discovery phase toward the clinical stage. Examples of concrete situations to be overcome during the development of the drug candidate will be discussed, namely contractual aspects with academic institutions, intellectual property constraints, financing of the preclinical development, regulatory questions and clinical setup.

STÉPHANE DEMOTZ
Ph.D. Founder and CEO, DORPHAN

LEARN

- *Regulatory, clinical and business competencies are required early on during preclinical development of drug candidates*
- *Early discussions with investors and pharmaceutical companies allow anticipating the preclinical data package required for out-licensing*
- *Building a dense network of stakeholders (scientists, clinicians, patients, pharmaceutical companies, investors…) is of the essence for the success of the program*

11:40 - 12:00

CHALLENGES IN PRIORITIZATION AND CLINICAL DEVELOPMENT OF ORPHAN DRUGS

The key objectives of this session will focus on major issues in prioritizing disorders or programs as a corporate entity developing orphan drugs, focusing on growing their pipeline. The session will address strengths and limitations of major criteria for the prioritization of disorders or groups of disorders; key challenges in the clinical development programs; and will discuss case studies on different approaches taken by various companies.

HOOTAN KHATAMI
M.D, Escala Therapeutics

LEARN

- *IDifferent approaches to prioritization of orphan disorders and strengths & limitations of various approaches*
- *IKey challenges in the clinical development of orphan disorders and strategies that might be used to mitigate*
- *ICase studies of successful approaches to meet both objectives*

12:00 - 12:30

CLINICAL TRIALS PANEL: TRIAL DESIGN: HOW TO MAKE IT REGULATORY ACCEPTABLE AND COST EFFECTIVE?

JOHANNA H. (HANNEKE) VAN DER LEE
MD, PhD

STÉPHANE DEMOTZ
Ph.D. Founder and CEO, DORPHAN

HOOTAN KHATAMI
M.D, Escala Therapeutics

12:30 - 13:30

NETWORKING LUNCH

AGENDA

DAY 2
September 22nd

13:30 - 15:00
SHARK TANK

13:30 - 15:00
SHARK TANK

The shark tank is a platform that brings successful drug developers, patient organisations or industry associations together with venture capitalists - sharks. Whether you have a breakthrough research, a far advanced clinical trial or already operating successfully and looking to expand and could use financial backing, the shark tank is the platform for you. Book your 15 minutes with the sharks!

13:30 - 13:50
PATIENT SPOTLIGHT

13:30 - 13:50
I HAVE A RARE DISEASE AND I AM CROSSING BORDERS

This session is about a Patient Advocate that is "on a mission" for Ataxia / rare diseases, using the the power of the internet to communicate to a wider audience.

ALAN THOMAS
Founder, Ataxia and Me

13:50 - 14:10
THE WISDOM OF A SMALL CROWD: WAIHONAPEDIA

To find out more about the ultra rare Marshall Smith Syndrome Henk-Willem started a unique collaboration between researchers and families to acquire more knowledge through a so-called Waihonapedia. This has led to many additional insights about the syndrome. How Henk-Willem overcame the challenges of cooperation, language barriers and how this initiative will be continued in a collaborative project.

LEARN

- *Collaboration researchers - patients*
- *The role of the internet / social media in building knowledge into rare diseases*
- *Empowering patients/families*

HENK-WILLEM VAN DER LAAN MSC
Founder, MSS Research Foundation

14:10 - 14:30
THE PATIENT AS A PARTNER

Patient organizations play a major role in identifying challenges and partnering with academia and industry. They can help to solve the problems many drug developers are facing. In this session we will discuss the misunderstandings of the impact patient organisations can have in promoting drug development for their disease.

LEARN

- *The role of patients and how to partner with them*
- *The misunderstandings of the impact of patient organisations*
- *How patients can promote drug development*

AGENDA

DAY 2
September 22nd

13:30 - 15:00
SHARK TANK

13:30 - 15:00
SHARK TANK CONTINUES

13:30 - 13:50
PATIENT SPOTLIGHT

14:30 - 15:00
PATIENT SPOTLIGHT PANEL: WHAT IS THE ROLE OF THE PATIENT?

