



Patient involvement in the development process & early access

An industry perspective

Lode Dewulf, 14 Dec 2015, London

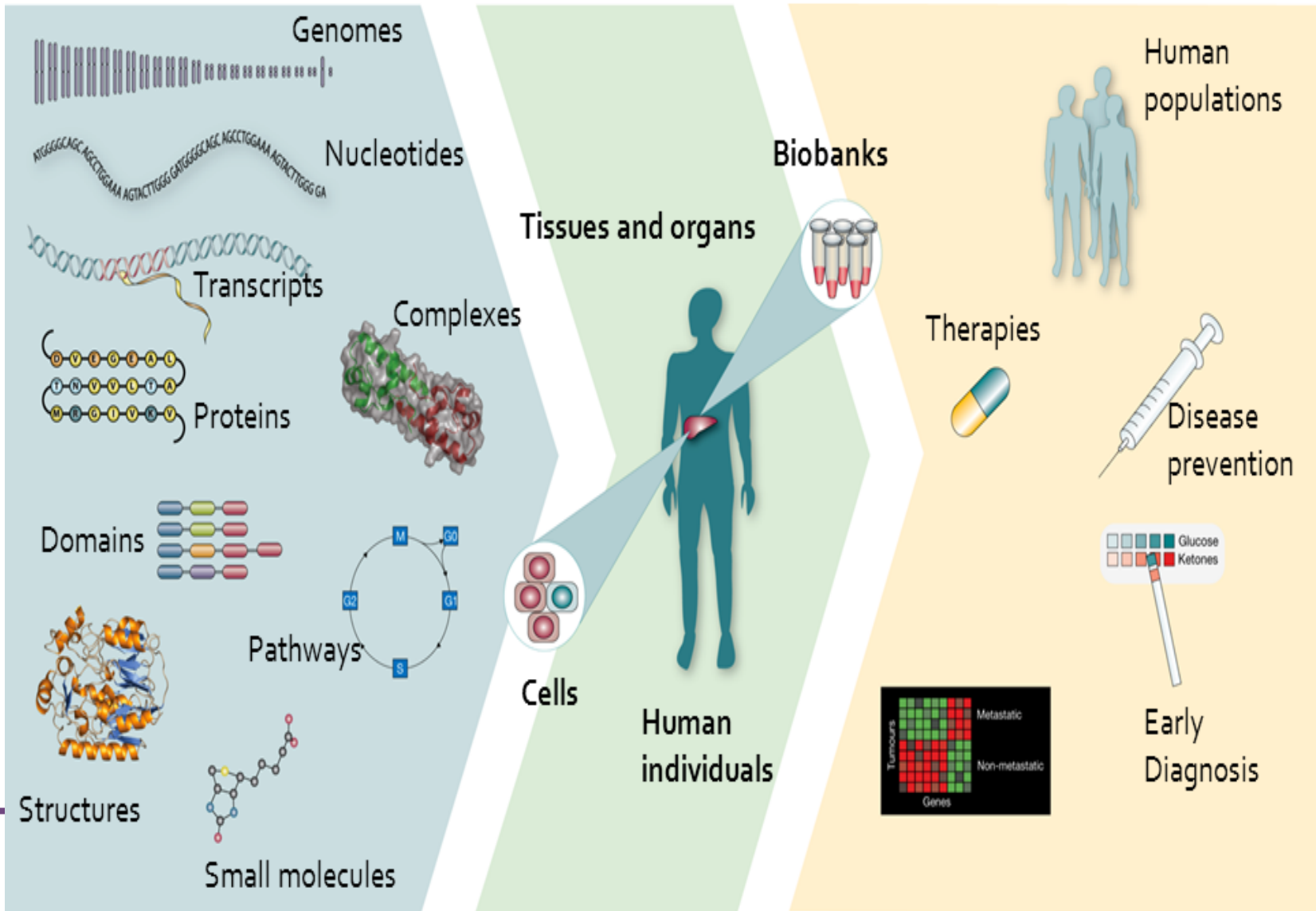
The definition of innovation is changing

