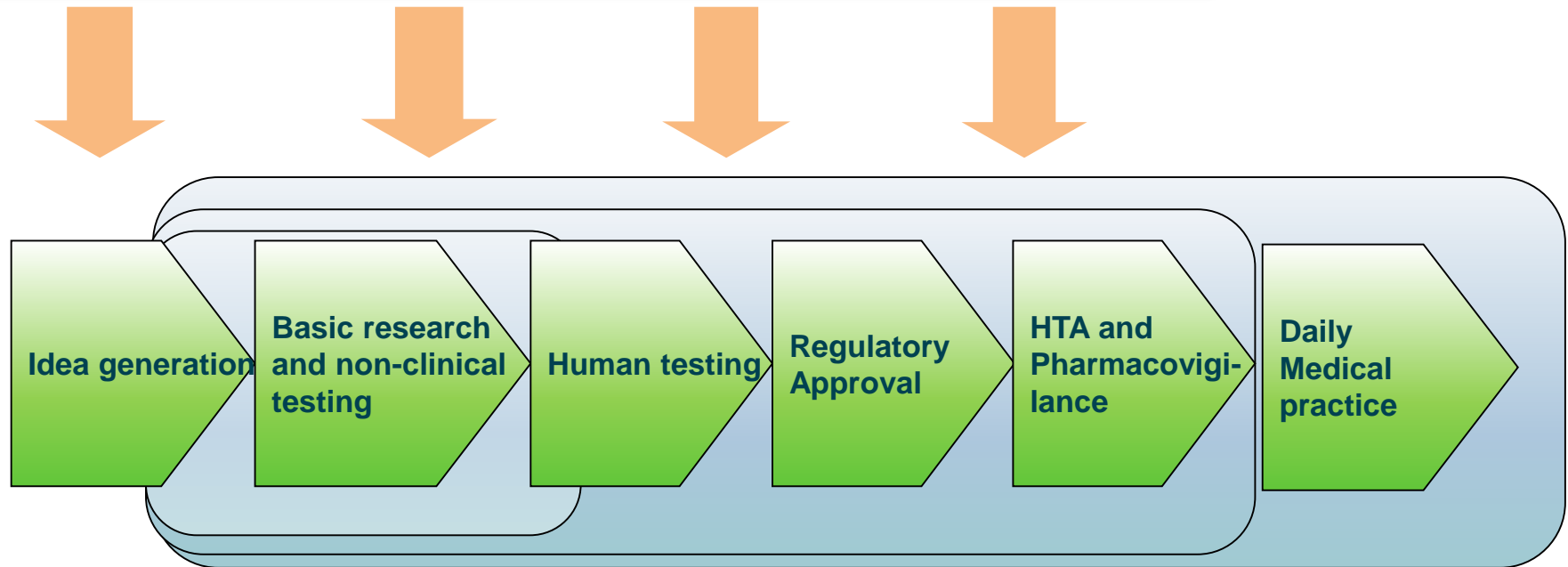# Introduction IMI2 Strategic Research Agenda: the vision and structure, and how to contribute to shaping the programme of work?

Magda Chlebus, Director Science Policy, EFPIA

IMI2 Info Day – Budapest, 25 September 2014

European Federation of Pharmaceutical Industries and Associations

www.efpia.eu

Evolution of IMI – the road to IMI2

Make Drug R&D processes in Europe more efficient and effective and enhance Europe's competitiveness in the Pharma sector