ALAN THOMAS
Founder, Ataxia and Me

HENK-WILLEM VAN DER LAAN MSC
Founder, MSS Research Foundation

15:00 - 15:30
NETWORKING BREAK

15:30 - 16:15
KEYNOTE: LISTENING TO THE VOICE OF THE PATIENT AND INTEGRATING THAT VOICE INTO THE REGULATORY DECISION MAKING PROCESS: BENEFIT AND RISK STUDIES IN DUCHENNE MUSCULAR DYSTROPHY

Diverse stakeholders continue to advocate for incorporating the voice of patients and caregivers into drug development and regulatory benefit-risk analysis. While there will always be a role for patient and caregiver testimony, there has been a strong emphasis on the using rigorous scientific methods to document the priorities and preferences of patients. Regulatory Agencies are encouraged to develop frameworks and give clear guidance on methods that can be used to document the patient preferences, there is a great deal of anticipation on how current patient-focused drug development strategies can be extended to include more scientifically rigorous methods to document patient and caregiver preferences. The goal of any study must be to apply cutting-edge stated-preference methods to measure and compare the treatment preferences of patients and caregivers for emerging treatments for DMD. These methods will be used to document meaningful benefits, acceptable benefit-risk tradeoff, and preference heterogeneity and patients are hopeful their Voice will be considered as regulatory agencies analyze and consider approvals.

PATRICIA FURLONG
President and CEO
Parent Project Muscular Dystrophy

16:15 - 17:00
KEYNOTE: COST-EFFECTIVENESS OF ORPHAN DRUGS

At the Norwegian Medicines Agency they are involved in the approval of orphan drugs and do cost effect analysis. Their mission is to evolve and safeguard public and animal health by ensuring the efficacy, quality and safety of medicines. In this session Steinar will elaborate on how they ensure that medicines marketed in Norway are safe and effective that cost-effective medicines are used.

STEINAR MADSEN
Medical Director, Norwegian Medicines Agency

17:00
EVENING NETWORKING PROGRAMME

AGENDA

DAY 3
September 23rd

08:45 - 09:30

KEYNOTE: VALUE BASED ASSESSMENT OF ORPHAN DRUGS - HOW DO WE VALUE INNOVATION WITH RARE DISEASES? THE PAYER'S PERSPECTIVE

OMAR ALI
NHS Pharmacy Payer & Adviser to NICE

LEARN

- *NICE Assessments for Highly Specialised Technologies - 6 key themes*
- *Health Technology Appraisals for Orphan Drugs - key challenges*
- *Does rarity justify a high price ? What is an acceptable cost per patient*
- *Case Examples - drivers for positive and negative NICE determinations with Orphan Drugs*
- *Orphan Cancers - new directions in Conditional Reimbursement, Commissioning through Evaluation and Outcomes Based Payer Models*

09:30 - 10:15

VIB'S IMPACT FROM SCIENCE TO PATIENTS – ONCURIOUS AS A CASE

Scientific academic breakthroughs require a strong focus and a set of activities with the goal of having an impact for patients. VIB is taking advanced initiatives in early drug discovery with partners and development to realize this potential, especially in the area of orphan indications. In this session, Jérôme, will discuss the case of Oncurious. This is a joint venture with Thrombogenics around the potential TB-403, a humanized monoclonal antibody targeting PLGF. TB-403 was originally developed based on a scientific collaboration and license from VIB and has now been repurposed to address a large unmet medical need in Medulloblastoma, an orphan pediatric cancer indication.

JÉRÔME VAN BIERVLIET
DVM, PhD, DACVIM, Senior Business Development Manager, VIB

LEARN

- *Exciting opportunities for orphan diseases by translational research on top of excellent basic research*
- *The potential to synergize with academic research, companies and/or charities*
- *Oncurious as a recent startup promising to realize new targeted therapy for patients currently underserved*

10:15 - 10:45

NETWORKING BREAK

AGENDA

DAY 3
September 23rd

10:45 - 12:15
LAB: PRICING & REIMBURSEMENT

10:45 - 11:05
DUTCH ORPHAN DRUGS POLICY - EU COLLABORATION REGARDING ASSESSMENT AND REIMBURSEMENT

In this session Ad Schuurman of the National Health Care Institute of the Netherlands explains the main missions and mandates of his organization with regards to the basic insurance package covering all Dutch citizens, as well as its perception of pay-for-performance models pan-European joint-reimbursement initiatives and Zorginstituut's policies on orphan drugs reimbursement and quality of care improvement

AD SCHUURMAN

Head of Business Contact Centre and International Affairs National Health Care Institute

11:05 - 11:25
AFFORDABILITY & MAINTAINABILITY: SOCIALLY RESPONSIBLE PRICING AND ENSURING PATIENT ACCESS TO TREATMENTS

Establishing a foundation for price that is balanced and sustainable while satisfying multiple parties with different interests is a big challenge. Drug development is very costly, but is the patient the one that has to pay the price with the risk of them not being able to access the medication they need?

