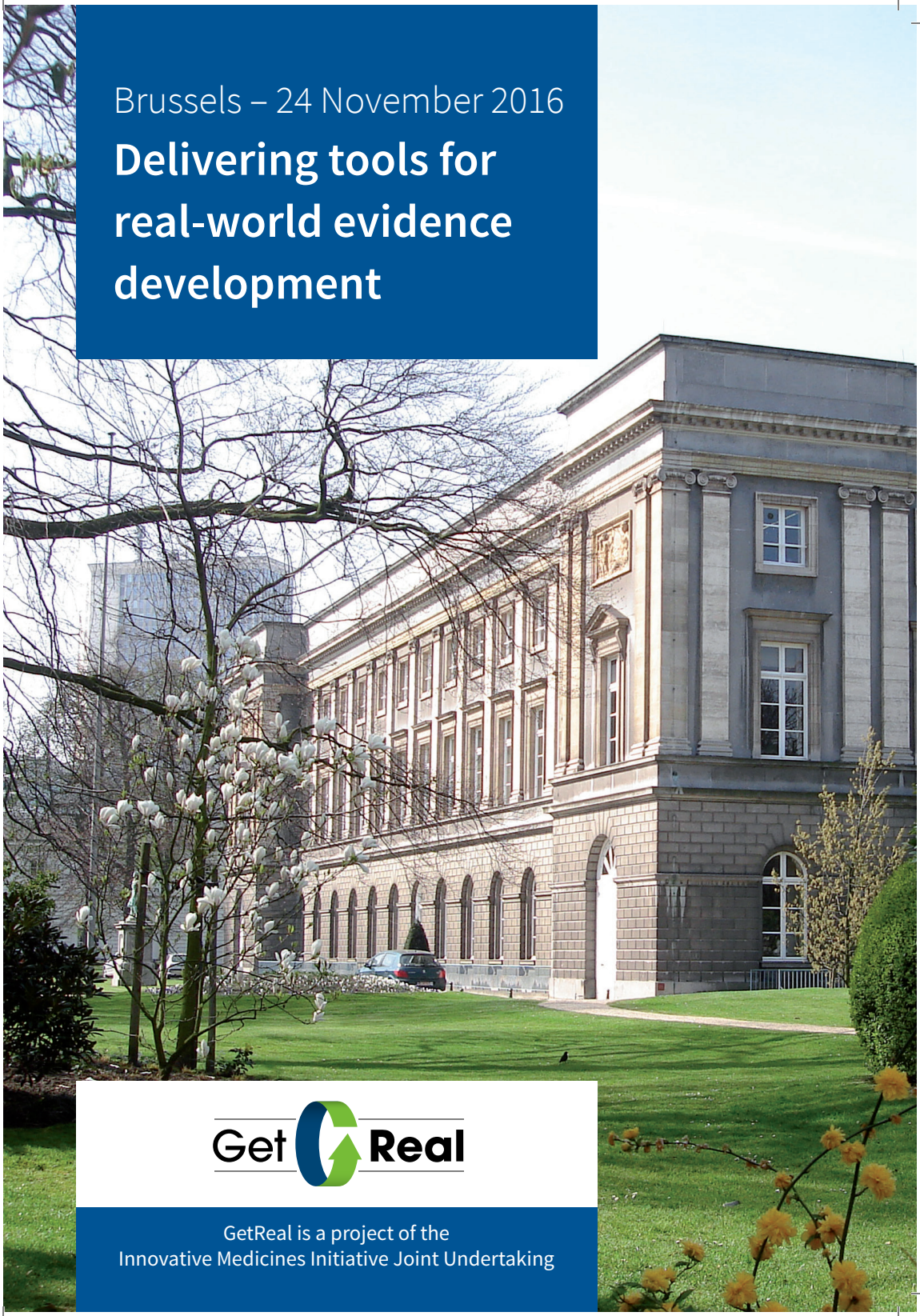

Brussels – 24 November 2016

# Delivering tools for real-world evidence development

GetReal

GetReal is a project of the
Innovative Medicines Initiative Joint Undertaking

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Royal Flemish Academy of Belgium for Science and the Arts

## Foreword

Welcome to the final meeting of the IMI 1 GetReal consortium entitled "Delivering tools for real-world evidence development"!

It is our pleasure to welcome you today to the Royal Flemish Academy of Belgium for Science and the Arts. The Academy was established in 1772 to facilitate the societal debate on scientific and artistic topics in a neutral and interdisciplinary way. As such, it is an excellent venue for a GetReal meeting which includes stakeholders from a wide variety of backgrounds to address a topic of scientific and societal relevance.