Idea generation → Basic research and non-clinical testing → Human testing → Regulatory Approval → HTA and Pharmacovigi-lance → Daily Medical practice
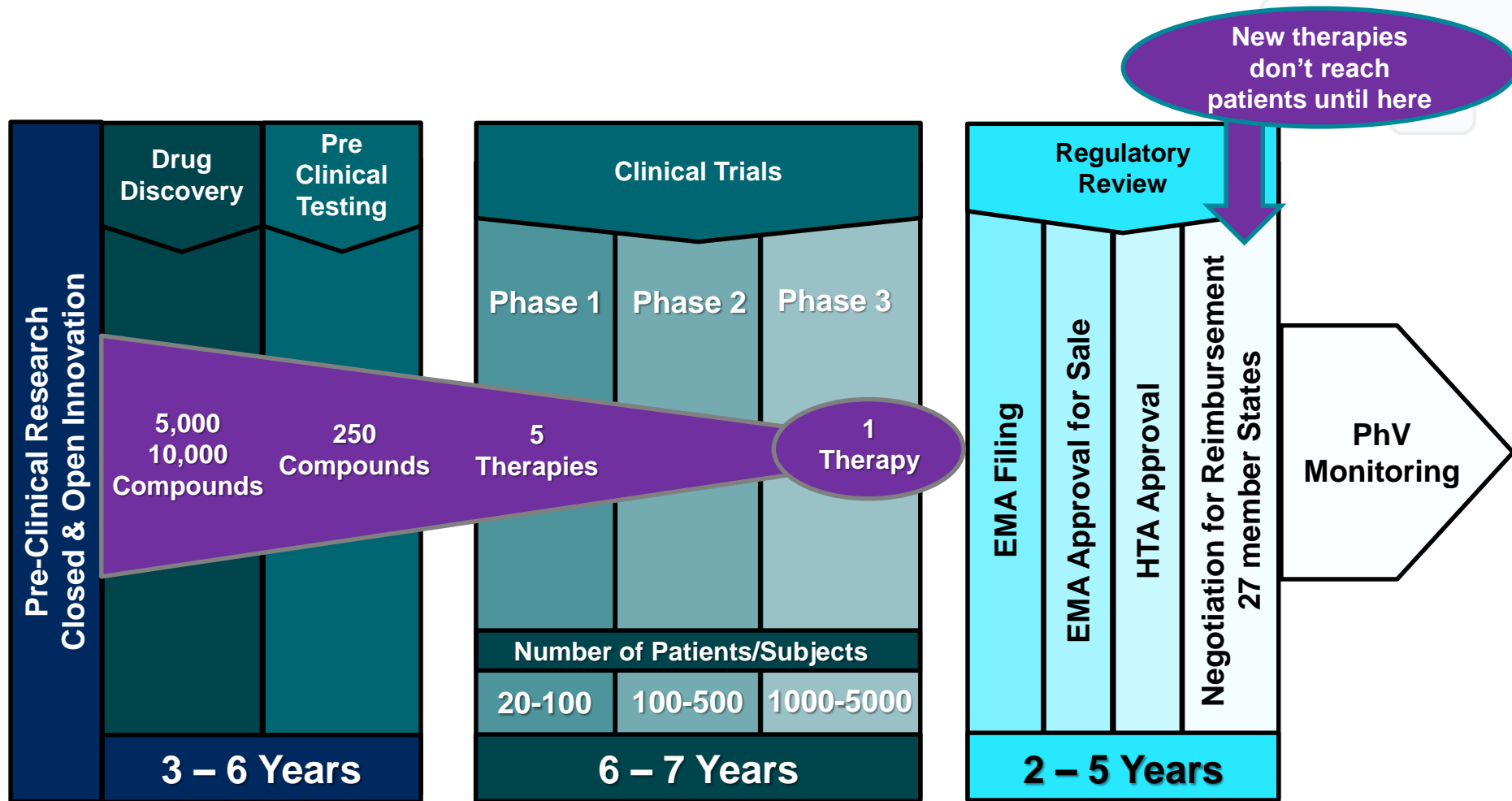
Primary focus of early IMI calls 2007 SRA