LEARN

- *Foundation mechanisms for pricing*
- *Ensuring patient access to treatments*

10:45 - 12:15
LAB: MARKET ACCESS

10:45 - 11:05
DRUG REPURPOSAL AS AN ALTERNATIVE WAY FOR ORPHAN DRUG RESEARCH

If we know the pathway to target in a disease, we may find an appropriate out of label use of a drug to be used in rare disease. There is very few research in rare diseases (only around 10% of the rare diseases have some ongoing research). We have around 7.000-8.000 rare diseases, out of them less than 4% have some kind of treatment. The need to find new therapies is urgent. The program H2020 was claiming 200 new orphan drugs for 2020.

11:05 - 11:25
IS GLOBAL COLLABORATION THE ANSWER TO REACHING ALL POTENTIAL PATIENTS?

Some diseases are so rare that they only affect a handful of people in a certain country. With the lack of a central database designed specifically to list patient registries it is very hard to detect patients that suffer from the same disease. The questions is if global collaboration is the right answer to reaching patients and developing and identifying new treatments?

LEARN

- *Is global collaboration an answer?*
- *Developing & identifying new treatments through global collaboration*

LUISA-MARIA BOTELLA

Research Scientist, group leader in a group belonging to the Cell and Molecular Medicine department, specialty rare diseases, Centro de Investigaciones Biológicas CSIC

AGENDA

DAY 3
September 23rd

10:45 - 12:15
LAB: PRICING & REIMBURSEMENT

11:25 - 11:45
PRICING OF ORPHAN DRUGS: ESTABLISHING A REIMBURSEMENT FOUNDATION

Pricing of orphan drugs is a delicate question, since it needs to be balanced and sustainable. Establishing the right reimbursement foundation that satisfies the developer whilst enables equal access for patients is easier said than done. It asks for close collaboration between different stakeholders.

LEARN

- *How to establish a foundation for reimbursement*
- *How to price orphan drugs in a way that is balanced and sustainable*

11:45 - 12:15
PRICING & REIMBURSEMENT PANEL: WHAT IS THE RIGHT PRICE?

AD SCHUURMAN
Head of Business Contact Centre and International Affairs National Health Care Institute

10:45 - 12:15
LAB: MARKET ACCESS

11:25 - 11:45
STRATEGIES FOR REGULATORY FAST TRACKS AND GLOBAL MARKET ACCESS

Most rare diseases are serious conditions with a high unmet medical need. In order to speed up development there is a demand for regulatory fast tracks. But this does not come without a strategy. But what are these strategies? How can you work together with the regulatory bodies to secure global market access?

LEARN

- *What are strategies for regulatory fast tracks?*
- *How can you work together with regulatory bodies?*

11:45 - 12:15
MARKET ACCESS PANEL: ORPHAN DRUGS IN EMERGING MARKETS: HOW TO ENSURE MARKET ACCESS?

LUISA-MARIA BOTELLA
Research Scientist, group leader in a group belonging to the Cell and Molecular Medicine department, specialty rare diseases, Centro de Investigaciones Biológicas CSIC

12:15 - 13:00
KEYNOTE: DATA & INNOVATION

Data plays a more and more important role in healthcare. By collecting data we are getting more and easier insights into patients and possible treatments. In this session we will dive into the future of healthcare and discuss the impact of data and innovation.

LEARN

- *What role can data play in the development and discovery of orphan drugs?*
- *Practical examples of data driven medicine*
- *What can we expect in the future?*

13:00
END OF EVENT, ONE FOR THE ROAD

SPEAKERS

SPEAKER

MARTINE ZIMMERMANN

VP Global Regulatory Affairs, Alexion Pharma International

Martine Zimmermann PharmD. is VP global Regulatory Affairs at Alexion. She is based in Zurich. She has over 20 years of combined R&D and global regulatory strategy experience. She joined Alexion in 2009 and since then has been dedicated to the registration of Orphan Medical products as well as shaping a favorable regulatory environment for orphans Throughout her career, Martine has been directly involved in multiple regulatory approvals across the globe and most recently Strensiq approval in 2015 in Japan, Europe and US. ,Martine received her Pharm D from University Louis Pasteur in Strasbourg (France).

