Priority Medicines for Europe and the World:
Setting a public health based medicines development agenda

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Definition of Priority Medicines:

* Medicines which are needed to meet the priority health care needs of the population which have not yet been developed

Background of the report (1)

- Priority Medicines Report 2004 commissioned by Dutch Ministry of Health
  - Top Institute Pharma established
  - Used by EC for calls in Framework Programmes

- December 2010: Council of the EU invites EC and MSs to "*take the initiative to update the 2004 Priority Medicines report in cooperation with WHO experts*"

- Update started June 2012, final report launched 2013

Background of the report (2)

* **WHO commissioned by EC** (DG Enterprise and Industry)
* **Close involvement of**
  * DG Research and Innovation & DG Health and Consumers
  * International Project Advisory Group, including among others MSs, EFPIA members, NGOs, EC & WHO experts
* **Collaboration with**
  * Boston University (Chapters 1-6)
  * Utrecht University (Chapters 7 and 8, commissioned by Dutch MoH)
  * Individual authors for Background Papers

Objectives of 2013 Update

- Provide a methodology for identifying pharmaceutical "gaps" from a public health perspective for Europe and the World

- Provide a public health based pharmaceutical R&D agenda for use by the EC (Horizon 2020) and IMI

- Identify opportunities for innovation to address gaps

- Develop One final Report but a series of background papers

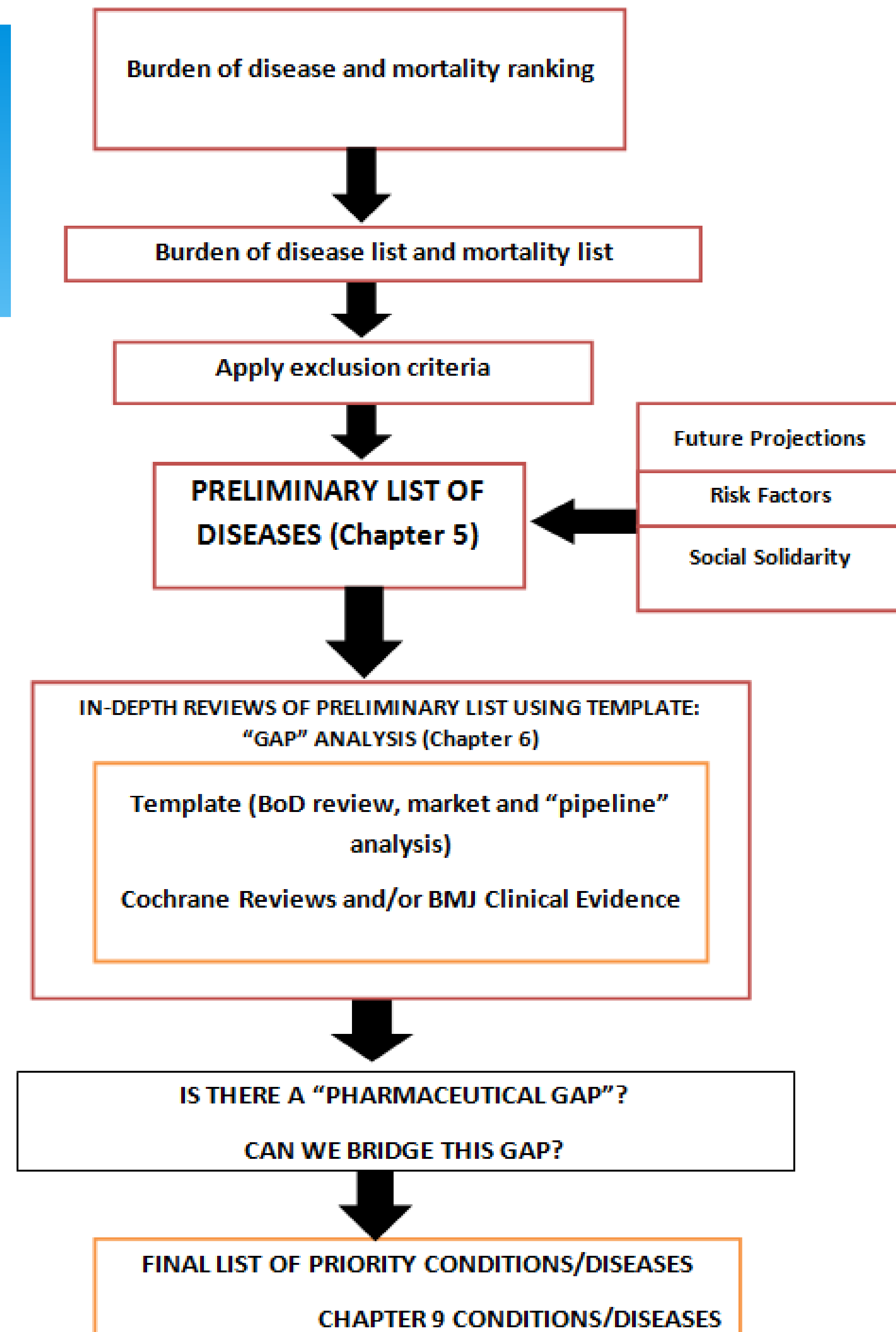
Four inter-related criteria to identify Priority Medicines