Shift to also addressing challenges in in society and healthcare 2011 SRA

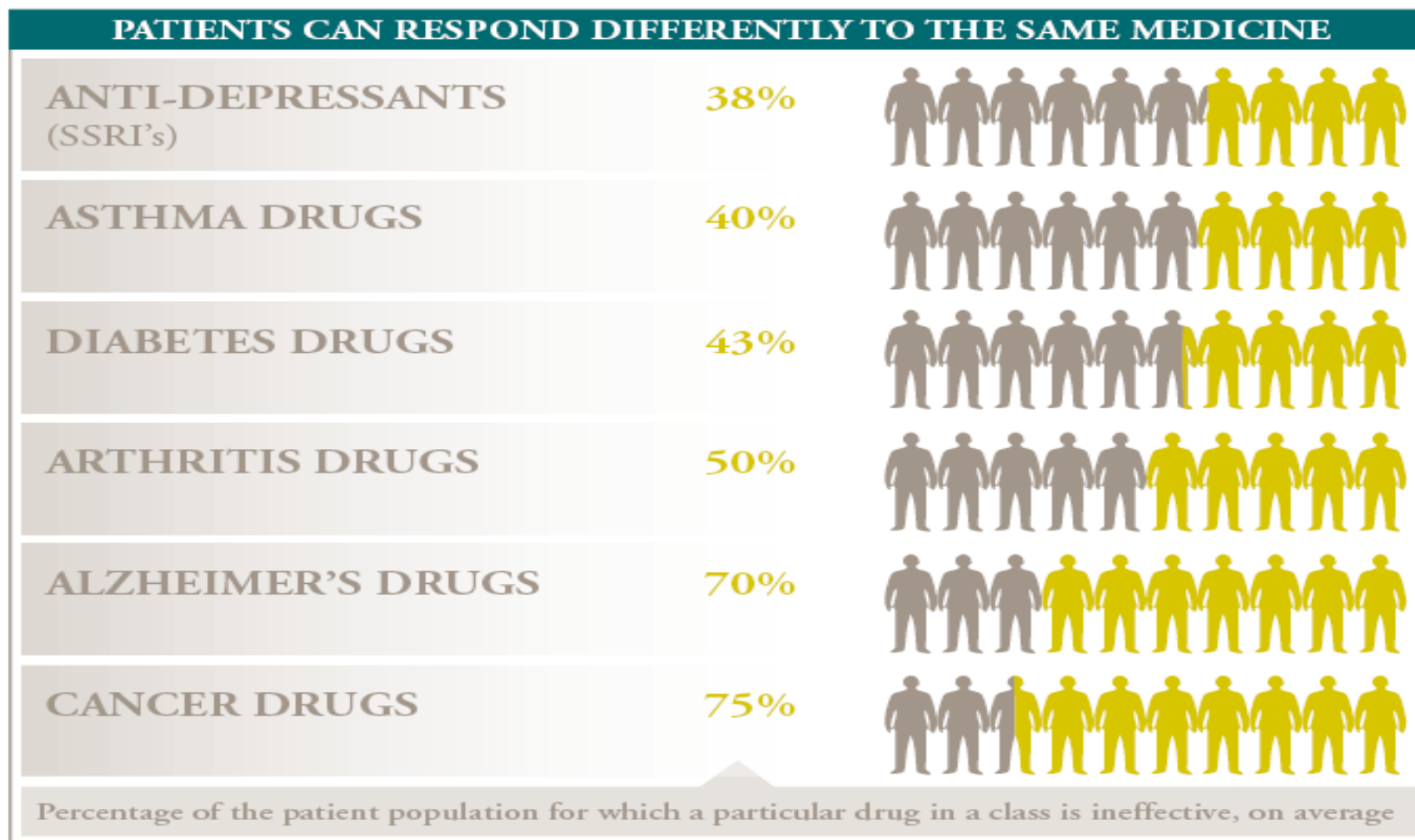**IMI 2 includes real life medical practice 2013 SRA**

SRA – Strategic Research Agenda

The pathways to patients are expensive and slow

New therapies don't reach patients until here

Pre-Clinical Research Closed & Open Innovation	Drug Discovery	Pre Clinical Testing	Clinical Trials			Regulatory Review				PhV Monitoring
			Phase 1	Phase 2	Phase 3	EMA Filing	EMA Approval for Sale	HTA Approval	Negotiation for Reimbursement 27 member States	
	5,000 10,000 Compounds	250 Compounds	5 Therapies		1 Therapy					