SPEAKER

ALAN THOMAS

Founder, Ataxia and Me

Alan Thomas is a patient engagement advocate for rare diseases in general and Ataxia in particular. He is known as the 'rare disease warrior' and, as a patient with a 'life-limiting' rare disease, he can convey the patient engagement message from his point of view in all issues regarding the wellbeing of patients. Alan has established a patient-driven and focused group and campaigns at many levels, including patient forums, local and regional health boards, Welsh/UK government organisations, as well as taking part in many global conferences, in person or via the internet. Ataxia means 'lack of order'. People with ataxia have problems with movement, balance and speech. Alan is fighting to return order to the lack of order. He was proud to receive a nomination from the Bevan Foundation in 2015 in the Health and Wellbeing category. Plus he participated in the Peer mentoring programme, run by Findacure, to progress Atacsia a Fi - Ataxia and Me©, with the introduction of www.areyouATAXIAaware.wales

SPEAKER

STEINAR MADSEN

Medical Director, Norwegian Medicines Agency

SPEAKER & ADVISORY BOARD

OMAR ALI

NHS Pharmacy Payer & Adviser to NICE

Qualified with a hospital pharmacy background, Omar has been working as the Formulary Advisor for Surrey & Sussex Healthcare NHS Trust for nearly 15 years. He sits on the regional Joint Drugs & Therapeutics Committee as well as the CCG/Commissioning Prescribing Clinical Network. Omar has been a visiting Lecturer at UCLH Pharmacy Programme and was both Lecturer & Examiner on the Independent Prescribing V300 Course at the University of Surrey and has over 30 publications to date. He is an Editorial Content Adviser to Guidelines and has recently been invited to the position of Associate Editor to the Canadian Journal of Population Therapeutics & Clinical Pharmacology. In 2010, Omar has served a position on the External Reference Group on Cost Impact Modelling for NICE for 5 years and in 2016 was appointed Panel Member for the newly formed Adoption & Impact Program for NICE. He advises foreign investors (US Embassy) on 'Value Based Assessments (hosted by the UK Department of Trade & Industry) and recently delivered a Healthcare NHS Reform program to over 40 Healthcare Insurance Provider delegates visiting from the US. Omar heads up an exciting new 'Medicines Management Payer Network for 2016/17; aims to provide Payers with a peer review of New Medicines & Pharma value propositions in a rapidly accessible, user friendly format. Most recently, he was invited to address the Italian Healthcare Senate on Pharmaco-economic Evaluation & Sustainability Models of Healthcare and featured at the Westminster Health Forum Keynote Seminars on the Future for Pharmacy Commissioning, Organisation & Delivery.

SPEAKER

HENK-WILLEM LAAN

MSc, Founder, MSS Research Foundation

Henk-Willem is founder of the MSS Research Foundation. His son Joas suffers from the ultra rare Marshall-Smith syndrome. Henk-Willem founded eight years ago the MSS Resarch Foundation and already raised over € 500,000 for research, awareness and fellow sufferer contact. With giving grants to researchers they already found the responsible gene NF1X. The foundation also has developed Standards of Care. With the (only) 40 families worldwide Henk-Willem has built a community where knowledge and experiences are shared. Henk-Willem is educated as a Fiscal Economist. He is now CFO/Deputy Director for Junis, a Child Care Organization. Besides his voluntary work for the foundation he has some voluntary Board positions in civil society.

SPEAKER

AD SCHUURMAN

Head of Business Contact Centre and International Affairs National Health Care Institute

Ad Schuurman completed his study in Utrecht as a clinical psychologist in 1980. He subsequently worked for five years as head of a patient's association. In the following years, as staff officer of a regional Institute for Mental Health, he organized the cooperation of the Regional Institutes for Mental Health and the other health care organisations. After spending several years as manager of the national project relating to pharmacotherapeutic decision making, he became head of a geriatric department. In the meantime he completed studies on management consultancy and on business administration (MBA). As deputy director of the Dutch College of General Practitioners (NHG), he set up the Electronic Prescription System. In recent years, as manager of the Pharmacy department, he was responsible for the pharmacy related activities of the CVZ. Since 2006 he was head of the Reimbursement Department, covering the healthcare reimbursement issues in the Netherlands. In 2006 he became President of the Medical Evaluation Committee (MEDEV) in Brussels, in which reimbursement authorities of 18 EU countries cooperate. Since 2010, Ad is Head of the Business Contact Centre and International Affairs of the Dutch HealthCare INsurance Board (CVZ).

