Innovation in Personalized Medicine: Is Our Healthcare System Ready?

Institute of Health Economics/Canadian Association for Population Therapeutics Precision Health Workshop, October 22 2017

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McGill University, PhDc "Patenting and Biomedical Innovation, a Balancing Act"

1. Implementing personalized medicine innovations that are **"ready for use".**

2. Sustaining long-term improvement and transformation of healthcare

The Promise: Potential for Transformation

- Direct targeted therapy, reduce trial-and-error prescribing
- Reduce adverse drug reactions
- Reveal new uses for medicines and drug candidates
- Shift emphasis in medicine from reaction to prevention
- Inform health care spending

PM Innovation - Status Quo

132 — FDA approved drugs – test indicate on label

42% — drugs in development with biomarker (73% in onco.)

$25 B — Market value approved drugs with CDx

25% — % NME approved 2016 - test indicated (5% 2005)

6,5000 — # genetic tests, single or multiple BM, available in US

Personalized Medicine

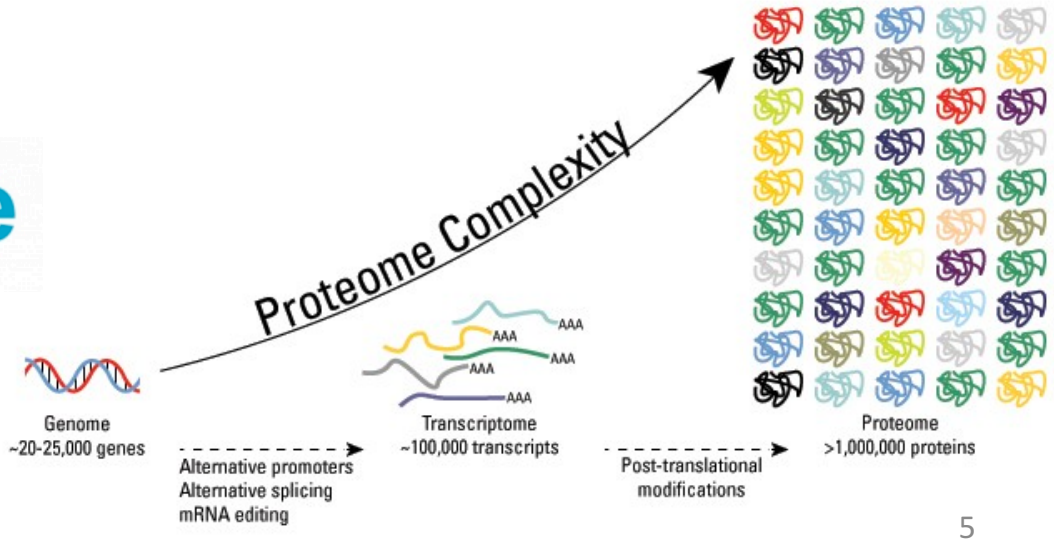
Pharmacogenomics

genomic medicine

Stratified Medicine

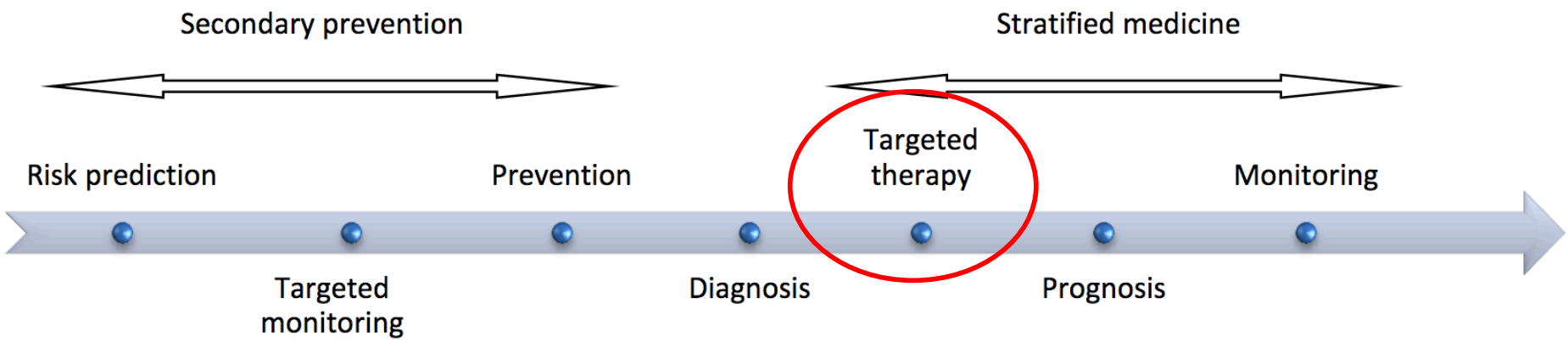
PRECISION MEDICINE

3 BILLION
base pairs in DNA, which code for
20,000
genes in the human body



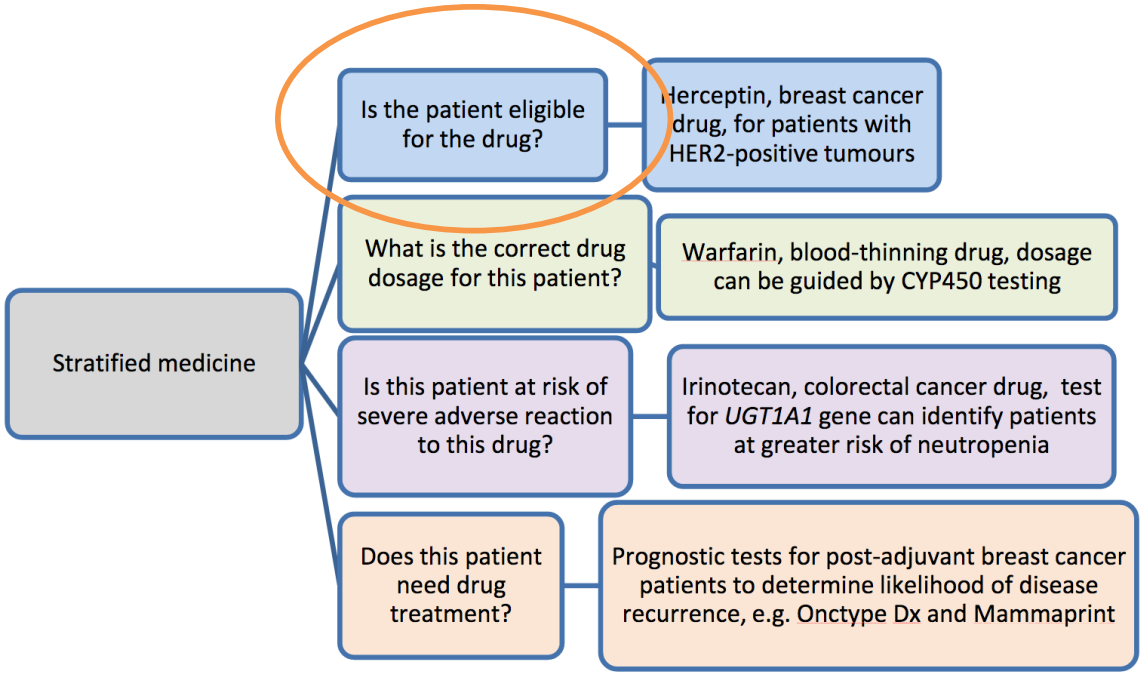
Clinical Application of MDx

Figure 1: Expanding the Use of Diagnostics



* Extracted from Personalized Medicine - A Topology Briefing for CADTH, Hogarth 2016