The IMI GetReal project was launched in October of 2013 with the aim to determine how robust new methods of real-world evidence (RWE) collection and synthesis could be adopted earlier in pharmaceutical R&D and in the healthcare decision making process. The ambition being that earlier availability of robust effectiveness data would lead to more informed decision making by industry, regulators, HTA bodies, payers as well as inform physicians and patients with regard to available treatment options.

Today we want to take the opportunity to showcase the results from our project: innovative methods for effectiveness research as well as the tools and frameworks that we have created to make these methods available to a wide range of decision makers. The day we will include a mixture of presentations from GetReal members, panel discussions to seek perspectives from our key stakeholders and also interactive demonstrations during the lunch break.

We'd like to thank you for taking time out of your busy schedule to join us today,and hope that you will enjoy the day, sharing views and insights that can inspire all of us in our work to increase the value of RWE generated during medicines development and to help make better decisions for patients!

Elaine Irving
GSK
Coordinator IMI GetReal

Rick Grobbee
University Medical Center Utrecht
Managing Entity IMI GetReal

# About IMI GetReal

Launched in October 2013, GetReal is a three-year project of the Innovative Medicines Initiative (IMI), a EU public-private consortium consisting of pharmaceutical companies, academia, HTA agencies and regulators (e.g., NICE, HAS, EMA and ZIN), patient organisations and SMEs.

GetReal aims to show how robust new methods of RWE collection and synthesis could be developed and considered for adoption earlier in pharmaceutical R&D and the healthcare decision making process. This will require companies, healthcare decision makers and other stakeholders to work together to generate a consensus on best practice in the use of RWE in regulatory and reimbursement decision-making.

GetReal is carrying out work within four work packages (WPs) to develop intelligence, evidence, tools, techniques and training to realise the full potential of RWE:

- WP1: Collaborating with key stakeholders in medicine development to assess: the acceptability and usefulness of Real World Evidence (RWE), and approaches to the analyses of RWE, in estimating the effectiveness of new medicines.
- WP2: Studying the scientific validity of RWE study designs and analytical approaches, to better inform pharmaceutical R&D and healthcare decision makers on their potential for use in assessment of effectiveness.
- WP3: Identifying the operational challenges of performing RWE studies early in the medicine development process and developing practical solutions to better inform their planning and delivery.
- WP4: Identifying and sharing best practice in evidence synthesis and predictive modelling of different types of data to estimate effectiveness of medicines.

For more information see our website: www.imi-getreal.eu

# Agenda

IMI 1 GetReal closing meeting:
## Delivering tools for real-world evidence development
24 November 2016, Royal Flemish Academy of Belgium for Science and the Arts, Brussels, Belgium

**09:30 – 10:30** Coffee and registration

**10:30 – 10:35** Welcome on behalf of the GetReal project
- Elaine Irving, GSK
- Rick Grobbee, UMCU

**10:35 – 10:45** Real-world data and the IMI GetReal project
- Elaine Irving, GSK
- Rick Grobbee, UMCU

**10:45 – 12:25** Insights from GetReal: what has our collaboration delivered?

WP1: Working with stakeholders to explore alternative evidence generation: What have we learnt?
- Pall Jonsson, NICE
- Rob Thwaites, Takeda

WP2: Identifying 'gaps' and signposting solutions for efficacy and effectiveness
- Chris Chinn, Sanofi
- Lucien Abenhaim, LASER Analytica

WP3: Making pragmatic trials work – Identification of operational challenges and development of a decision support tool for pragmatic trial design
- Iris Goetz, Eli Lilly
- Mira Zuidgeest, UMCU



WP4: Evidence synthesis and predictive modelling of relative effectiveness – Paving the way to best practice
- Chrissie Fletcher, Amgen
- Matthias Egger, University of Bern

**12:25 – 12:30** Intro to demos of GetReal tools
- Elaine Irving, GSK
- Pieter Stolk, UMCU

**12:30 – 14:00** Lunch and demos/try-out of GetReal tools and outputs
*(for more information, see page 8 and 9)*
- WP1: RWE Navigator
- WP2: Toolbox showing innovative methodological techniques and tools for a better anticipation of effectiveness before launch.
- WP3: PragMagic tool and the JCE paper series
- WP4: ADDIS and high level summaries of research as applied in a series of case studies
- WP5: GetReal online course 'Real-world evidence in medicine development'

**14:00 – 15:15** Panel discussion 'Real-world data and drug development: how has GetReal helped?'

**15:15 – 15:35** Coffee and tea

**15:35 – 16:50** Panel discussion 'Getting real about the future of real-world data research'

**16:50 – 17:00** Wrap up
- Elaine Irving, GSK
- Rick Grobbee, UMCU

# GetReal tools | Market of ideas

**WP 1**

**WP 1 | Meet the creators of the RWE Navigator**

Work package 1, tasked to produce a framework to aid the design of drug development strategies, have created a **web-based decision-support tool** called the **RWE Navigator**. This tool has 3 main aims: to give users a background on the current real-world data policies and perspectives in Europe; to take users through the potential issues of demonstrating effectiveness of medicines ('effectiveness issues'); and to take users through potential RWE options to address these effectiveness issues. The tool pulls together the research outputs across GetReal into a coherent framework, showing users the potential for RWE to support development of new medicines. The development team will be on hand to demonstrate the tool and discuss how it can be useful for anyone interested in exploring the role of RWE in effectiveness research.