SPEAKER

LUISA-MARIA BOTELLA

Research Scientist, group leader in a group belonging to the Cell and Molecular Medicine department, specialty rare diseases, Centro de Investigaciones Biológicas CSIC

Luisa-Maria got her Doctor in Biology and a PhD in Genetics from the University of Valencia in 1985. She is a Postdoctoral granted student from Lund University in Sweden and up to now she is a staff scientist in the National Research Council of Spain. Since 2002, Luisa-Maria is engaged in the Spanish HHT unit (translational research with Sierrallana/Valdecilla Hospitals in Spain), and with the patient associations. She is the main investigator in 5 consecutive projects that are devoted to the diagnosis, molecular basis and therapeutical approaches in HHT (Hereditary Hemorrhagic Telangiectasia). Since 2013 she is also investigating therapies for von Hippel Lindau Disease. During her career she has reached two orphan drugs designations by the EMA: raloxifene hydrochloride in 2010 (both EMA & FDA approved) and bazedoxifene acetate in November 2014 (EMA). She has written 40 papers dealing with HHT and more recently VHL from 2002 up to now. She has won an award from the Spanish Federation of Rare Diseases for her rare disease research in 2014 and another award from the National Research Council of Spain.

SPEAKERS

SPEAKER

JÉRÔME VAN BIERVLIET

DVM, PhD, DACVIM, Senior Business Development Manager, VIB

Jerome has a broad clinical medicine background with an advanced training in neurological diseases. By training, he is a Doctor in veterinary Medicine. He is widely published in clinical veterinary journals on neurological diseases of animals. He studied at the internal medicine residency at Cornell University and has broad certified as a diplomate for the American college of Veterinary medicine. Jerome got his PhD in Molecular Medicine at VIB / KU Leuven. He has strategic business consultancy experience from Bain and Company and strong business development experience at the interface of academic research, companies and investors. He is currently Senior Business Development Manager at VIB, an excellence-based entrepreneurial research institute in Belgium. He helped set-up and is now involved in the management of Oncurious, a VIB-Thrombogenics joint venture company centred around Medulloblastoma, an orphan disease.

SPEAKER

HOOTAN KHATAMI

M.D. Escala Therapeutics

Dr. Khatami is a University of California, San Francisco (UCSF)-trained endocrinologist, with prior research in proof of concept research in leptin and HIV-associated lipoatrophy, work that contributed to orphan indication for leptin and lipoatrophy. He has been CEO and founder of Escala Therapeutics, Inc. since its inception in 2015. His career includes positions of growing responsibility in early and late-stage clinical development and medical affairs for a range of metabolic products, including global leadership for the launch of mipomersen (KynamroTM) at Genzyme/Sanofi. Prior experience also included contributing to the development of sitagliptin (JanviaTM) at Merck & Co; launch and pre-launch planning at Roche/Genentech for Taspoglutide; global medical affairs leadership for biologics & biosimilars at Sanofi; and early development and licensing/partnering at Daiichi Sankyo. Dr. Khatami received his bachelors in French and Biology at the University of California, Berkeley; M.D. degree from Albany Medical College; and training in internal medicine residency at California Pacific Medical Center, followed by an Endocrinology fellowship at UCSF.

SPEAKER

PATRICIA FURLONG

Founding president & CEO, Parent Project Muscular Dystrophy

Pat Furlong is the Founding President and CEO of Parent Project Muscular Dystrophy (PPMD),the largest nonprofit organization in the United States solely focused on Duchenne musculardystrophy (Duchenne). Their mission is to end Duchenne. They accelerate research, raise their voices in Washington, demand optimal care for all young men, and educate the global community. Duchenne is the most common fatal, genetic childhood disorder, which affects approximately 1 out of every 3,500 boys each year worldwide. It currently has no cure.When doctors diagnosed her two sons, Christopher and Patrick, with Duchenne in 1984, Pat didn't accept "there's no hope and little help" as an answer. Pat immersed herself in Duchenne, working to understand the pathology of the disorder, the extent of research investment and the mechanisms for optimal care. Her sons lost their battle with Duchenne in their teenage years, but she continues to fight—in their honor and for all families affected by Duchenne. In 1994, Pat, together with other parents of young men with Duchenne, founded PPMD to change the course of Duchenne and, ultimately, to find a cure. Today, Pat continues to lead the organization and is considered one of the foremost authorities on Duchenne in the world.