From linear with the patient at the end

Molecular components

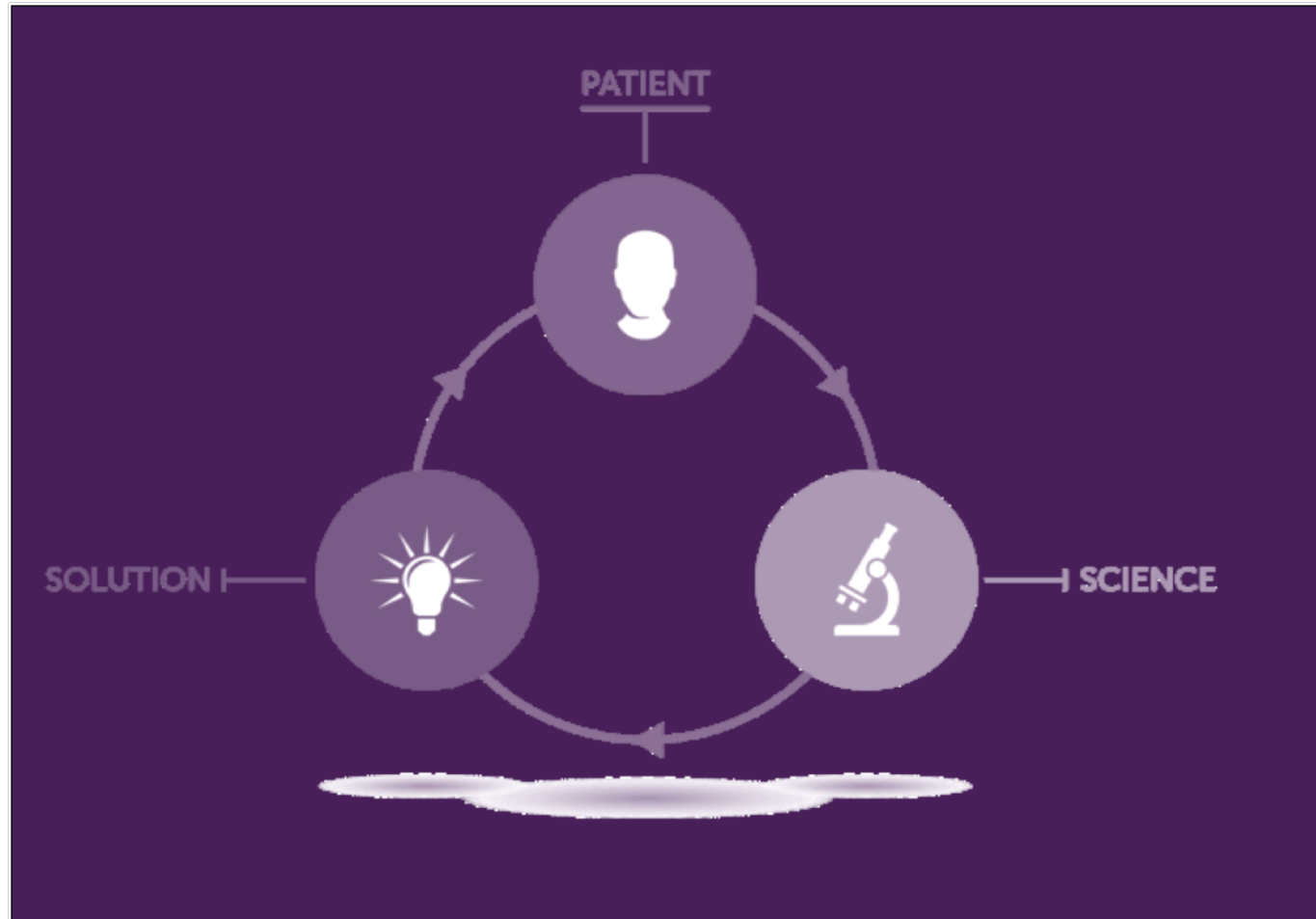
Integration

Translation



The definition of innovation is changing

To circular with the patient engaged all the time