**WP 2**

**WP 2 |**

During the last 3 years, the WP2 has developed and tested methods to identify drivers of effectiveness, early during drug development. With its focus on methods to be applied in traditional RCTs and randomized pragmatic trials, the WP2 has also developed and tested innovative methods such as: (1) the enrichment of RCT population to be combined with predictive modeling, to better estimate effectiveness before launch, (2) the use of tailored statistical methods to measure drugs effect size in randomized pragmatic trials (trials within cohorts, multiplicity of comparators, etc.) and (3) methods to control for channeling bias at a moment of a new drug's launch. During the closing meeting, WP2 participants will **showcase these innovative methodological techniques** and tools, for a better anticipation of effectiveness before launch.



**WP 3**

**WP 3 |**

WP3 will show a look-and-feel version of PragMagic, **a decision support tool to facilitate the design and planning of pragmatic trials.** It will provide trial designers with insights to better anticipate potential operational challenges. It will also help to balance generalisability of the trial results to routine practice with the operational feasibility of the trial and other relevant implications, including trial validity and stakeholder views. Participants can run through the tool, guided by one of the tool developers.

**WP 4**

**WP 4 |**

Come to the WP4 stand to learn how to conduct network meta-analyses (NMAs) using the **ADDIS software,** including:

- how to import data to form the network,
- how to set up your NMA model,
- how to interpret model diagnostics, and
- how to conduct sensitivity analyses.

Also WP4 will be showcasing high level summaries of the research as applied in a series of case studies.

**WP 5**

**WP 5 |**

WP5 offers a sneak preview of the **online course** 'Real-World Data in Medicine Development' which was developed by content experts from different parts of Europe. Learn more about the medicine development landscape, Real-world evidence generation & synthesis, and decision-making and weighing evidence. Try out the different learning activities yourself and scroll through course materials such as web lectures, quizzes and discussion forums.

# Speaker info

## Lucien Abenhaim

LASER Analytica, Chairman

Lucien Abenhaim holds an MD, an MSc (Experimental Medicine) and a PhD (Information Science). He founded the LASER Group in 2004 after an outstanding career in academia and in public health, marked by the following milestones:

- Expert at the EMA, the FDA and several European and Canadian agencies
- Professor of Epidemiology & Biostatistics, McGill University, Canada (1988-2005)
- Co-founder and Director of McGill Pharmacoepidemiology Education Program (1988-2005)
- General Director of Health (Chief Medical Officer) for the French Ministry of Health (1999–2003)
- Member of the Executive Council and of the General Assembly of the World Health Organisation (WHO), United Nations, Geneva (2001–2003)
- Honorary Professor of Epidemiology, London School of Hygiene & Tropical Medicine, UK (2005-present)

## Chris Chinn

Sanofi, Head of Real World Data Strategy and Partnerships

Chris graduated from Oxford University with a degree in Biochemistry. He qualified as a chartered accountant with Ernst & Young in London and completed a MSc in Health Economics at City University, London. He has led health outcomes research teams at Eli Lilly and GSK, and in his current role at Sanofi, Chris leads Real World Data Strategy and Partnerships. Chris is also the deputy Coordinator of the IMI GetReal consortium.



## Matthias Egger

ISPM, University of Berne, Professor of Epidemiology & Public Health and Chair

Matthias Egger heads the Institute of Social & Preventive Medicine (ISPM) at University of Berne. His research is concerned with methodological and substantive issues in clinical epidemiology and public health, with a focus on analyses of large cohort studies and meta-analytical research in infectious diseases and cancer. In the last 5 years he has published over 150 original articles, commentaries, and reviews and throughout his career he has made contributions to teaching both at the undergraduate and postgraduate level.

## Chrissie Fletcher

Amgen Ltd, Executive Director Biostatistics and Regional Head, Global Biostatistical Science

Chrissie is a Regional Head in Global Biostatistical Science at Amgen and she leads a Health Technology Assessment (HTA) Biostatistics group. Chrissie has worked in the Pharmaceutical Industry for over 25 years and has experience of developing and commercialising new medicines from a variety of therapeutic areas across all phases of clinical development. Chrissie is a Chartered Statistician and Chartered Scientist of the Royal Statistical Society (RSS). Chrissie has an MSc in Applied Statistics and a BSc (Hons) in Statistics with Management Science Techniques.