Number of Patients/Subjects

	20-100	100-500	1000-5000

3 – 6 Years	6 – 7 Years	2 – 5 Years

"The average drug developed by a major pharmaceutical company costs at least $4 billion, and it can be as much as $11 billion."
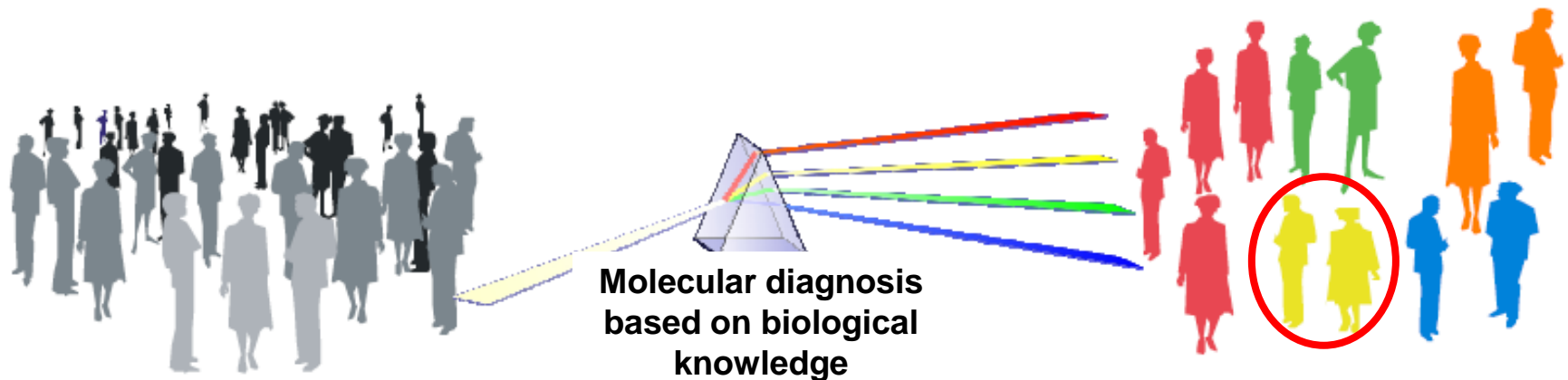
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Modern Medicines – non-responder rates

PATIENTS CAN RESPOND DIFFERENTLY TO THE SAME MEDICINE

ANTI-DEPRESSANTS (SSRI's)	38%	
ASTHMA DRUGS	40%	
DIABETES DRUGS	43%	
ARTHRITIS DRUGS	50%	
ALZHEIMER'S DRUGS	70%	
CANCER DRUGS	75%	

Percentage of the patient population for which a particular drug in a class is ineffective, on average

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The Vision for IMI2 (and the Pharma industry)

From population → **to individual**

Molecular diagnosis based on biological knowledge

| We "treat" a population. Some respond and some don't | We "treat" a *targeted* population They all respond |

Science is driving advances in diagnosis: breast cancer is actually 10 different diseases

NHS

Thursday April 19 2012

"A landmark study has reclassified the country's most common cancer in breakthrough research that could revolutionise the way we treat breast tumours… scientists found breast cancer could be classified into 10 different broad types according to their common genetic features."

http://www.nhs.uk/news/2012/04april/Pages/breast-cancer-genetic-diversity-mapped.aspx

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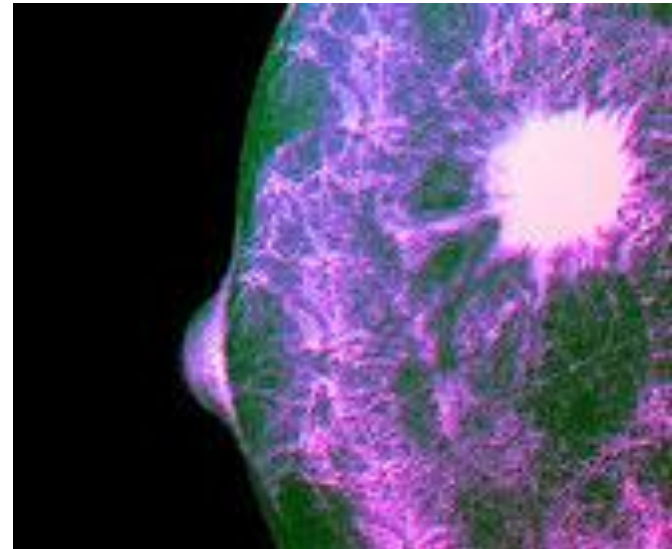
Unmet medical needs

Priority Medicines for Europe and the World 2013 Update

Warren Kaplan, Veronika J. Wirtz, Aukje Mantel-Teeuwisse, Pieter Stolk, Béatrice Duthey, Richard Laing