1. The estimated European and global burden of disease
2. The prediction of disease burden trends, based on epidemiological and demographic changes in Europe and the world
3. The principle of “social solidarity” applied to diseases for which there are currently no market incentives to develop treatments
4. The common risk factors amenable to pharmacological intervention that have an impact on many high-burden diseases

Methodology (1)

Data sources:

- WHO Global Burden of Disease Database (projections for 2008)
- 2010 Global Burden of Disease Study (Lancet, December 2012)

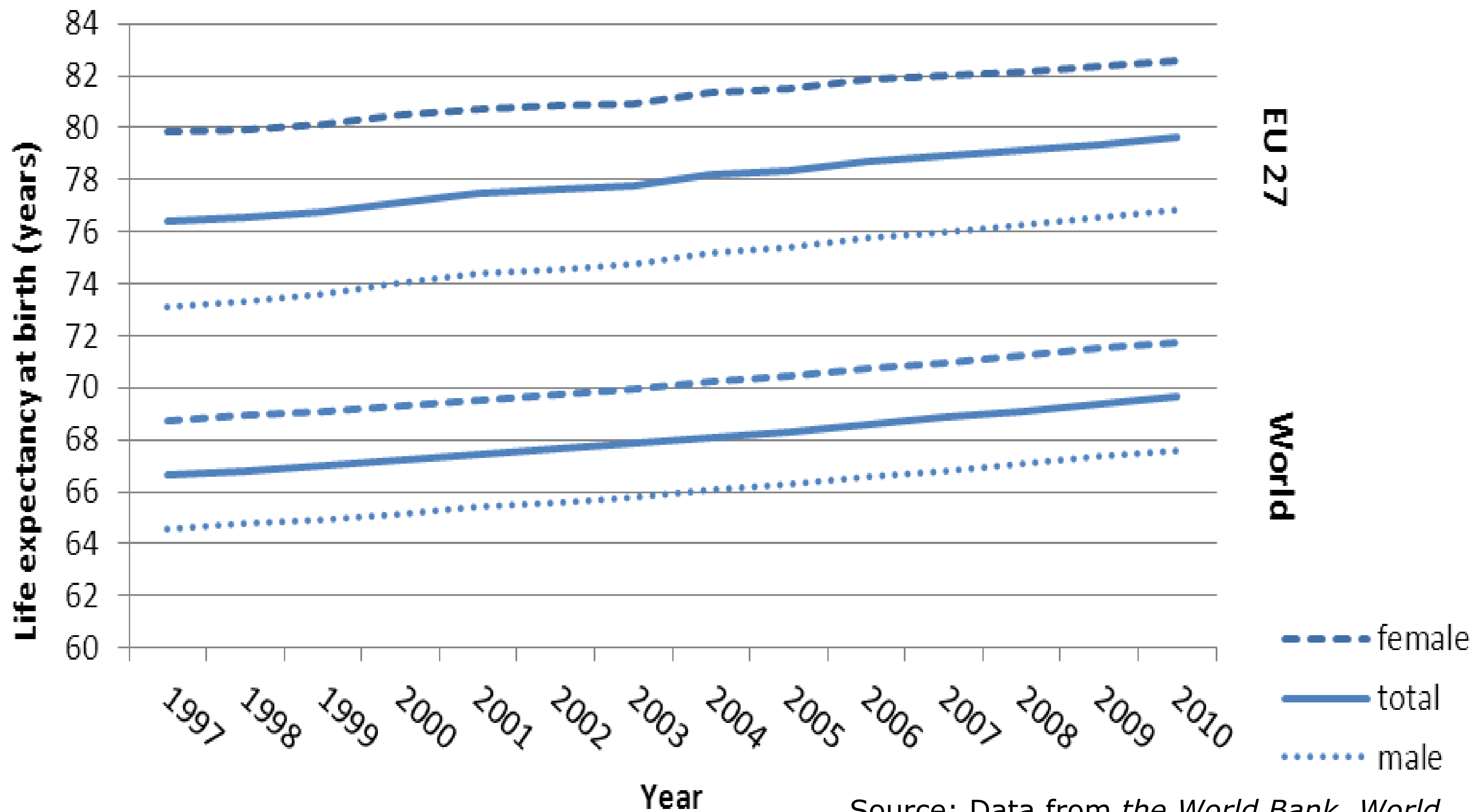


Methodology (2)