## Sarah Garner

NICE, Associate Director Science Policy and Research

Professor Sarah Garner is a pharmacist specialising in the interface between Health Technology Assessment (HTA) and regulation. Sarah is the Associate Director for R&D at the UK's National Institute for Health and Care Excellence (NICE) and an honorary professor at UCL and Manchester University. Sarah is working with the NEWDIGS team at MIT to identify policy solutions using a systems approach focussed on adaptive pathways. Sarah is on the UK Expert Group on Innovation in Regulation of Healthcare and the Regulation of Medicines Review Panel, which carries out independent reviews of UK licensing authority decisions.

## Iris Goetz

Eli Lilly, Epidemiologist, Global Health Outcomes

Iris graduated in medicine in Germany prior to undertaking a research fellow ship at Great Ormond Street Hospital, London, UK. Following a MSc in Epidemiology at London School of Hygiene and Tropical Medicine she joined the Health Outcomes Research Department of Eli Lilly in the UK. Her primary focus of work lies in Real World Evidence research including pragmatic trials and observational studies.



## Diederick E. Grobbee

University Medical Center Utrecht, Professor of Clinical Epidemiology and Julius Clinical, Utrecht and Zeist, the Netherlands, Chief Scientific Officer

Rick Grobbee, academic co-lead of IMI GetReal, is founder of the Julius Center for Health Sciences and Primary Care at the University Medical Center Utrecht (1996) and of Julius Clinical (2008), a full service Academic Clinical Research Organization. He has been a (principal) investigator in many large-scale epidemiologic studies and randomized intervention trials relating to the prevention and treatment of cardiovascular, cardiometabolic, and cardiorenal disease. In addition he works on the principles and methods of treatment research, trial design and data analysis.

## Elaine A Irving

GlaxoSmithKline, Head of Real World Study Delivery

With her team, Elaine is responsible for designing and delivering high quality effectiveness studies to support the GSK portfolio. During her 18 years with GSK, Elaine has gained extensive R&D experience through the leadership of multiple asset teams as well as leading the integration of translational science across Neuroscience drug discovery within GSK. Elaine also played a key role in driving the development of the IMI portfolio, most notably leading the formation of the New Drugs for Bad Bugs initiative and authorship of the IMI2 strategic research agenda.  Elaine holds a BSc in Pharmacology and PhD in Neurodegenerative Research.

## Pall Jonsson

NICE, Senior Scientific Adviser, Science Policy and Research

Pall is the operational lead of WP1 of GetReal. He has a background in research and experience in synthesising, analysing and appraising scientific and clinical evidence, gained through work in academia, not-for-profit sector and the pharmaceutical industry. He has doctorate in biochemistry and bioinformatics from University College London. Pall has expertise in health technology assessments of drugs and diagnostics gained within UK's National Institute for Health and Care Excellence (NICE). His responsibilities have included technical oversight of guidance production and methodological development. He has also been involved in development of NICE methods and frameworks.

## Pieter Stolk

University Medical Centre Utrecht, Project Manager

Pieter Stolk, PhD, was trained as a pharmacist with a PhD from Utrecht University. Pieter is interested in all aspects of medicines regulation and policy (with a focus on marketing authorisation and HTA of medicines). He has been involved in several EU public private partnerships (incl. IMI GetReal) contributing to both the content and consortium management. He has worked as an independent consultant for governments, companies, NGOs and research organizations.



## Rob Thwaites

Takeda, Senior Director

Rob Thwaites is co-leader for Work Package 1 in IMI GetReal. Rob is a Senior Director at Takeda with responsibility for the global outcomes research teams based in the London office. Rob has 25 years' experience in the pharmaceutical industry in the UK, the US, and Australia, and has been leading RWD studies since 1993. Rob is active in EFPIA and ABPI, where he is Chair of the UK Pharmaceutical industry Health Information Group, the body responsible for activities at an industry level to encourage the development and use of RWD and associated capabilities in the UK. Rob holds degrees in Economics from the University of Cambridge and the University of New South Wales.

## Mira Zuidgeest

University Medical Centre Utrecht, Assistant Professor

Mira Zuidgeest works as an assistant professor at the Julius Center for Health Sciences and Primary Care, UMCU, the Netherlands. In the IMI GetReal project she is the scientific co-lead for the work package which focuses on the operational challenges of performing pragmatic relative effectiveness trials earlier in the drug development process. Mira completed a PhD in pharmacoepidemiology at Utrecht University, the Netherlands and has a special interest in pediatric asthma.





























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