9 July 2013

Universiteit Utrecht — WHO Collaborating Centre for Pharmaceutical Policy and Regulation

BOSTON UNIVERSITY — WHO Collaborating Center in Pharmaceutical Policy

World Health Organization

* Burden of disease on patient and society = total cost of disease for healthcare and social security

* Unmet need:
  * No treatment
  * Inadequate treatment (resistance or treating symptoms, not cause)
  * Inadequate formulation for specific population (geriatric, pediatric, etc)

* Barriers and incentives

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Strategic Research Agenda

Comprehensive framework for a 10-year programme

Prepared with input from 80+ organisations (internet and targeted)

Project ideas from industry and third parties will be screened against it

http://goo.gl/jqMP9g

The right prevention and treatment for the right patient at the right time

Strategic Research Agenda for Innovative Medicines Initiative 2

Therapeutic areas covered by the IMI2 SRA

WHO 2013 report on priority medicines for Europe and the World

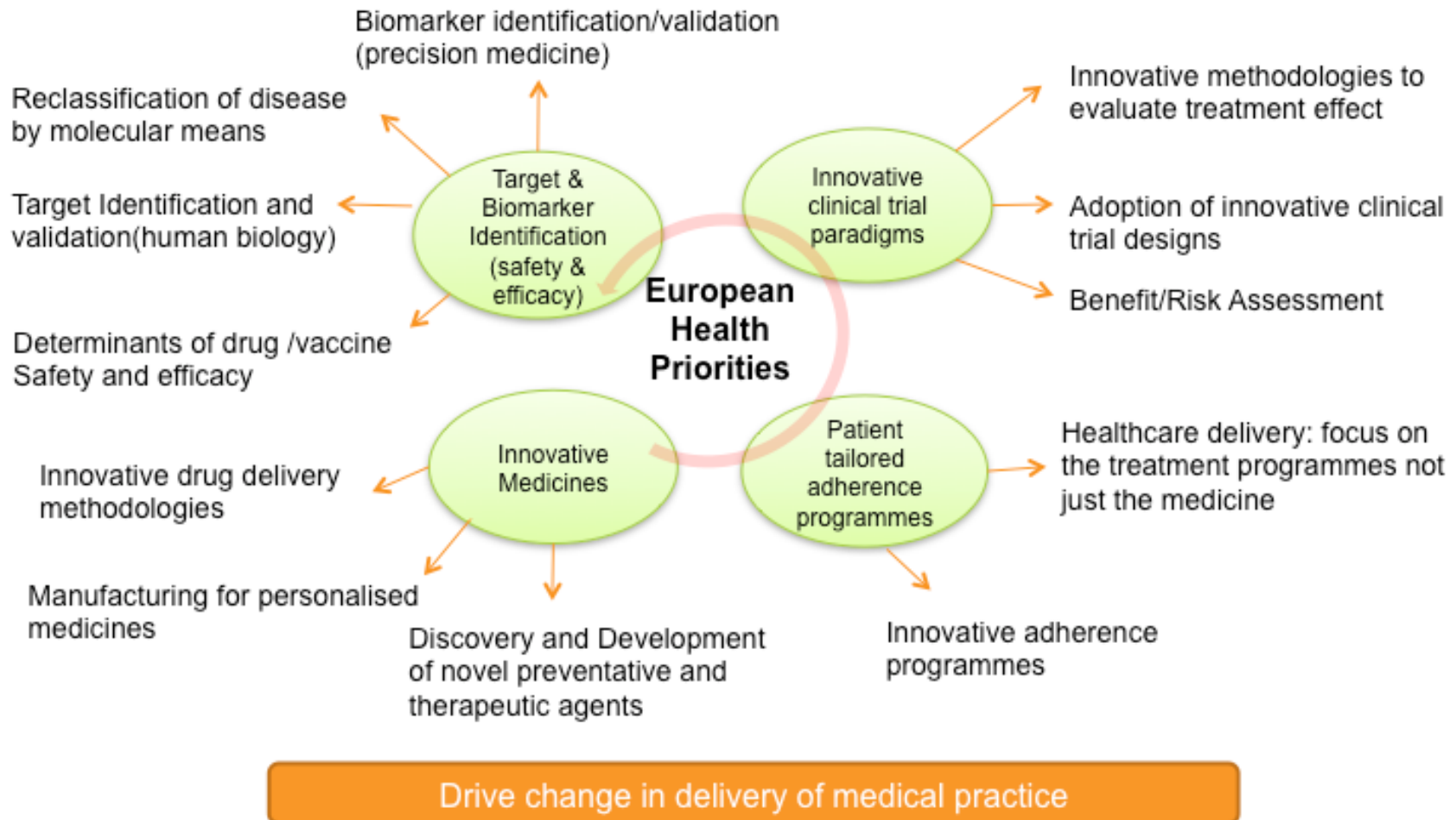Percentage of DALYs for top 20 high burden diseases and conditions
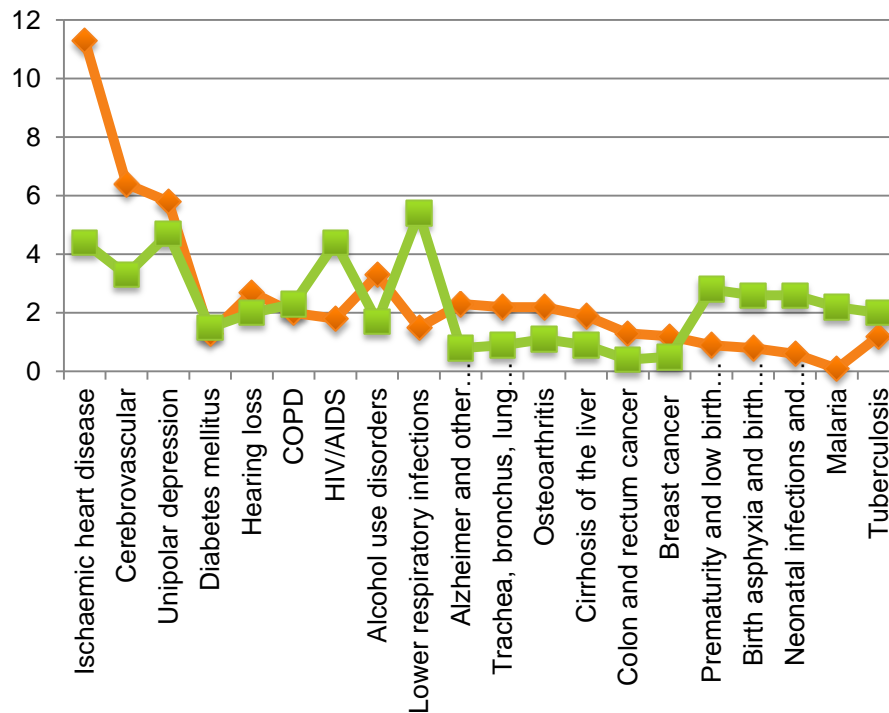
Therapeutic Areas in IMI2 SRA
(no priority order)

