Strategic Drug Repositioning and New Indications Forum
Exploit Opportunities and Accelerate R&D by Re-profiling Your Drugs

Charing Cross Hotel London, United Kingdom
26th – 27th September, 2013

Unlock Your Pipeline Potential By Re-Discovering Your Compounds’ Capabilities

Half-Day Interactive Workshop on 26th September 2013
Open Innovation for Drug Repositioning
Led By:
Bruce E. Bloom
President & Chief Science Officer
Cures Within Reach

Interactive Panel Discussions Include
Problems Associated with Traditional Drug Development – A Need for Change in Strategy
Potential of Funding for Halted Drugs

Attending This Premier marcus evans Conference Will Enable You to
• Explore the latest screening platforms to find new indications
• Benefit from case studies on successfully repositioned drugs
• Maximise your knowledge of patent protection
• Learn about reformulation of existing compounds
• Ensure success with new indications for already marketed products

Learn from Key Practical Case Studies
• Johnson & Johnson on using open innovation to drive drug repositioning
• Shire AG on data mining for identification of potential targets
• Minoryx Therapeutics S.L. on drug repositioning for rare diseases
• SOM Biotech on development of new treatments from already marketed drugs

In the Chair
Nigel McCracken
Senior Director, Translational Medicine
Shire AG

Expert Speaker Panel
Graeme Wilkinson
Associate Director, New Opportunities iMed
AstraZeneca
Nigel McCracken
Senior Director, Translational Medicine
Shire AG
Raúl Insa
CEO
SOM Biotech
Fred Marín
CEO
GMP-Orphan SAS
Sasha Alexandre Akoulitchev
Scientific Director
Chronos Therapeutics
Hannah Tipney
Computational Biologist
GlaxoSmithKline
Andrew Doig
Professor of Biochemistry
The University of Manchester
Theo Meert
Senior Director
Johnson & Johnson
Marc Martinell
Founder & CEO
Minoryx Therapeutics S.L.
Valery Alakhov
Vice President R&D & CSO
Supratek Pharma Inc.
William Garner
CEO
EGB Advisors, LLC
Bruce E. Bloom
President & Chief Science Officer
Cures Within Reach
Michael R. Jackson
Vice President Drug Discovery & Development
Sanford-Burnham Medical Research Institute
Day One

26th September 2013

08:30 Registration and Coffee

09:00 Opening Address from the Chair
   Nigel McCracken
   Senior Director, Translational Medicine
   Shire AG

09:10 Opening Address
   Drug Repositioning Informed by “Disease in a Dish”
   Phenotypic Screening of Patient Derived Cells
   • Inherited diseases – loss of functions at the cellular level in patient derived cells
   • Image-based phenotypic assays to generate “disease in a dish” screens
   • Reversing the cellular dysfunction
   • Phenotypic screens – representing a holistic approach to drug repurposing for inherited disease
   Michael R. Jackson
   Vice President Drug Discovery & Development
   Sanford-Burnham Medical Research Institute

09:50 Drug Discovery and Repositioning for Alzheimer’s Disease
   • Rational design of amyloid aggregation inhibitors
   • Screening drug libraries to reduce amyloid toxicity
   • Example of a Parkinson’s drug repositioned for Alzheimer’s
   • Commercialisation with PharmaKure
   Andrew Doig
   Professor of Biochemistry
   The University of Manchester

10:30 Business Card Exchange
   An early opportunity in the conference to meet all other conference attendees by systematically moving around the room. Make use of the opportunity to learn about companies, projects and backgrounds of other attendees and swap business cards in an informal environment

10:40 Coffee and Networking Break

11:10 Case Study
   Data Mining for Identification of Potential Targets and Repositioning Candidates
   • Accessing information from public databases
   • Understanding the potential of off target interactions
   • Predicting success rates
   • What is the best software being used?
   Nigel McCracken
   Senior Director, Translational Medicine
   Shire AG

11:30 Case Study
   Challenges and Opportunities in Computational Drug Repositioning
   • Using computational techniques to detect and classify drug repositioning potential
   • The application of our own and publicly available approaches for systematic drug repurposing
   • Use cases and proof-of-concept examples
   • What are the challenges and opportunities?
   Hannah Timpney
   Computational Biologist
   G laxoSmithKline

11:50 The Promise of Drug Repositioning
   • What makes a repositioned drug successful?
   • How are we to overcome the barriers to succeed?
   • Who has helped to make improvements in the field?
   • What does the future hold?
   Moderated by:
   William Garner
   CEO
   EGB Advisors, LLC