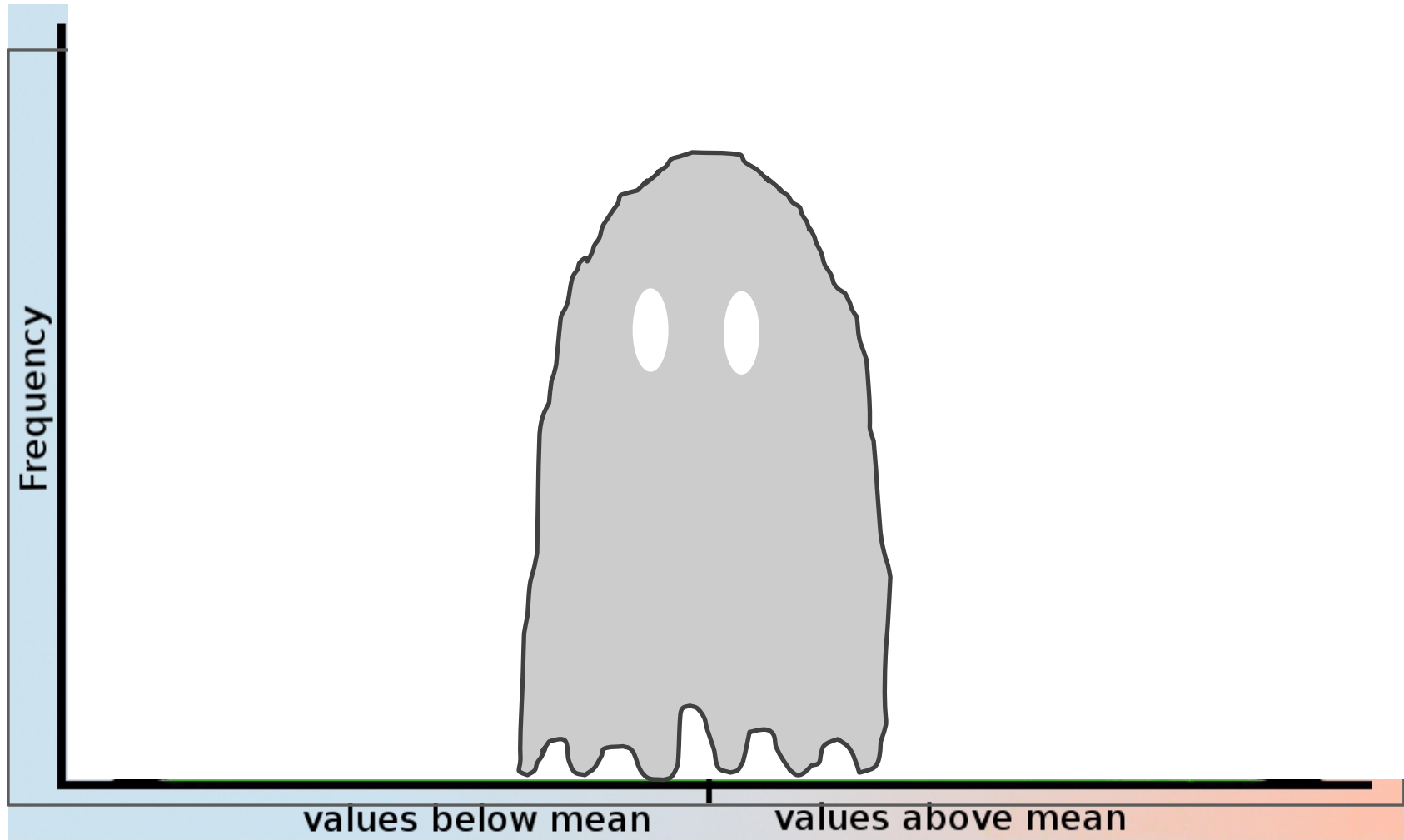


Patient Engagement throughout the development

3 Current Key Challenges for Industry

1. From the **population average** to the **individual patient**

The ghost of Karl Friedrich Gauss...



