Gaps and Needs

CSA PerMed Workshop 1, Berlin 27./28.03.2014

Presenters: Angela Brand, Lada Leyens, Erica Hackenitz

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Aim WP1

Inventory of Activities, Key Players,
Identification of Gaps & Needs

„(1) Identify relevant fields, organisations, current national, European and selected international initiatives, policies and capacities related to Personalized Medicine based on an inventory and synthesis of existing relevant information...

(2) Highlight European and national showcases and best practice examples for already successful approaches in all areas related to Personalized Medicine“

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Approach

What has been already done?

(1) Identification of **strategic reports**
(e.g., EuroBioForum, PHGEN, EAPM or the ESF Forward Look)

(2) **Interviews** with key stakeholders across sectors

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Method

(1) SWOT, gaps & needs identified in strategic reports:

- Tracing and analysis

(2) **SWOT, gaps & needs identified in Interviews:**

- List of key stakeholders (basic sciences/new technologies, translational research, regulation/reimbursement/market access, health care system (input PerMed consortium)
- Semi-structured interviews (interview guidelines)
- Draft Interview summary reports
- Authorized summary reports

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Presentation & Handouts

Lada Leyens
Analysis of strategic reports focussing on major areas

Erica Hackenitz
Analysis of interviews within these major areas

=> More details in PerMed folder
=> Further discussion in the sessions in the afternoon

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SWOT, Gaps – Needs

On the basis of 19 available reports
on Personalised Medicine

Lada Leyens
Berlin, 27.03.2014

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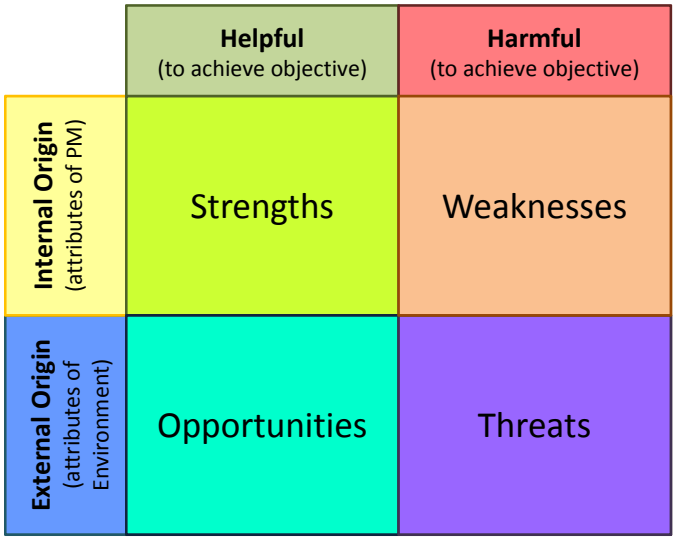
SWOT

SWOT, Gaps and Needs extracted from 19 strategic reports. Details of the reports can be found in the workshop folder together with the complete summary of the SWOT, Gaps and Needs



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Strengths	Weaknesses
Patient empowerment	Inaccuracy and un-specificity of CDx → Depends on accurate and specific CDx
Increased efficacy and safety → optimized risk-benefit	Risk evaluation and rare AEs not detected in small populations (in CTs)
Increased treatment cost-benefit and cost containment	Cannot be fully implemented in current health care system
Patient-centered	Definition not harmonized
Improved Q of Care	Misleads patients to think it is truly personalized
Complements Population -based health approaches	Initial high costs and investments
Early diagnosis, early treatment and prevention	Stratification paradigm (inequalities in access to PM)
Diagnosis accuracy and informed medical decision	May increase health inequalities
P4: personalized, predictive, preventive, participatory	
encourages collaboration within and across sectors	
Targeted and efficient drug development	

Opportunities	Threats
Changes in demographics and epidemiology	Current economic environment
Advances in science and technology	Increasing pressures on pharmaceutical industry
Reduced genetic testing costs	Outdated legislations and reimbursement process
Current HC systems are no longer fit for purpose	Non-harmonized regulatory frameworks across countries and between Drugs and IVDs
Investment of non-traditional players	Outdated training programs
Modern diagnostic methods	Low patient/public awareness
Implementation of IT solutions to healthcare (e.g. health and patient electronic records)	Long and costly drug development and slow technology transfer / uptake into HC
Considered in new EU legislative initiatives	Health inequalities between and within EU
Improvement of CT and IVD legal framework	Slow implementation of IT solutions in HC
Current improvements on health literacy and interest → increased demand of PM	Each stakeholder protects their own interest, stopping change in HC
Big Data	Data collection, interpretation and data privacy
Definition of new drug development models	Use of health information against the patient
Re-positioning of fallen angels	Lack of definite scientific proof and know-how
	Medico-genetic „Big-Brother“

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Gaps – Needs

SWOT, Gaps and Needs extracted from 19 strategic reports. Details of the reports can be found in the workshop folder together with the complete summary of the SWOT, Gaps and Needs

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Gaps	
Legislation	Outdated and inappropriate legislation and reimbursement process Fragmentation and outdated HTA, pricing and reimbursement in MSs
Health Care System	Focused on acute care rather than prevention/chronic management Fragmented financing and delivery of care Lack of infrastructure
Training	Outdated training of healthcare professionals Knowledge of statistical science missing in all stakeholders
Literacy	Low patient/public awareness and literacy on PM
Drug development process	Failure to translate from pre-clinical model to clinical setting Clinical Trials design Weak technology transfer capacities
Stakeholders	Current unidirectional approach of HC companies, without collaboration Lack of collaboration between research institutions
Data	Non-standardized data with different quality Data silos and secrecy from stakeholders Privacy issues unresolved
eHealth / ICT	Slow implementation Information technologies are not part of training/competences
Disease classification	Outdated disease classification