12:30 Lunch

13:30 Case Study
   Challenges and Opportunities in Computational Drug Repositioning
   • Using computational techniques to detect and classify drug repositioning potential
   • The application of our own and publicly available approaches for systematic drug repurposing
   • Use cases and proof-of-concept examples
   • What are the challenges and opportunities?
   Hannah Timpney
   Computational Biologist
   G laxoSmithKline

14:10 Case Study
   Using Open Innovation to Drive Drug Repositioning – AZ-MRC and NIH NCATS Initiatives
   • New plans for boosting drug development
   • Updates on progress of AZ-MRC and NIH initiatives
   • What are academic researchers offering?
   • What is the funding plan for this programme?
   • What does the industry partner bring?
   • Key considerations from an industry perspective
   Graeme Wilkinson
   Associate Director, New Opportunities iMed
   AstraZeneca

14:50 Coffee and Networking Break

15:20 Problems Associated with Traditional Drug Development – A Need for Change in Strategy
   • Staggering costs of developing new drugs
   • Long time frameworks – time span from R&D to market launch
   • Handling risks of failure during development
   • Problems with gaining regulatory approval
   • From identifying active chemical ingredients to target identification of compounds
   Panelists:
   Graeme Wilkinson
   Associate Director, New Opportunities iMed
   AstraZeneca
   Fred Marin
   CEO
   GMP-Orphan SAS
   Raúl Insa
   CEO
   SOM Biotech
   Moderator:
   Nigel McCracken
   Senior Director, Translational Medicine
   Shire AG

16:00 Closing Comments From Chair and End of Day One
**Open Innovation for Drug Repositioning**

26th September 2013

16:10 Workshop Leader Introduction

16:25 **Academic Input for Finding New Targets**
- New technological developments
- Tapping into new ideas from researchers
- Identifying new medical opportunities

17:40 Coffee and Networking Break

18:10 **The Industry Interaction**
- Progressing to advanced clinical trials
- Commercialisation of the product
- IP rights for each party

19:10 Closing Remarks from the Workshop Leader

*Led By:*

Bruce E. Bloom
President & Chief Science Officer
Cures Within Reach

**What You Will Learn in the Workshop**

In this workshop we will discuss how open innovation can drive drug repositioning. It will show the novel cutting-edge and innovative technologies being developed by academic institutions. With tightened budget and time pressure, the industry is embracing the unlimited potential of repurposing drugs through open innovation with academics. Delegates will share their experience in how to accelerate clinical trials as well as commercialising the products. We will also address how to manage IP ownership in such an environment.

**About the Workshop Leader**

**Bruce E. Bloom**

Bruce is the President and Chief Science Officer of Cures Within Reach, a charitable organisation that funds medical Rediscovery Research™. It uses existing science and medicine to quickly, safely and affordably create new treatments for patients with catastrophic diseases. Dr. Bloom and the leadership team at Cures Within Reach bring funds and researchers together to inspire and carry out rediscovery research projects that often touch patients’lives in two years or less by repurposing drugs and devices, modifying treatment protocols and scientifically testing clinical observations. Hundreds of thousands of patients with life-altering diseases already have new and better rediscovery research treatments as a direct result of the work of Cures Within Reach.

**Learning Target**

- You learn about academic and industry input towards the open innovation strategy
- New and exciting innovative technologies being developed
- Exploring and identifying new targets
- Working towards clinical trials and commercialisation of the products

- You can address your questions to the group and get answers from other experienced workshop participants
- You can also share some of your own ideas and approaches to the benefit of the audience

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**marcus evans conferences**

Producers of high quality business to business conferences designed to address the strategic information needs of senior executives. Speakers are practitioners from international blue-chip organisations and financial institutions, as well as business schools, academic bodies and government ministries. More than 1,000 conferences take place each year, attracting over 100,000 delegates and 16,000 speakers.

**marcus evans summits**

Producers and promoters of over 150 of the world’s leading business and economic summits every year for senior decision makers at exclusive locations around the world. These events provide attendees with a unique opportunity to access the latest developments in their chosen industry and to network in a structured environment with leading industry practitioners.

**marcus evans professional training**

Sector Focused and General Management Training through high quality courses, videos and CD-ROMs enables organisations of any size and geographical location to benefit from working with marcus evans for all their training needs. The clients’ demands for high quality hands-on training drives the focus for content, and thorough research ensures a compatibility with current business concerns.

**marcus evans language training**

marcus evans linguarama offers effective training in language, communication and culture for business and professional people. With more than 30 years’ experience and over 20 centres in Europe, we are one of Europe’s largest business language and communication training organisations. Each year marcus evans linguarama trains people from over 60 countries worldwide and provides over 1,000 companies and organisations with total training solutions where our courses are focused entirely on the needs of the individual participant or group and have practical relevance to business and professional life.