SPEAKER

JOHANNA H. (HANNEKE) VAN DER LEE

MD, PhD

After graduation from medical school (Erasmus University Rotterdam, 1987), Hanneke van der Lee worked as an epidemiological researcher in general practice and in pediatrics (hemoglobinopathies and other congenital anomalies). Her PhD study on forced use of the upper extremity in chronic stroke patients (2001) was funded by NWO and performed at the department of rehabilitation medicine of the VU medical centre in Amsterdam. Hanneke has worked as an epidemiologist at the Pediatric clinical research Office in the Emma Children's Hospital AMC Amsterdam since 2003. Her main interests are systematic reviews, clinimetrics, and RCT methodology related to optimal sample sizes. Since July 2009 she has been the convener of the international Standard Development Groups on Data Monitoring Committees and Adequate Sample Sizes (www.starchildhealth.org). From 2011 to 2015 she participated in the EU FP7 funded Global Research in Pediatrics (GRiP) network of excellence (http://www.grip-network.org), and since October 2013 she has been the leader of work package 4 (Improved use of patient level information and perspectives) in the Asterix (Advances in Small Trials dEsign or Regulatory Innovation and eXcellence) consortium (http://www.asterix-fp7.eu).

SPEAKER

STÉPHANE DEMOTZ

Ph.D. Founder and CEO, DORPHAN

Dr. Stéphane Demotz studied biology at the University of Lausanne, Switzerland. Stéphane has over 20 years of experience in drug development gained in the pharmaceutical industry where he occupied various positions in start-ups and large companies (Roche, Pfizer and Glenmark). In 2008 Stéphane co-founded Edimer Pharmaceuticals, whose goal was the development of a drug for the rare genetic disease X-linked hypohidrotic ectodermal dysplasia. The drug candidate is currently undergoing phase 2 clinical trials in the US and Europe. In 2012, he co-founded DORPHAN, whose aim is the development of drugs for orphan and rare genetic diseases, more particularly in the field of the mucopolysaccharidoses. The company is now proceeding with the preclinical development and clinical set-up of its lead compound, a pharmacological chaperone drug candidate for the treatment of GM1-gangliosidosis and mucopolysaccharidosis IVB (Morquio disease type B).

ADVISORY BOARD 2016

The Advisory Board is instrumental in setting the tone and direction of the event, thanks to the accumulated years of experience of all the members within the Orphan Drugs community.

ADVISORY BOARD

DR. HOSS A DOWLAT

Vice President, Regulatory Affairs, Global Strategy, PharmaBio Consulting

Dr. Hoss A Dowlat has more than 31 years of drug development experience in the majority of therapeutic areas in the European and North American Pharma industry, 24 years of which have been in Regulatory Affairs of generics, biosimilars, and original drugs or biologics. Until 2010, Hoss was Vice President, Technical, Drug Development and Regulatory Global Strategy Services, at the leading CRO PAREXEL. In PAREXEL CONSULTING 2000-2010 he served 50 client companies from across Europe, America, Canada, Korea, Japan, and India, leading multidisciplinary teams of up to 40. He currently provides drug development, registration and due diligence support, for Pharma industry and Financial institutions with regional and international presence.

ADVISORY BOARD

JEAN NORDSTROM

CEO, Sixera Pharma

Industrialist and seasoned manager with 40 years of International Management positions in several Industry sectors. Last 25 years in Pharmaceutical and Biotech. Thorough experience from managing companies with substantial context in development and R&D. Have worked with large companies as well as SMEs. Former Executive President of Pharmacía Spain and member of the Corporate Management group, VP Corporate and Business Development at Arexis AB, Swedish Biotech Company. CEO of Labiana Group, Spanish CMO and producer of own products for human and animal health. Accustomed to work with companies with financial owners as well as industrial partners. Mr. Nordstrom has in parallel to his employments as executive also carried out various assignments as advisor in strategy and development issues. Since 10 years special interest in developing Orphan Drugs. Sixera Pharma is now main focus and Jean Nordstrom is also co-founder and shareholder of the company.