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Needs (I)	
Legislation	Update and adapt regulations: simplified, harmonized, predictable, coherent Coordinated reimbursement considering PM particularities (e.g. Drug + CDx, personal utility) New CT methodologies Bottom-up approaches in policy making
Health Care System	Change in healthcare systems and models: structures, financing, professionals, patient behavior, focus on prevention, insurance model… Reduce inequalities in health and in access
Training	Update all curricula: ICT, patient communication, cross-disciplinary interaction, applications of genomics and proteomics… Form specialized-trained teams in hospitals
Literacy	Inform, educate, empower

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Needs (II)	
Drug development process	Timely, effective and responsible translation Reward for innovation and incentives Post-marketing surveillance and observational studies: spot rare AEs and efficacy
Stakeholders	Increased collaboration and early engagement
Data	Ensure compatible and comparable data of high quality and avoid silos Infrastructure and framework for data collection, storage, management and analysis Trust and transparency for patients and ownership of data Correct perception of genetic exceptionalism
health / ICT	Development of new decision supporting tools to analyze and interpret data
Disease classification	Re-classification into diseasomes
Biobanks	Update legal framework, specially considering consent of patients

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SWOT, Gaps – Needs

On the basis of interviews with experts working in the field of Personalised Medicine

Erica Hackenitz

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Many thanks to:

- The people interviewed
- The PerMed partners who did the interviews

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Interview harvest

30 interview reports endorsed

2 interview reports pending

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Exemplaric focal points addressed in interviews

B/B+T	Regulatory	Health system	
A BETTER RATIONAL TREATMENT	INSIGHT in WHAT MAKES PEOPLE DIFFERENT	A HELP in the SUSTAINABILITY OF HEALTH CARE SYSTEMS	Strengths
TRADITIONAL MIND-SET of MEDICAL PRO-FESSION IS a HURDLE	INDIVIDUAL PARAMETERS DO NOT GIVE the FULL STORY	PHARMAs LIKE PM to AVOID REQUIRED EVI-DENCE BASED RESEARc	Weaknesses
COMBINED EFFORTS of the PHARMAs and DIAGNOSTIC INDUSTRY	INFORMED **D**RUG **D**E-VELOPMENT LEADS to a COST EFFICIENT **DD**	MAY BE USEFUL for BROADER PUBLIC HEALTH INTERVENtions	Opportunities
PREMATURE IMPLEMENTATION WILL LACK CREDIBILITY	PATIENT GROUPS MAY BECOME IMPATIENT DUE to the LONG WAY	NEW DRUG SIMILAR OUTCOME, HIGHER COST	Threats

Everyone can become a patient …

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Exemplaric Gaps from the interviews

Patients	• The way patients can access information • Independent funding for academic research • Lack of education by the public media about personalised medicine
Basic research	• Basic research and PM are too far apart with the exception of some cancer types
Translational research	• Researchers seem not to be really aware of today's daily life needs of society.
Regulation bodies	• A gap between the concept of personalised medicine and its application: e.g. companion diagnostics can exclude high risk groups, but how do we handle the increasing number of companion diagnostics when it is not a formal part of approval?
Health system	• There is currently a gap between amounts spent for oversight versus research. More oversight is required. We can use the analogy of a car factory, that would not have a sufficient quality control set established …

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Exemplaric Needs from the interviews

Patients	• We need to have companion diagnostics that differentiate and identify certain types of targeted treatment
Basic research	• Long term policies in genomics/clinical data integration • Quantitatative omics and modelling could be jointly used to improve prognosis and treatment
Translational research	• Instead of rebranding existing research as PM research, we need to increase the use of large data sets to improve clinical decisions • Involve professionals at an early stage to draft an implementation plan. That way the can feel the innovation is theirs as well
Regulation bodies	• Funders should focus on generating evidence that shows a link between specific tests and outcomes of treatment • Sustainability must be a concern in the study environment
Health system	• Ways of defining which kind of information should be available to citizens to empower them (Health literacy)

Many more results from the strategic reports and the interviews can be found in the workshop documents

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Workshop sessions

An introduction

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Grouped by working area

1. Basic research
2. Translational research
3. Regulation
4. Health care system

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Aim of the sessions

To narrow down the focus of PerMed to draft a workable strategic agenda for personalised medicine..

.. in line with other efforts in the field (CaSyM/ERA-net systems medicine; strategic approaches by stakeholders)..

..to complete the research cycle bringing personalised medicine forth into the medical practice..

..and back to research.

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Session tasks

1. Rank five major areas to be prioritized
2. Rank three specific areas within the five major areas
3. How would you link to the other working groups: identify any overlaps and complimentarities to:
   - basic research
   - translational research
   - regulatory issues
   - healthcare systems
4. How do you take into account the patient/citizen perspective and cultural aspects.

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Provide the reasoning to each choice made

- Ex equo is possible, provided the reasoning
- The results from the reports and interviews about gaps and needs can be used as guidance and structure
- Linking your detail priority areas to the areas of the other working groups is a pre-requisite (mind the research cycle)
- Identification of missing expert areas and representatives to be involved right from the start will be appreciated.

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"If any country has some evidence on PM in a pilot, there is a good reason to convince others to develop another means to check the validity or utility in another setting" Off record interview

Wishing you inspiring discussions

Many thanks for your input!

The PerMed partners

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