Clinical Question Related to a Therapy



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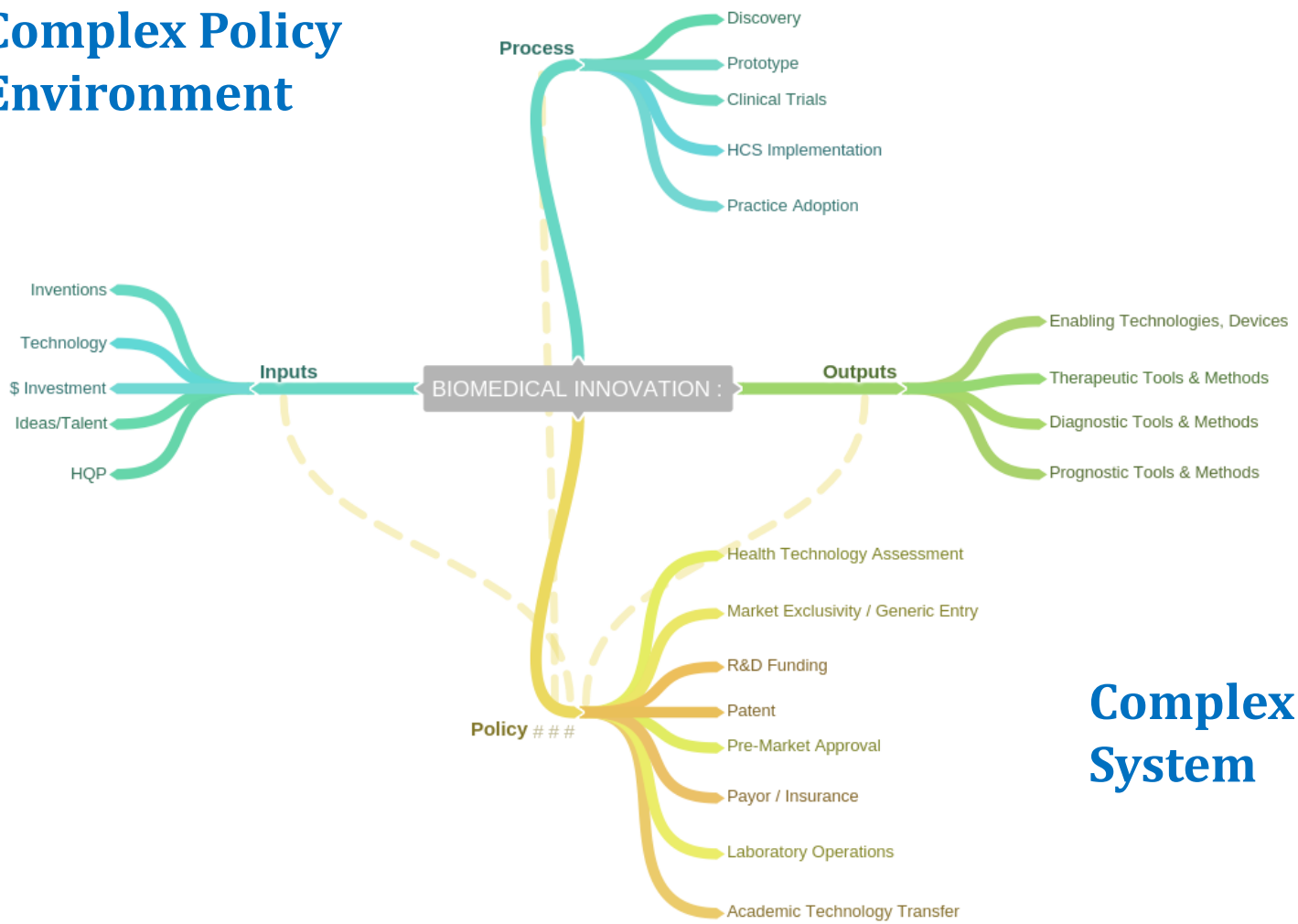
Complex Policy Environment