* Three **<u>different types of gaps</u>**:
  1. Treatment(s) exist but will soon become <u>ineffective</u>
  2. Treatment(s) exist but the pharmaceutical delivery mechanism or formulation is <u>not</u> appropriate for the target population
  3. Treatment does not exist OR is not sufficiently effective

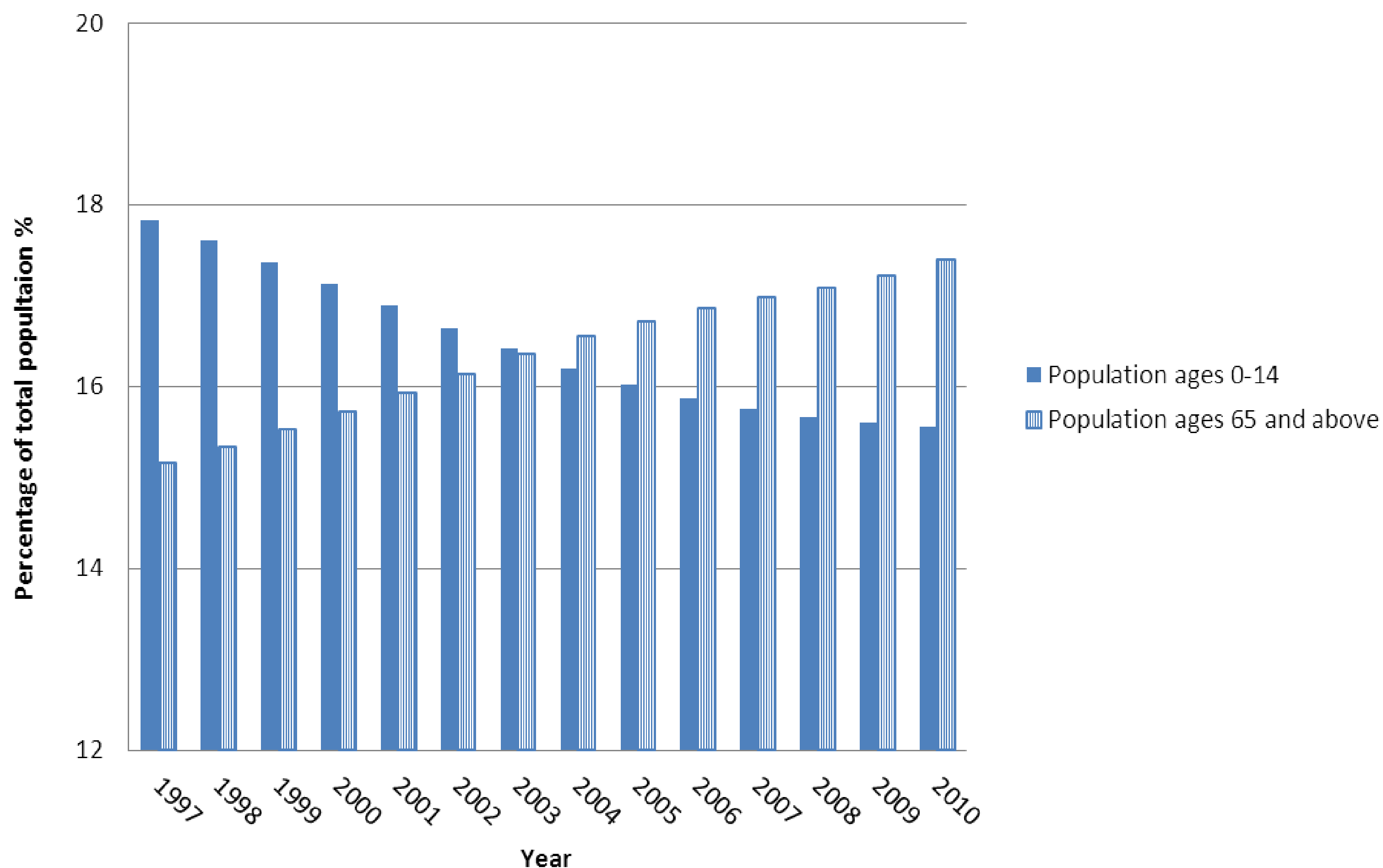
* Also look at contextual factors to foster innovation (e.g. policy reform)

