



PROOF
Centre of | Centre d'
EXCELLENCE

*Biomarker
solutions for
health care.*
*Biomarqueurs
– Solutions en soins
de santé.*

Regulatory Challenges and Opportunities in the Era of Molecular Solutions for Health Care

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Wednesday, March 9, 2011



Government of Canada
Networks of Centres
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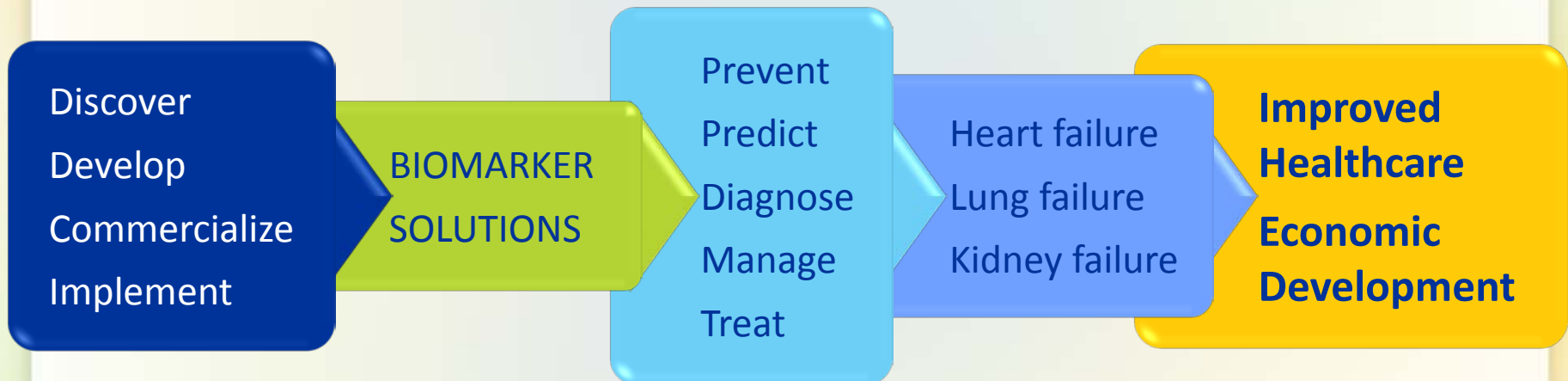


a place of mind
THE UNIVERSITY OF BRITISH COLUMBIA

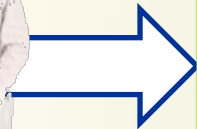


PROOF Centre Mission

- Not-for-profit Society established in 2008 through federal funding as an NCE CECR
- Anchored at St. Paul's Hospital and hosted by the University of British Columbia



What are biomarker solutions?