**marcus evans corporate hospitality**

Specialists in corporate hospitality linked to the premier international sporting events around the world. Trading as The Hospitality Group (thg), Sports Marketing Group (smg) and International Championship Marketing, the event diary is nothing less than an international sporting directory.

**marcus evans congresses**

marcus evans congresses bring together the leading vendors and decision makers from a wide range of services and industries. Each event provides an opportunity for key suppliers to exhibit and demonstrate their products to the region’s key professionals. In addition to the exhibition format the event’s educational conference is attended only by delegates who are pre-qualified, in most cases to a minimum spend of US$5 million and many with budgets in excess of $50 million.
Day Two

27th September 2013

08:30 Registration and Coffee

09:00 Opening Address from the Chair

Nigel McCracken
Senior Director, Translational Medicine
Shire AG

EFFECTIVE BUSINESS STRATEGIES FOR A PROMISING FUTURE

09:10 Opening Address
Repositioning Oncology Drugs Through the Optimisation of Drug Development and Regulatory Processes Using Novel Drug Delivery Approaches

• Evaluating weakness issues of oncology drugs with clinical history that are related to ADME problems and resolving these issues by reformulating the product
• Regulatory aspects and ways to accelerate the development process
• Using the previous history of the drug to reduce the development risk factors, and increase the attractiveness of the new candidate for licensing

Valery Alakhov
Vice President R&D & CSO
Supratek Pharma Inc.

09:50 Case Study
Development of New Treatments from Already Marketed Drugs

• A lower risk alternative
• Identifying new market trends and unmet medical needs
• Methodologies to generate ideas from approved drugs

Raúl Insa
CEO
SOM Biotech

10:30 Coffee and Networking Break

REPOSITIONING DRUGS FOR INCREASED VALUE

11:00 Case Study
Repositioning of Drugs – Value For Large Pharma

• Is there a role for drug repositioning in neuroscience?
• The issues and consequences in different types of drug repositioning based on developmental candidates and commercial products
• Possibilities in open/external innovation

Theo Meert
Senior Director
Johnson & Johnson

11:40 Case Study
Drug Repositioning in Rare Diseases

• Benefits from orphan drugs and market exclusivity
• Challenges of repositioning in orphan indications
• Finding repositioning opportunities in rare diseases

Marc Martinell
Founder & CEO
Minoryx Therapeutics S.L.

12:20 Lunch

13:20 Coffee and Networking Break

13:50 Case Study
A Screening Library for Rare Disease Drug Candidates

• New repositioned drug candidates
• Anti-aging repositioned drugs
• Detection and evaluation of biological activities
• Further screening analysis

Sasha Alexandre Akoulitchev
Scientific Director
Chronos Therapeutic

14:30 Case Study
Repositioning Off Patent Already Registered Molecules In The Orphan Arena – A Real-Life Business Approach

• How to identify compounds of interest
• How to put in place your IP strategy
• Regulators feedbacks
• Is premium pricing hopeless?

Fred Marin
CEO
GMP-Orphan SAS

15:10 Coffee and Networking Break

FUNDING AND REIMBURSEMENT FOR DRUG REPOSITIONING

15:40 Monetising Strategies for Generic Drug Repositioning

• Off-label use versus marketing approval
• IP strategies
  – Method of use patents, regulatory exclusivity, orphan drug opportunities
  – Reformulation, combinations of generic drugs, combining generic drug with patent drug, delivery alternatives
  – Creating new analogs from generic drug success
• Non-IP strategies
  – ROI from healthcare cost savings
  – Creating profitable alternate generic drug manufacture and delivery mechanisms
  – Government programmes
  – Partnerships with advocacy groups

Bruce E. Bloom
President & Chief Science Officer
Cures Within Reach

16:20 ROUND TABLE DISCUSSION

Potential of Funding for Halted Drugs

• Establishing collaborations with repositioning companies
• How to convince the decision makers for further funding for failed drugs
• How is the EU helping with funding for drug repositioning?