Results: Life Expectancy



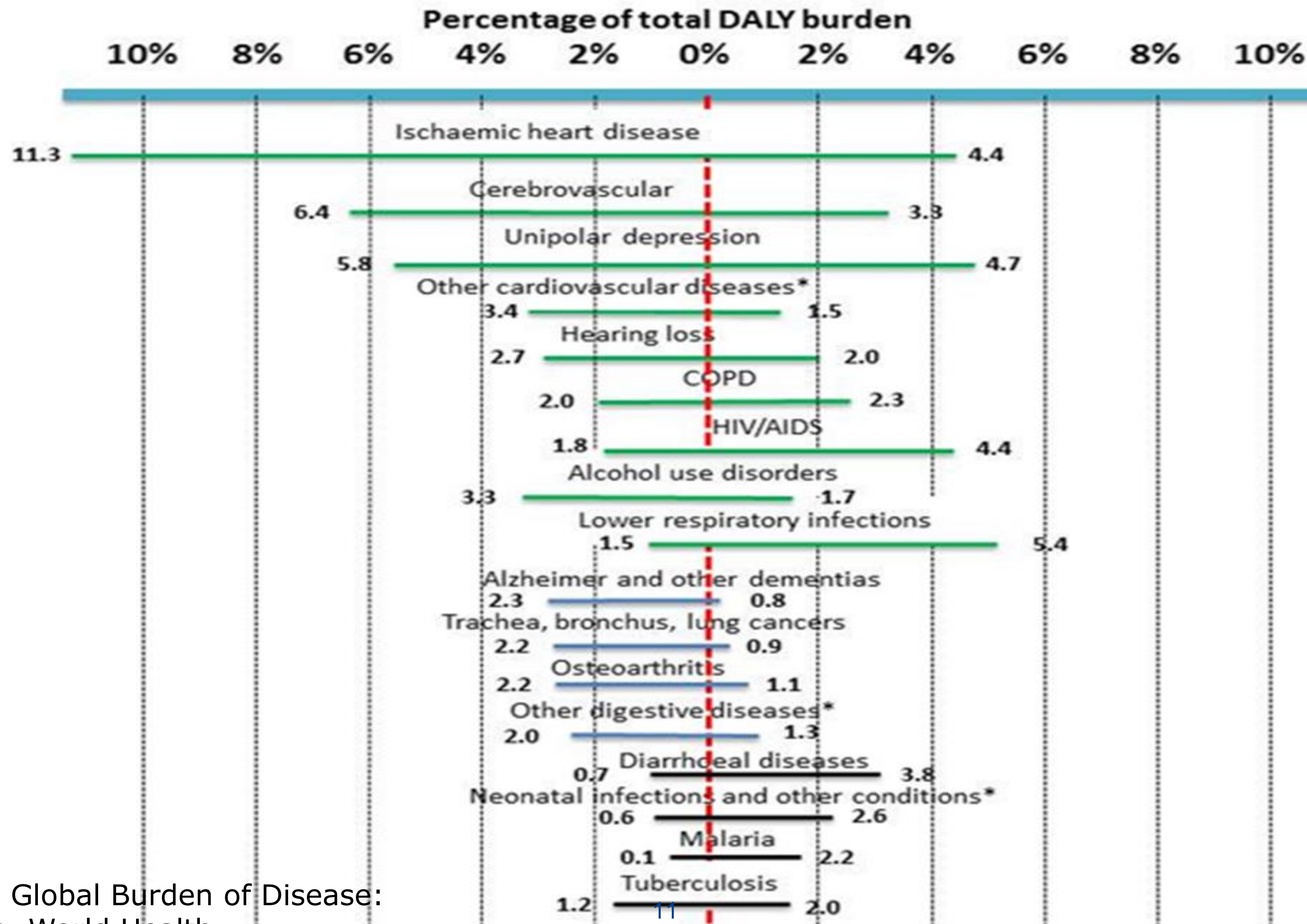
Source: Data from *the World Bank. World Development Indicators.* Available at: http://databank.worldbank.org

Europe is Aging!