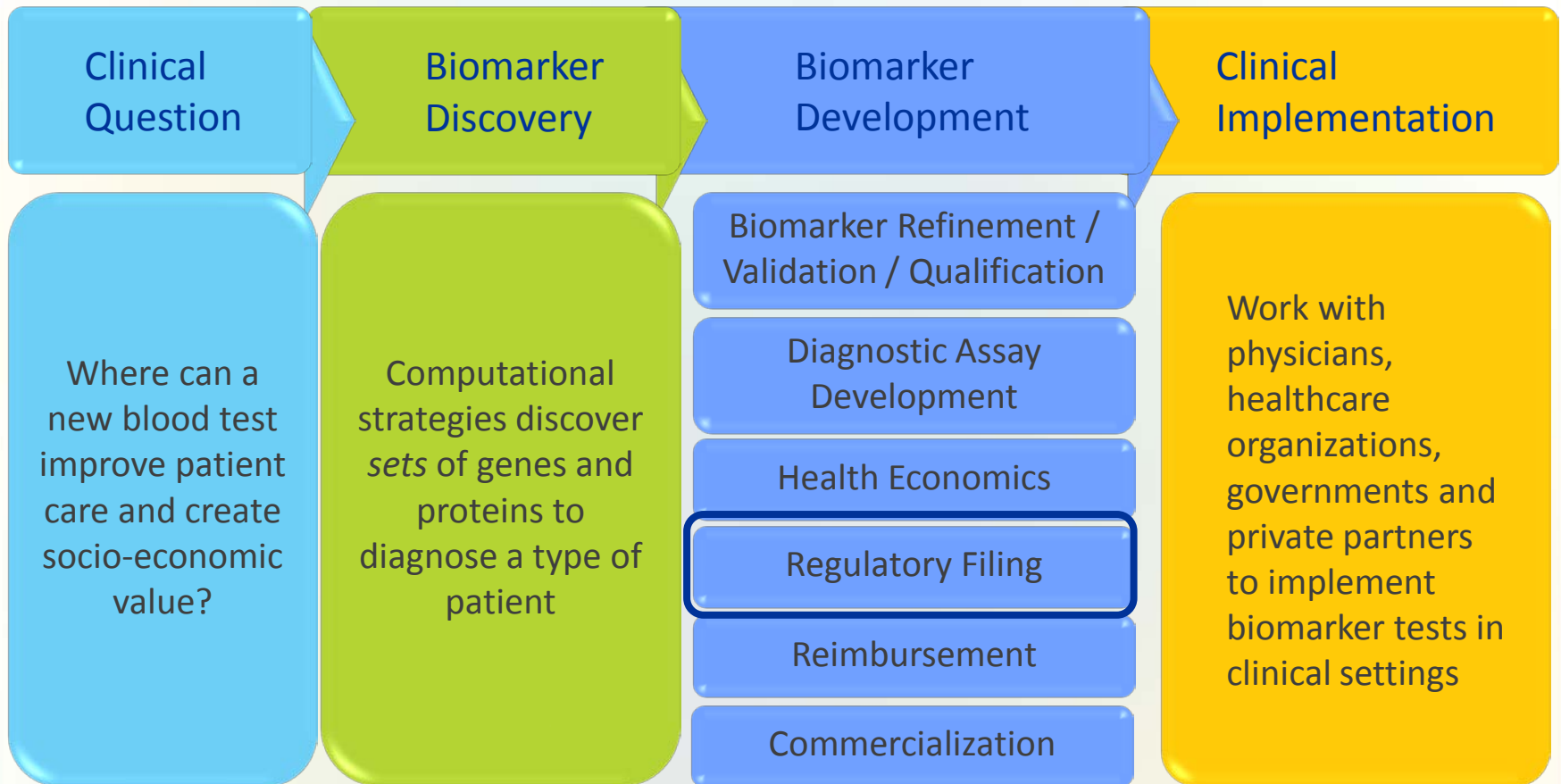
**BIOMARKER
SOLUTIONS**

Sets of genes and/or proteins in the blood identified using a non-targeted approach and computational power, in combination with clinical variables and knowledge derived from other work, that address a clinical need





PROOF Centre Biomarker Pipeline





Major Challenges / Opportunities

Biomarker Discovery

Analytical
Validity

Clinical
Validity

Clinical
Utility

Outcomes
Evaluation

IP Clinical Utility / Validation FDA Adoption Evaluation

Pre-
Clinical

Phase I – Phase III
Clinical Trial

Phase IV
(Post-market
Evaluation)

Therapeutic Discovery





Major Challenges / Opportunities

Intellectual Property

- Can a gene be patented?
- What about panels of molecular signatures that contain previously patented genes or proteins?
- What are new possibilities for intellectual property that allows for increased utilization of knowledge?

M^URIAD
GENE PATENT LITIGATION





Major Challenges / Opportunities

Clinical Utility and Validation

- How can we be better at...
 - Engaging clinical, health care, and payor acumen early on?
 - Evaluating options for truly improving clinical care?
- Many new discoveries to date have not been validated
 - How do we share high quality specimens and data so patients can reap the benefit?





Major Challenges / Opportunities

Regulatory Approval

- Biosimilars
- Laboratory Developed Tests (LDTs)
- Multiplex Tests (IVDMIA) / Voluntary eXploratory Data Submission
- Direct-to-Consumer
 - Advertising of Prescription Drugs
 - Genetic Testing
- Post-marketing Surveillance





Major Challenges / Opportunities

Regulatory Approval

- FDA Clearance...
 - Can be (but not always) considered a major milestone
 - Allows a company to promote and distribute the product *BUT* only within the intended use
 - Does not provide acceptance in the medical community