Patient Engagement throughout the development

3 Current Key Challenges for Industry

1. From the **population average** to the **individual patient**

2. Medicine Development is **global** and Patient Engagement is **local**

We need an ICH on Patient Engagement

Review Article

DIA DEVELOP
INNOVATE
ADVANCE

Partnering With Patients in the Development and Lifecycle of Medicines: A Call for Action

Therapeutic Innovation
& Regulatory Science
1-11
© The Author(s) 2015
Reprints and permission:
sagepub.com/journalsPermissions.nav
DOI: 10.1177/2168479015580384
tirs.sagepub.com

Anton Hoos, MD¹, James Anderson, MA, MBA², Marc Boutin, JD³, Lode Dewulf, MD, Dip Pharm Med, FFPM⁴, Jan Geissler, Dipl-Kfm⁵, Graeme Johnston, LLB, IPFA⁶, Angelika Joos, MPharm⁷, Marilyn Metcalf, PhD⁸, Jeanne Regnante, MS⁹, Ifeanyi Sargeant, DPhil¹⁰, Roslyn F. Schneider, MD, MSc¹¹, Veronica Todaro, MPH¹², and Gervais Tougas, MD, CM¹³

Abstract

The purpose of medicines is to improve patients' lives. Stakeholders involved in the development and lifecycle management of medicines agree that more effective patient involvement is needed to ensure that patient needs and priorities are identified and met. Despite the increasing number and scope of patient involvement initiatives, there is no accepted master framework for systematic patient involvement in industry-led medicines research and development, regulatory review, or market access decisions. Patient engagement is very productive in some indications, but inconsistent and fragmentary on a broader level. This often results in inefficient drug development, increasing evidence requirements, lack of patient-centered outcomes that address unmet medical needs and facilitate adherence, and consequently, lack of required therapeutic options and high costs to society and involved parties. Improved patient involvement can drive the development of innovative medicines that deliver more relevant and impactful patient outcomes and make medicine development faster, more efficient, and more productive. It can lead to better prioritization of early research; improved resource allocation; improved trial protocol designs that better reflect patient needs; and, by addressing potential barriers to patient participation, enhanced recruitment and retention. It may also improve trial conduct and lead to more focused, economically viable clinical trials. At launch and beyond, systematic patient involvement can also improve the ongoing benefit-risk assessment, ensure that public funds prioritize medicines of value to patients, and further the development of the medicine. Progress toward a universal framework for patient involvement requires a joint, precompetitive, and international approach by all stakeholders, working in true partnership to consolidate outputs from existing initiatives, identify gaps, and develop a comprehensive framework. It is essential that all stakeholders participate to drive adoption and implementation of the framework and to ensure that patients and their needs are embedded at the heart of medicines development and lifecycle management.

