CHAIRMAN'S SUMMARY OF WORKSHOP MESSAGES

POLICY ISSUES FOR THE DEVELOPMENT AND USE OF BIOMARKERS IN HEALTH

6-7 October 2008 – Hinxton, United Kingdom

A workshop on "Policy Issues for Biomarkers in Health" was held in Hinxton, UK in October 2008. Experts discussed draft analytical reports and how to improve the development and use of biomarkers in health care. Topics included: knowledge sharing from biomarker research; the creation of an evidence base and the clinical evaluation of biomarkers; the regulatory and policy framework for safe and efficient development of biomarkers; and business models for biomarker discovery, development and commercialisation. Experts made specific recommendations to the Working Party on Biotechnology.

The policy environment for biomarker development in pre-competitive and proof of concept R&D phases functions well.

- Biomarkers have broad applicability in research, health care and the health industry. The impact of policy will depend on the context of use and the nature of biomarkers.

- Biomarkers can be used for screening, diagnosis, risk assessment, prognosis and treatment management monitoring; a high proportion of the current workload is in treatment management and monitoring.

- With all these different possible applications, biomarkers present an opportunity to reduce public health expenditures and industry development expenses.

- However, depending on their application, there are some issues which could slow their uptake by industry or in clinical practice.

There is an evidence gap for clinical evaluation of diagnostic biomarkers.

- Government, industry and the medical community recognize that there is a need to constitute an evidence base for the clinical evaluation of biomarker-based test. The existing evidence base, though vast, is fragmented and ill suited to the specific task of determining clinical utility and subsequent implementation.

- The paucity of relevant data, knowledge and clinical studies needed for evaluation has multiple sources (*e.g.* a lack of biomarker data sharing, a lack of investment in larger scale clinical evaluation studies, and the difficulty of accessing health outcomes information).

- Funding or other incentives will be needed in order to build an evidence base for establishing the clinical evaluation as this presently falls outside the purview of either industry or government.

- The costs of building the evidence base and of evaluating and disseminating results will need to be shared (between industry and government and perhaps other stakeholders) and further reflection is needed on how this might be done because there is a lack of consensus on options.

- Within countries, there is a need for an independent system that will collate studies, define thresholds, and make judgements about clinical evaluation. This system will need to be able to update any evaluation in light of the rapidly evolving nature of evidence. Such evaluations will need to be disseminated to users and easily accessible.

- There needs to be agreement on the evaluation methodology with a view to share findings from studies across country boundaries, as well as incorporating them into systematic reviews and meta-analytic assessments.

- The resulting evidence base may be used in the process of market approval and/or reimbursement of diagnostic biomarker products.

The future of biomarker diagnostics will challenge business and government…

- Biomarkers will increasingly be combined into complexes of multiple assays that will deliver a risk profile for an individual. Based on sophisticated algorithms, these tools will provide both predictive and diagnostic information about health conditions. These tools will be hard for single physicians or even a community of "experts" to evaluate.

- Non-hypothesis driven identification of biomarkers through HTS will be a key element for drug discovery (*i.e.* in systems biology).

- Since diagnostics are considered low value added by health care payers in comparison with therapeutics, firms are unwilling to invest heavily in the development of diagnostic biomarkers unless they are associated with the prescription of a particular drug which captures value for the test, as is the case in pharmacogenomics or theranostics. For the moment, the reimbursement for diagnostic tests is heavily based on cost rather than on value.

- Differences across countries in the type of intellectual property protection that can be used (*i.e.* database protection, patents on software, algorithms, medical procedures and business methods) will likely have an important impact on industry strategies and the types of products that are ultimately commercialised.

- Government can influence the incentives for the development of biomarker diagnostics through changes in reimbursement policies that better reflect the value of diagnostics.

- Payers (*e.g.* governments and insurers) need to better understand the advantages and disadvantages (*i.e.* cost-effectiveness) of using biomarkers as diagnostic tests in establishing their payment and reimbursement plans.

- However, it has proven difficult to evaluate the health and economic benefits of novel diagnostic tests. More work in this area may be needed.

Changes will also be needed within medical care…

- Better information about the clinical utility of biomarkers is needed at point of care.

- Physicians and health care providers will need to be educated and receive statistical training to use and understand diagnostic biomarker results.

- There needs to be a greater recognition that introduction of diagnostic tests will change the process of care, and this will need to be taken into account if there is to be effective implementation.

- There is a need to develop diagnostic and treatment guidelines and protocols which recognise the utility of biomarkers.

- Direct-to-consumer tests and services will be increasingly available. There is no consensus whether and what oversight and governance should be in place. This is a subject for further consideration.

- Where tests are marketed directly to the public, there is concern that normal market forces will not function properly, because the difficulty of evaluating these tests will mean that purchasers may not be able to evaluate objectively what they are buying. Consequently, reports from the independent evaluation system should be made available to the public in appropriately non-technical language.

Workshop participants called for better dialogue amongst stakeholders.

- Industry, government, academia need to engage in a dialogue about *(i)* how to generate necessary validation data and *(ii)* how to move the diagnostics towards a more "value based reward" model (*e.g.* reimbursement policies, consortia, intellectual property).

- Governments have a role to play in creating a platform for dialogue amongst stakeholders as a consensus building process for understanding the use and value of biomarkers.