Source: Data from *the World Bank. World Development Indicators.* Available at: http://databank.worldbank.org

Burden of disease



Source: The Global Burden of Disease: 2004 update, World Health Organization, 2008

Diseases addressed

Antibacterial drug resistance	Chronic obstructive pulmonary disease
Pandemic influenza	Alcohol use disorders and alcoholic liver disease
Ischaemic heart disease	Depression
Diabetes	Postpartum haemorrhage
Cancer	Tobacco use
Acute stroke	Obesity
HIV/AIDS	Rare disease
Tuberculosis	Diarrhoea
Neglected tropical diseases	Hearing loss
Malaria	Pneumonica
Alzheimer disease and other dementias	Neonatal conditions
Osteoarthritis	Low back pain

Leading risk factors for the burden of disease

Table 5.2: The leading risk factors for the Burden of disease, 2004, ranked in order of percent of total DALY[a]

WHO European Region[b]		World	
Risk factor	***%***	***Risk factor***	***%***
Tobacco use	11,7	Underweight	5,9
Alcohol use	11,4	Unsafe sex	4,6
High blood pressure	11,3	Alcohol use	4,5
Overweight and obesity	7,8	Unsafe water, sanitation, hygiene	4,2
High cholesterol	5,9	High blood pressure	3,7
Physical inactivity	5,5	Tobacco use	3,7
High blood glucose	4,8	Sub-optimal breastfeeding	2,9
Low fruit and vegetable intake	2,4	High blood glucose	2,7
Occupational risks	1,7	Indoor smoke from solid fuels	2,7
Illicit drug use	1,6	Overweight and obesity	2,3

[a] Source: Global Burden of Disease, 2004 update, World Health Organization.

Summary of disease or risk factor results

- **Treatment(s) exist but will soon become <u>ineffective</u>**
  - Antibacterial resistance, pandemic flu

- **Treatment(s) exist but the pharmaceutical delivery mechanism or formulation is <u>not</u> appropriate**
  - CVD, HIV, cancer, depression, diabetes, pneumonia, diarrhea, neonatal diseases, malaria, tuberculosis, NTD, postpartum hemorrhage

- **Treatment does not exist OR is not sufficiently effective**
  - Stroke, osteoarthritis, Alzheimer and other dementias, COPD, hearing loss, low back pain

Key findings & recommendations

* Marked increase in diseases of the elderly (e.g. Alzheimer, osteoarthritis, hearing loss)
* New medicines and improvement of existing NCD medicines
* Optimise secondary prevention of CVD
* Identification of biomarkers for many diseases to diagnose, monitor disease progression and assess treatment effects
* Research needed on pharmacological interventions to target important risk factors

Key findings & recommendations

- Malaria, TB, AMR/pandemic flu → Resistance will remain threat until primary prevention (vaccination) occurs
- Diarrhea, pneumonia, neonatal conditions and maternal mortality → Improvement of diagnosis and treatment, including reducing costs
- NTDs and rare diseases → new mechanisms to promote translation of basic research into products

Children, Women & Elderly: recommended area for research

- Develop age-appropriate medicines;
- Study the impact of regulations and interventions on patient and public health outcomes (incl. evaluation of [cost-]effectiveness);
- Increase use of electronic health records for safety and effectiveness (especially for off-label use);
- Improve (information on) the rational use of medicines and assure that it is being acted upon (e.g. sharing of information, communication, electronic solutions).

Public-Private Partnerships: Recommended areas for research

- Need to learn more about the most succesful models:
  - Most useful indicators (structural, process, output or outcome)?
  - What is a succesful partnership?
  - What can actually be achieved?
- How can we best assure project sustainability?
- Research possibilities for stakeholder involvement, particularly patient and citizen involvement.

Regulatory system: recommended areas for research

* Instruments to optimize regulatory requirements (e.g. the use of surrogate outcome measures and adaptive study design) and benefit-risk assessment;
* Clearly identify expectations and key performance indicators for new regulations and set up prospective studies;
* Establish constructive collaborations and dialogues with key actors;
* Invest in sharing and analysis of regulatory datasets for system evaluation and strengthen DRS methodologies.

Pricing policies: recommended areas of research

* Research the broader environment of pricing and reimbursement (e.g. perception of innovation, financial crisis);
* Develop and assess methods used for pricing and reimbursement policies (e.g. value-based pricing);
* Appropriate research infrastructure.

Patient participation: recommended areas of research

- Develop a consensus model or a framework for meaningful involvement (building on work in, e.g., Value+, G-I-N, INVOLVE and the Participatory Methods Toolkit);
- Build capacity to ensure the meaningful involvement in priority setting for pharmaceutical innovation;
- Assure structural outcome assessment of initiatives to involve patients and citizens.

Discussion

* Important to establish a list of priority medicines to guide current and future investment in R&D
* Opportunity for Europe to address global needs for R&D of priority medicines
* Contextual factors are relevant to consider to align incentives and sustainability of R&D
* A series of opportunities to improve population health in Europe and the world through public health based innovation

Document available online

* All documents (report and background papers) are available through WHO website http://www.who.int/medicines/areas/priority_medicines/en/

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