PROOF Centre Experience

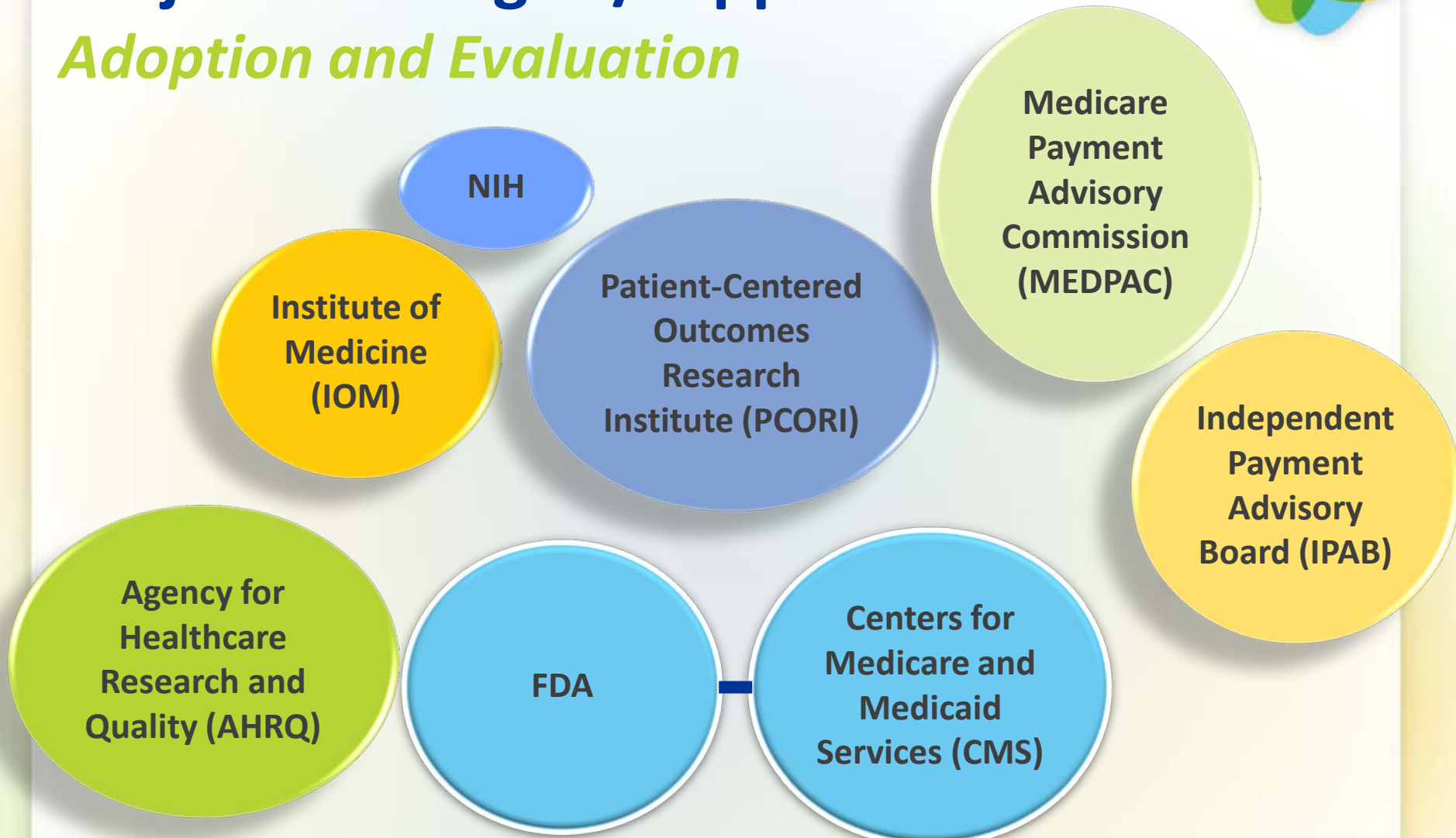
- VXDS (CDER)
 - Improved processes to bring forward validated biomarkers
 - Collaborative
- CDRH
 - Promoting pre-IDE submission to open discussions *early* in the process





Major Challenges / Opportunities

Adoption and Evaluation





Major Challenges / Opportunities

Adoption and Evaluation

- Improved *early* engagement of physicians and payors through clinical utility discussions
- [in Canada] How do we engage provinces for a more streamlined process to adopt new tests?
- [in US] CPT Coding changes underway through the Association for Molecular Pathology
- How do we ensure outcomes data is available for appropriate evaluation?



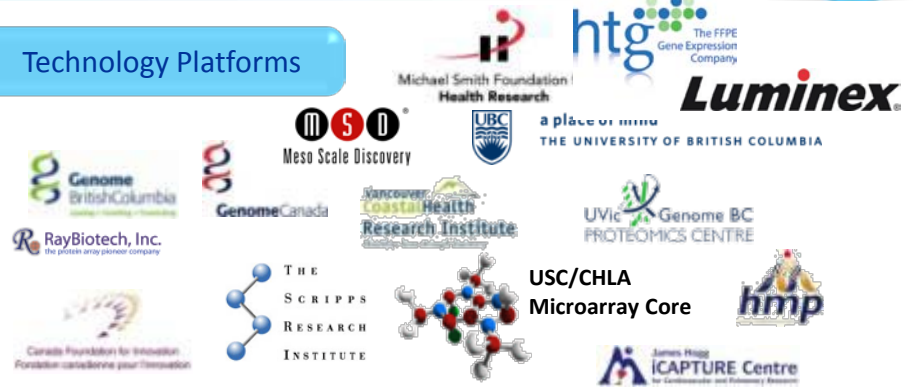
PROOF Centre Community of Partners



Patient Cohorts



Technology Platforms



Computation



Financial Resources



Biomarker Science



Health Economics



Health Systems



Commercialization