Led by:

Theo Meert
Senior Director
Johnson & Johnson

17:00 Closing Comments from Chair and End of Conference

Business Development Opportunities:

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

Ola Samuelsson, Marketing Manager, marcus evans London
Tel: +44 (0) 20 3002 3276 E-Mail: OlaS@marcusevansuk.com
Graeme Wilkinson
Graeme Wilkinson is an Associate Director in the emerging innovations function at AstraZeneca. Graeme is a pharmacologist by training and has been with AstraZeneca for almost 17 years where he has wide experience of leading discovery and early development projects. For the past two and a half years, he has led the drug repositioning program within AstraZeneca’s new opportunities group (now Emerging Innovations) with the remit of identifying and progressing into clinical development new projects for existing AstraZeneca and MedImmune clinical stage small molecules and biologics. A central theme of these repositioning activities has been to engage with the academic community to widen the scope of ideas through open innovation. In his talk, Graeme will discuss the ground breaking alliances with the MRC and NIH and reflect on progress and learning to date for the future of such partnerships between the Pharmaceutical sector and Academy.

Bruce E. Bloom
Bruce E. Bloom holds a JD from Chicago Kent College of Law, a DDS from University of Illinois Medical Center, and a BS in Biology from University of Illinois Urbana. Bruce E. Bloom’s business and entrepreneurial experience spans both not-for-profit and for-profit work in medical research, law, healthcare, insurance, regulatory affairs, product development, food service, art, and education. He is currently President and Chief Science Officer of Cures Within Reach, a 501c3 that funds medical Rediscovery Research™ that uses existing science and medicine to quickly, safely and affordably create new treatments for patients with catastrophic diseases. Bruce E. Bloom and the leadership team at Cures Within Reach bring funds and researchers together to inspire and carry out Rediscovery Research projects that often touch patients’ lives in two years or less by repurposing drugs and devices, modifying treatment protocols and scientifically testing clinical observations.

Marc Martinell
Marc Martinell is founder and CEO of Minoryx Therapeutics, a company committed to finding innovative treatments for life threatening rare diseases combining the development of pharmacological chaperones with repositioning-based projects. Marc has a broad experience in drug discovery and biotechnology, and prior funding Minoryx he participated in companies such as Crystax Pharmaceuticals and Oryxon Genomics. In these companies, Marc occupied positions of high responsibility managing several research projects and directing the group of scientists in charge of target selection, structural biology, computational chemistry and hit ID through a fragment-based approach.

Raúl Insa
Raúl Insa, MD, PhD, MBA, is the CEO and Founder of SOM Biotech, a start-up biopharmaceutical company established in the Barcelona Scientific Park and specialized in drug repositioning through an in-silico solution. Previously, he has been working for more than 20 years in multinational pharmaceutical companies, such as Parke-Davis (now Pfizer), UCB Pharma, Uriach Group (now Palau Pharma) and ISDIN (from Esteve Group). Raúl received his Medical training and his PhD in Clinical Neurology at the University of Alicante, Spain; a MBA from ESADE Business School in Barcelona and an Executive Education Program from IESE, Barcelona. He has also attended a couple of biotech leadership programs from Harvard Business School, Boston.

Andrew Doig
Andrew Doig graduated in Chemistry from the University of Cambridge, where he stayed to complete his PhD on protein folding and peptide binding with Professor Dudley Williams, FRS, in 1991. He then carried out post-doctoral research in the Biochemistry Department at Stanford with Professor Robert Baldwin before joining UMIST in 1994. He is now a Professor of Biochemistry in the Faculty of Life Sciences at the University of Manchester. In addition to being an expert on Alzheimer’s disease and protein structure, Professor Doig was one of the founders of Senexis, a Cambridge based company focussed on novel treatments for diseases resulting from the toxicity of amyloid-like proteins and is a founder of PharmaKure, a new company focussed on drug repositioning for Alzheimer’s Disease. Other research interests include: de-immunising fluorescent proteins; properties of essential genes for mammalian development; effects of –amyloid and amylin on the proteome and metabolism; determination of protein structure from vibrational spectra; properties of drug target proteins; graph theory and the Maximum Independent Set problem; how mutations in tau affect the cytoskeleton; and the nature of mutations that induce amyloidoses.

Hannah Tipney
Hannah Tipney graduated in Anatomy & Developmental Biology at University College London (UCL), before completing her MSc and PhD at University of Manchester focusing on the exploration of bioinformatic approaches to understand the genotype-phenotype relationships in Williams-Beuren Syndrome with Dr May Tassabehji, Prof Andy Brass & Prof Carole Goble. As a post-doctoral Fulbright scholar at the University of Colorado, Denver, Hannah worked with Prof. Larry Hunter on the development and application of computational methods to leverage knowledge in publications and databases to interpret genome-scale experimental data. She is currently an Investigator in Philippe Sanseau’s Computational Biology group at GlaxoSmithKline R&D Ltd, and a member of the GSK Computational Biology Systematic Drug Repositioning team lead by Pankaj Agarwal.