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Subject: **Report on the use of -omic technologies in the development of personalised medicine**
Agenda item 2 (b)

Personalised medicine starts with the patient. In practice, rather than having a unique treatment for each individual person, patients are sub-divided into groups based on their "molecular make up", i.e. using biomarkers[1]. By this stratification of patients, medical interventions can be tailored to be more effective in a particular group of patients. For the purpose of this document, the personalised medicine approaches refer to *a medical model using molecular profiling for tailoring the right therapeutic strategy for the right person at the right time, and/or to determine the predisposition to disease and/or to deliver timely and stratified prevention*. This rapidly developing science driven approach to health care has potentially very high benefits for both patients, clinicians and for health care systems alike. Some potential advantages offered by this new approach may include:

- Ability to make more informed medical decisions;
- Higher probability of desired outcomes thanks to better-targeted therapies;
- Reduced probability of adverse reactions to medicines;
- Focus on prevention and prediction of disease rather than reaction to it;
- Earlier disease intervention than has been possible in the past;
- Improved healthcare cost containment.

The importance of this medicine was notably recognized in the Commission Communication of 2008 on a Renewed Vision of the Pharmaceutical Sector. As a follow-up of this Communication, the Commission plans to issue a report on the use of -omic technologies in the development of personalised medicine this year.

[1] A biomarker is an indicator of a biological state. It is a characteristic that is objectively measured and evaluated as an indicator of normal biological processes, pathogenic processes, or pharmacologic responses to a therapeutic intervention. They can be used in the medicines development process as well as for diagnostic, prognostic, monitoring and screening purposes.

The aim of the report will be to:

1. Highlight the **potential and issues in research and development** of personalised medicine and the current **EU level research funding** relevant to the area;
2. Take stock of the **recent developments of EU legislation** to ensure the placing on the market of medicinal products and medical devices;
3. Analyse the **factors affecting the uptake of personalised medicine** including the Health Technology Assessment.

As regards the EU legislation affecting personalised medicine, the Commission intends to explain:

1. The procedure for authorising medicinal products and medical devices

Whilst in personalised medicine, the medicinal product and the screening of the genomic characteristics with a diagnostic test are closely inter-linked, the placing on the market of medicinal products and of the corresponding diagnostic medical devices follow two different regulatory frameworks. However, both regimes aim at ensuring high level of public health protection and at promoting the functioning of the internal market.

In vitro diagnostics

The Commission proposal for a revision of the EU medical devices legislation aims to reinforce the current regime for diagnostics in order to ensure an appropriate level of safety and performance as regards these tests. Moreover, the definition of the in vitro diagnostic used in the context of personalised medicine will be explicitly included in the definition of in vitro diagnostics medical device. The Commission proposal also establishes a consultation procedure for companion diagnostic to ensure that the device is suitable for the medicinal product concerned.

Medicinal products

The current EU legal framework for pharmaceuticals, coupled with detailed scientific guidance documents, enables industry to bring safe, efficacious and quality medicines to the market. This applies equally to the field of personalised medicine.

Moreover, the Commission tabled a proposal to revise the Clinical Trials Directive. Again, this is equally relevant for personalised medicine as it is for 'traditional' 'one-size-fits-all' medicines. This proposal, currently under discussion in the Council and the European Parliament, aims to respond to the decline of clinical trials in the EU in recent years. The Commission seeks to reduce unnecessary bureaucracy for industry and academia and to facilitate multi-national clinical trials. This last point is critical for personalised medicines which – because of the small patient populations – often require cross-border clinical trials. Only multinational clinical trials can reach recruitment targets and thus produce robust and reliable results. Moreover, the recently revised EU pharmacovigilance legislation offers the possibility to patients to directly report adverse events. This is expected to further facilitate integration of pharmacogenomic data into medical care. Linking adverse reaction reporting to pharmacogenetic studies could facilitate the development of a knowledge base. This will help to identify factors that increase the risk of adverse events. Such information could be useful for further product development.

2. Incentives

Placing a medicine on the market is associated with high costs for developing the product and generating pre-clinical and clinical data. The legislation offers incentives to allow industry to recoup the investments. The EU pharmaceutical legislation foresees the protection of clinical data and market exclusivity during a certain number of years.

Companies can also protect inventions by patent. In addition, the Commission provides incentives for medicines for life threatening and rare diseases. Indeed, an increasing number of such "orphan medicines"[2] are now based on a "personalised medicine approach". Possible incentives for orphan medicinal products include fee waivers, a 10 year market exclusivity period post authorisation and scientific assistance. In parallel, the European Medicines Agency can also provide scientific advice for the development of innovative products.

So far, some personalised medicinal products are already authorised by the European Commission.

For example, we have authorised a product for the treatment of patients with breast cancer whose tumours overexpressed HER2. In such disease, the cancer produces a protein called HER2 in large quantities on the surface of the tumour cells.

So, it shows that the current marketing authorisation procedure and the existing incentives can accommodate the placing on the market of medicines based on the personalised medicine approach.

Action to be taken:

For information and discussion

The Commission would like to seek the views of the group if regulatory aspects affecting personalised medicine have been properly covered. Further suggestions are welcome to feed the forthcoming report.

[2] Orphan medicinal products are intended for the diagnosis, prevention or treatment of life-threatening or very serious conditions that affect not more than 5 in 10,000 persons in the European Union.