ADVISORY BOARD

OMAR ALI

NHS Pharmacy Payer & Adviser to NICE (Bio on page 13)

ADVISORY BOARD

ANDERS WAAS

CEO, Tikomed

Anders Waas has over two decades of experience in the pharmaceutical, biotech and medical device industries. Mr. Waas has been the Director of Karo Bio since July 2011 and serves as a member of the Biotech Investment Committee at Life Science Angels, Inc.. From 2004 to 2008, he was Vice President Business Development at CV Therapeutics in Palo Alto (US). Between 1992 and 2004 he held various senior management positions in product development, marketing and business development at Astra and AstraZeneca. These included: Director of Cardiovascular Global Licensing activities at AstraZeneca and Director of Strategic Planning and Business Development for cardiovascular, metabolic and gastrointestinal products at Astra. In this position he was responsible for the global strategic support of products such as Losec, Nexium, Toprol XL, Atacand, Exanta and for the overall R&D efforts in cardiovascular, metabolic and gastrointestinal diseases. Anders was also active in the medical device industry as European Sales Leader and Medical Advisor at WL Gore & associates. Dr Anders Waas received his DDS (Dr Dental Surgery) from University of Umea, Sweden.

ADVISORY BOARD

ELIZABETH VROOM

Founder and President Duchenne Parent Project

Elizabeth Vroom is founder and president of the Duchenne Parent Project Netherlands since 1995. This organisation has played an active role in drug development for Duchenne Muscular Dystophy. Elizabeth is Chair and co-founder of the worldwide organisation UPPMD (United Parent Projects Muscular Dystrophy). She serves on several advisory boards regarding Care, Research, Ethics, Development of new medicines and Regulatory Issues in the Netherlands as well as international. She received the Biofarmind Innovation Award for her "creative and innovative activities in the field of drug development". As President of the foundation 'The Meeting' she works on projects to optimize the role of patient organisations in drug development.

ADVISORY BOARD

MARLENE E. HAFFNER

MD, MPH, CEO, Haffner Associates, LLC (former Director of OOPD at US FDA)

Marlene E. Haffner, MD, MPH is the CEO of Haffner Associates, LLC a firm dedicated to the strategy, development and policy of drug development with a special emphasis on rare diseases and the products that treatment them. Prior to establishing her own company, in March 2009, she served as Executive Director, Global Regulatory Policy and Intelligence at Amgen, Inc. For 20 years, Dr. Haffner served as Director of the Office of Orphan Products Development (OOPD) of the Food and Drug Administration (FDA). As OOPD Director she was responsible for the leadership and management of the FDA orphan products development program, the first Orphan Products program in the world. She is well known as an expert in orphan drug development and is a sought after speaker and consultant in that area of regulatory science. In addition to her consulting activities Marlene is Adjunct Professor, Department of Preventive Medicine and Biometrics, and Clinical Professor, Department of Medicine, at the F. Edward Hébert School of Medicine, Uniformed Services University of the Health Sciences (USUHS) in Bethesda, Maryland. For 36 years she served in the United States Public Health Service beginning her career with the Indian Health Service in Gallup, New Mexico. She received her MD from the George Washington University School of Medicine where she then interned in Internal Medicine. She received further training in internal medicine, dermatology and hematology at the Presbyterian Hospital, New York and that the Albert Einstein College of Medicine, New York. She received an MPH from the Johns Hopkins University Bloomberg School of Public Health. During her Public Health career, she rose to the rank of Rear Admiral in the USPHS. Dr. Haffner has received many awards for her work in drug development including The Outstanding Contributions to Pharmaceutical Medicine Award from the American Academy of Pharmaceutical Physicians, and in May 2009, the Woodrow Wilson Award for Outstanding Government Service from the Johns Hopkins University.

ADVISORY BOARD & CHAIRMAN

ROBERT DERHAM

Founder & President, CheckOrphan

Robert Derham is the founder and President of CheckOrphan (501c3 and registered non-profit in Switzerland), which is best described as the CNN/BBC for rare diseases. In the past, he has successfully launched interactive and dynamic life-science-based websites for the following companies: Axxora, BioValley, mondoBIOTECH, Novartis, and Syngenta. Robert is also a member and co-founder of the Global Web-Strategy Network, which consists of representatives from international companies such as: Novartis, Credit Suisse, UN, Nestlé and more. Robert blends 12 years of industry experience and 7 years of research experience, which helps to understand the evolving field of rare diseases and healthcare.