Keywords

patient involvement, medicines development

¹ M4P (Medicines 4 Patients) Consulting, London, UK

² GlaxoSmithKline, Brentford, Middlesex, UK

³ National Health Council, Washington, DC, USA

⁴ UCB Biopharma, Brussels, Belgium

Moving Forward & Going Global

We need to map **and synergize** Patient Engagement **I**

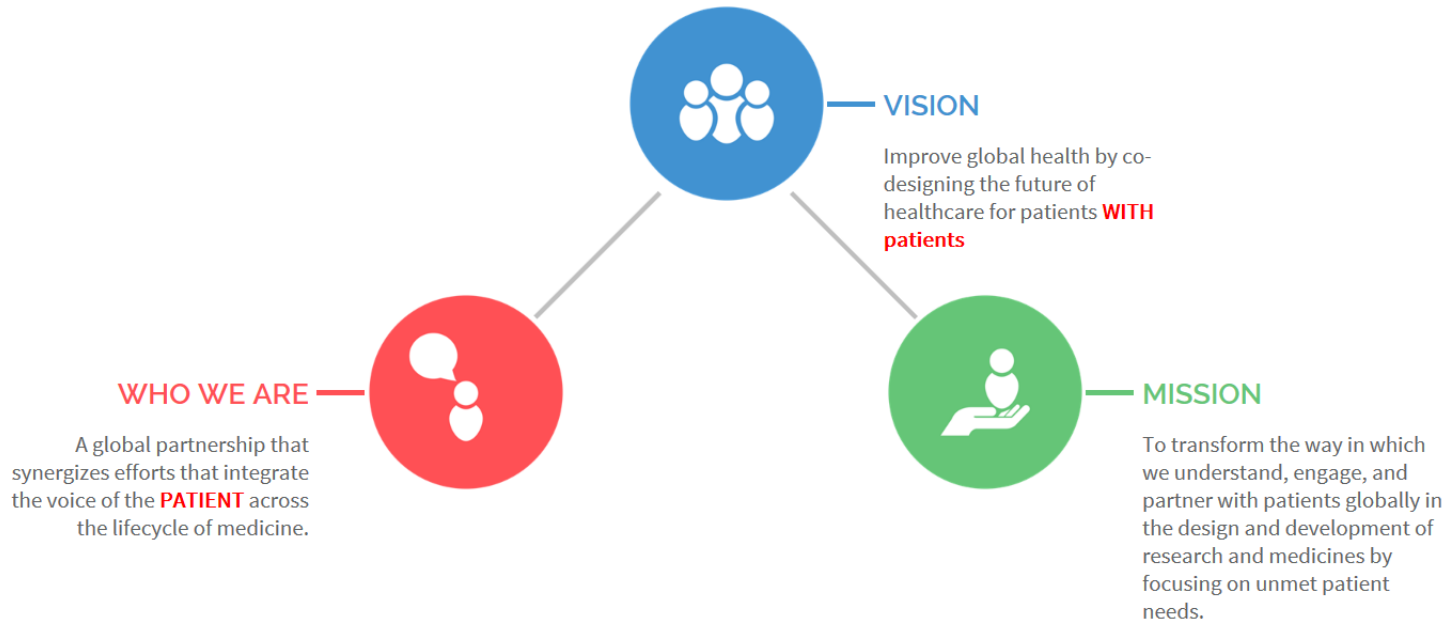
We need to map **and share** Patient Engagement **P**

G

PFMD launched in October 2015

www.pfmd.org

Patient Focused Medicine Development in a nutshell



PFMD launched in October 2015

www.pfmd.org

Our Path Forward



Mapping



Conduct a landscape assessment of initiatives that involve the patient.



Connections



Develop a platform allowing for cross-pollination.



Synergizing Framework Globally



Synergize and globalize good practices into an integrated patient involvement framework.



Patient Engagement throughout the development

3 Current Key Challenges for Industry

1. From the **population average** to the **individual patient**
2. Medicine Development is **global** and Patient Engagement is **local**
3. Engagement requires **trust** and Industry still faces **distrust**

Patient Engagement throughout the development

How we can build trust

P

- The science of Patient Input
- Early access is not a short cut

T

- Patient Engagement is largely pre-competitive
- Companies already collaborate; other stakeholders need to join

Thank you

|

|

A history of medicine...

Accident Based Medicine

A



Belief Based Medicine

B



Cash Based Medicine

C



Data Based Medicine

D



EVIDENCE Based Medicine

E



EXPERIENCE Based Medicine

E



From EBM to EMB 2.0

Evidence

Experience



The line between research and practice is fading.

.E



T



What is the definition and scope of RWE?

ABPI definition: Normal clinical practice

Proposed definition: clinical practice *plus*
Normal Life Living with a disease