Complex Innovation System

Companion Diagnostics (CDx) Policy Discussion Paper

Issues, Gaps and Opportunities for Change in Canada

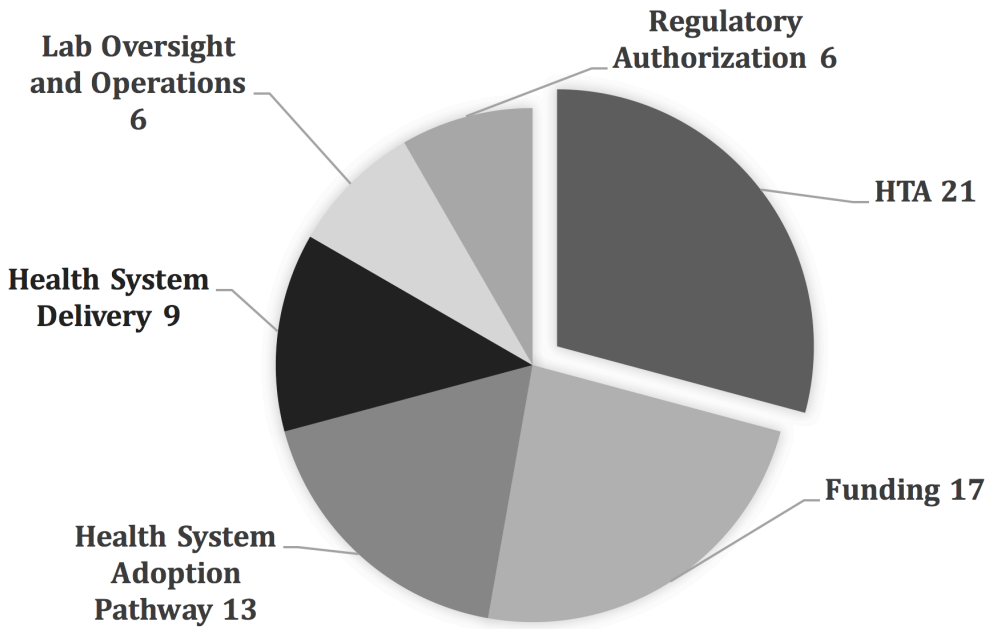
21 Documents Published 2012-2016

inclusion criteria

Expressed **Canadian** stakeholder opinions on **policy or operational** problems related to Canadian HCS **evaluation**, **funding** and **delivery** of **companion diagnostic** test

Sector	Stakeholder Type	Times Represented
Public	Provider	35
Public	Academic	25
Private	Diagnostics Company	12
Public	HTA Body	11
Public	Research Funding	9
Public / NPO	Advocacy	6
Public	Centre of Excellence-Network	5
Public	Policy Maker	5
Private	Pharmaceutical Company	3
Private	Consulting Firm	1
	TOTAL	**112**

Areas of Concern Expressed



Total NR Problems Expressed = 38

Policy / Operational Problems
Evaluation - Funding - Delivery

Thought Leaders: 2012-2016

Stakeholder Organization	Times Represented
Canadian Agency for Drugs and Technologies in Health (CADTH)	7
Diagnostic Services Manitoba	5
University of Toronto	5
Canadian Institutes of Health Research (CIHR)	4
Alberta Health Services	4
Roche Diagnostics	3
Ontario Ministry of Health and Long-Term Care	3
BC Cancer Agency	3
Canadian Partnership Against Cancer	3

Total Organizations Represented = 112

U.S. FOOD & DRUG ADMINISTRATION

- how does this translate into Canada
- articulate how & why our system is different
- national harmonization
- variety of approaches for a variety of molecular tests

Health Canada

FIGURE 10: POLICY AND GUIDANCE DOCUMENTS FROM THE U.S. FDA

2005	*Pharmacogenomic Data Submissions* (final guidance)
2007	*Pharmacogenomic Tests and Genetic Tests for Heritable Markers* (final guidance)
2007	*In Vitro Diagnostic Multivariate Index Assays* (draft guidance)
2008	*E15 Definitions for Genomic Biomarkers, Pharmacogenomics, Pharmacogenetics, Genomic Data, and Sample Coding Categories* (final guidance)
2011	*E16 Guidance on Biomarkers Related to Drug or Biotechnology Product Development: Context, Structure, and Format of Qualifications Submissions* (final guidance)
2012	*Enrichment Strategies for Clinical Trials to Support Approval of Human Drugs and Biological Products* (draft guidance)
2013	*Clinical Pharmacogenomics: Premarket Evaluation in Early-Phase Clinical Studies and Recommendations for Labeling* (final guidance)
2013	*Clinical Pharmacogenomics: Premarket Evaluation in Early-Phase Clinical Studies and Recommendations for Labeling* (final guidance)
2014	*Qualification Process for Drug Development Tools* (final guidance)
2014	*In Vitro Companion Diagnostic Devices* (final guidance)
2014	*Framework for Regulatory Oversight of Laboratory Developed Tests (LDTs)* (draft guidance)
2014	*FDA Notification and Medical Device Reporting for Laboratory Developed Tests (LDTs)* (draft guidance)
2016	*Use of Standards in FDA Regulatory Oversight of Next Generation Sequencing (NGS)-Based In Vitro Diagnostics (IVDs) Used for Diagnosing Germline Diseases* (draft guidance)
2016	*Use of Public Human Genetic Variant Databases to Support Clinical Validity for Next Generation Sequencing (NGS)-Based In Vitro Diagnostics* (draft guidance)
2016	*Principles for Codevelopment of an In Vitro Companion Diagnostic Device with a Therapeutic Product* (draft guidance)
2017	*Discussion Paper on Laboratory Developed Tests (LDTs)* (discussion paper)

- **Many problems** identified, few solutions
- **Good solutions** require well-defined problems
- Perspective, experience, knowledge of **ALL** stakeholders needed for well-defined problems
- Must consider short- & long-term **objectives** broadly
- Willingness to support cost-efficiency and **innovation** is critical