OUR DELEGATE'S VOICE

Great sharing of key insights by knowledgeable people.

DIRECTOR MEDICAL AFFAIRS, MEDIVIR

Highly informative meeting with the bonus of great networking opportunities covering a wide scope hoss dowlat, pharmabio consulting
Great meeting feels like a family reunion.

FOUNDER VP BD, TIKOMED AB

"Well done conference. Good venue. Good participants. Good agenda. Look forward to attending again next year!"

MANAGING DIRECTOR, BAY CITY CAPITAL

The annual Copperberg orphan drug event always tackles a wider range of subjects and attracts a wider range of delegates than other industry conferences on the topic making it very interesting, as well as enjoyable, to attend. Global

PHARMACEUTICAL BUSINESS ANALYST, DM PHARMA ISSUES LTD

Great opportunity to share and discuss challenges and personal experiences with all key stakeholders in the OD universe.

PRESIDENT EUROPE, MEDICAL MARKETING ECONOMICS - MME

WELCOME TO THE SOCIAL CLUB

September 22nd 2016

17:00

Amsterdam

COCKTAIL

DINNER

REGISTER AND STAY UPDATED

CHOOSE YOUR TICKET

Academic/Government	1090 EUR
Pharma/Biotech	1890 EUR
Solution Provider/Consultancy	3490 EUR

* Full Networking includes cocktail reception, all networking breaks and lunches, one banquet dinner, and access to pre-event meeting system to book meetings with other participants

JOINING IN A GROUP?

Our agenda is the perfect opportunity for you to bring a cross-functional team.

When attending as a team of five or more you'll receive additional benefits,

Book 1	= 0% discount
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TERMS & CONDITIONS

Your booking is binding. You may substitute a delegate at any time. Please note that substitutions are not permitted unless approved by the organizers. For all cancellations (without an approved substituted delegate) received in writing more than 5 business days prior to the event and, a €120 (+VAT) administrative fee will be charged and a credit voucher for the remaining amount will be issued. Credit vouchers may be used at any Midfield Media conference within one year of issuance. For cancellations less than 5 business days prior to the event, the full amount of the delegate pass is non-refundable. Full payment is due 10 days upon invoice and no later than 5 business days prior to the event. Delegates that have NOT submitted payment prior to the event will not be admitted to the event. Admittance is then only granted upon approval of credit card payment directly onsite.

VENUE
NOVOTEL AMSTERDAM SCHIPHOL AIRPORT

ABOUT Novotel Amsterdam Schiphol Airport, is a beautiful designed hotel with a great ambiance and on a very convenient location.

ACCOMODATION We have a prefered rate at the hotel for our delegates and partners.

ATTIRE Business Casual attire is recommended for the conference and evening events.

AT THE EVENT The conference ticket includes all refreshments and any evening activities for all attendees. Other costs such as travel, accomodation, airport transfers and general expenses are the responsibility of the individual delegate.

GET TO THE VENUE When coming by train or plane, take the train to Hoofddorp Station which is located at only 200 metres from the hotel.

The hotel has 652 daily connections with the airport (4 min by train & 9 min by bus). Hoofddorp station is located 200 m from the hotel - only 20 min from the heart of Amsterdam!

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EVENT CO-ORDINATION & LOGISTICS

All Copperberg conferences are run by Coordinatum Business Event Management

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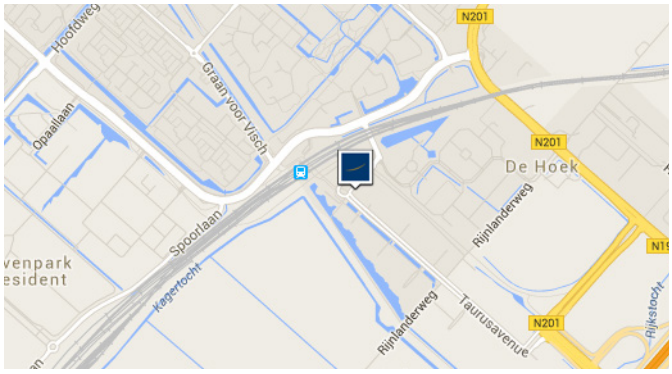

SIXTH
ANNUAL FORUM

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