6. EUROPEAN HEALTH PRIORITIES

6.1. Antimicrobial resistance
6.2. Osteoarthritis
6.3. Cardiovascular diseases
6.4. Diabetes
6.5. Neurodegenerative diseases
6.6. Psychiatric diseases
6.7. Respiratory diseases
6.8. Immune-mediated diseases
6.9. Ageing-associated diseases
6.10. Cancer
6.11. Rare/Orphan Diseases
6.12. Vaccines

06

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The right prevention and treatment to right patient at the right time

European Health Priorities

- Target & Biomarker Identification (safety & efficacy)
  - Biomarker identification/validation (precision medicine)
  - Reclassification of disease by molecular means
  - Target Identification and validation(human biology)
  - Determinants of drug /vaccine Safety and efficacy
- Innovative clinical trial paradigms
  - Innovative methodologies to evaluate treatment effect
  - Adoption of innovative clinical trial designs
  - Benefit/Risk Assessment
- Innovative Medicines
  - Innovative drug delivery methodologies
  - Manufacturing for personalised medicines
  - Discovery and Development of novel preventative and therapeutic agents
- Patient tailored adherence programmes
  - Healthcare delivery: focus on the treatment programmes not just the medicine
  - Innovative adherence programmes

Drive change in delivery of medical practice

13

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IMI2 scientific programme: First five big themes

Therapeutic Areas and Cross-cutting Themes

1. **Neuro-degeneration**
   - Successfully prevent and treat dementia and other neurodegenerative diseases

2. **Prevention and treatment of immune-mediated disease**
   Advance immunological understanding to deliver new medicines and new and better vaccines

3. **Metabolic disorders**
   - Tackle all phases of disease and its complications, including prevention and early interception (type 2 diabetes, obesity, dislipidemia, hypertension)

4. **Infection control**
   - Address big societal problem related to multidrug resistance and create incentives for reinvestment (including antimicrobials, antivirals, vaccines) and develop new and better vaccines

5. **Translational Safety**
   - identification of predictors of safety and development of point of care for safety biomarkers & Development of new human biology platform to predict toxicity and safety during early drug development

Differentiating Enablers for all themes

Towards early and effective patient access to innovative prevention and treatment solutions (MAPPs):

- Target validation based on human biology
- Stratified medicine, precision medicine
- Innovation in clinical trials
- Data generation and interpretation (knowledge management)
- Prevention, disease interception, patient adherence (incl. societal acceptance of vaccines)
- Effect on medical practice and outcomes (health/disease management)
- Regulatory framework (including pharmacovigilance)
- Patient access

IMI2 objectives – extract from IMI2 Regulation:

* increase the success rate in clinical trials

* where possible, reduce the time to reach clinical proof of concept in medicine development

* develop new therapies for diseases for which there is a high unmet need and limited market incentives

* develop diagnostic and treatment biomarkers for diseases clearly linked to clinical relevance and approved by regulators;

* reduce the failure rate of vaccine candidates in phase III clinical trials through new biomarkers for initial efficacy and safety checks;

* develop tools, standards and approaches to assess efficacy, safety and quality of regulated health products.

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Success will be driven by

✱ Focusing on the challenges of the future

✱ Leveraging the value added for working together, across sectors, effectively use resources and expertise

✱ Focussing on strategic, game changing, think big – around broader therapeutic areas (not indications)

✱ Change in research, regulatory, and healthcare practice

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http://imi.efpia.eu/
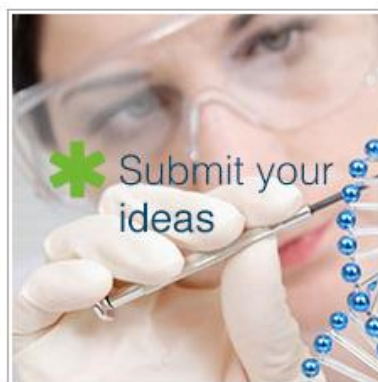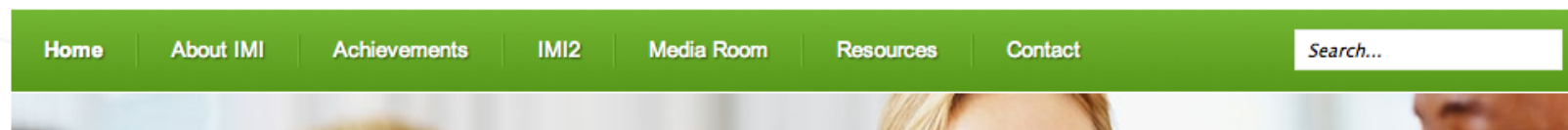
Conclusions

* Focused: stratified medicines and healthcare priorities
* Healthcare solutions: prevention and treatment
* End-to-end: R&D, regulatory, access/healthcare practice
* Multi-sector: within and beyond life sciences
* Submit your ideas: http://imi.efpia.eu/

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Right prevention and treatment, for the right patient, at the right time …

IMI and IMI2: from science to patients - together

SUCCESS

| New model developed & published | Setting new standards | In house implementation by industry | Impact on regulatory practice | Better drugs & impact on med. practice |

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Discussion

* Is there anything missing in the vision of the Strategic Research Agenda?

* What are the strengths of the Hungarian R&D ecosystem in light of the SRA?

* The right information, to the right players, at the right time: dissemination/sources of information to enhance participation

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