Methodological standards for clinical trials of Personalized Medicine in patients with Haematological Malignancies

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Introduction

Personalized medicine offers a structural model for efficient healthcare. It is preventive, coordinated, and evidence-based. It relies on a network of health records that link clinical and molecular information to help patients and physicians make optimal treatment decisions. It is proactive and participatory, engaging patients in lifestyle choices and active health maintenance to compensate for genetic susceptibilities.

There is a widespread consensus that Personalized Medicine is the future of medicine, especially in Cancer. However, this future never seems to arrive due to obstacles that this proposal attempts to overcome. A major obstacle is the need for a consensus among leading clinicians on how to evaluate and integrate the multiple approaches, genomics, proteomics, cytomics, bioinformatics, etc.

We propose a FP7 under the leadership of VIVIA Biotech to create this Methodological Standards project.

Methods

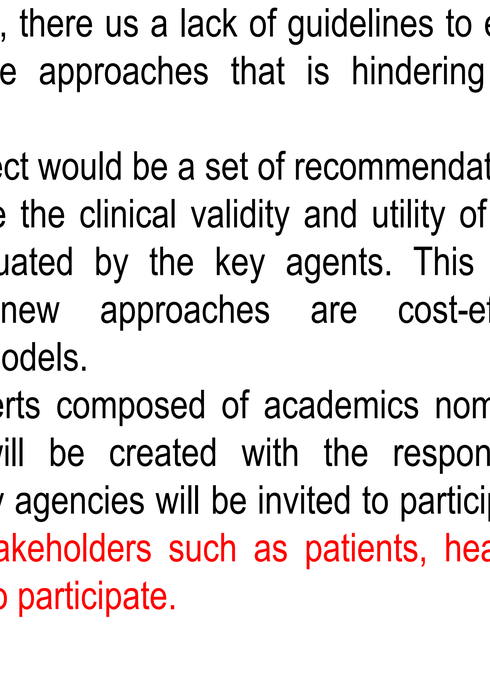
We propose to investigate and integrate the best approaches to define common standards to conduct clinical trials in Personalized Medicine (PM) within the area of oncology, hematology and AIDS. In spite of a wealth of innovation in this field, there us a lack of guidelines to evaluate clinically novel personalized medicine approaches that is hindering its implementation in clinical practice.

The result of this project would be a set of recommendations to set of European Standards to evaluate the clinical validity and utility of personalized medicine approaches consensuated by the key agents. This will include criteria to evaluate whether new approaches are cost-efficient according to pharmacoeconomic models.

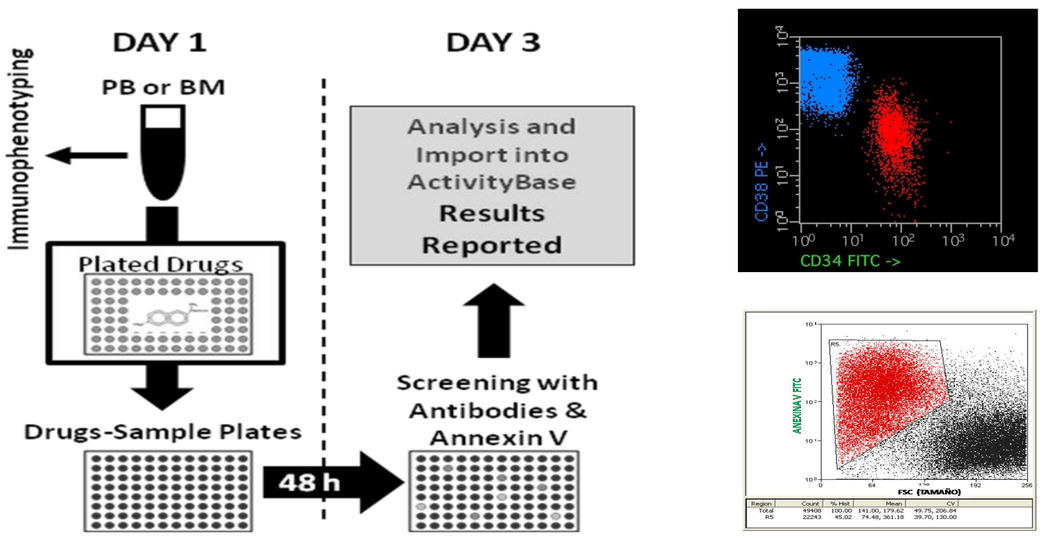
A Committee of Experts composed of academics nominated by the different scientific societies will be created with the responsibility to set up the guidelines. Regulatory agencies will be invited to participate in setting up these guidelines. Other stakeholders such as patients, health care systems, and CROs will be invited to participate.

Why we need new approach in Personalized Medicine

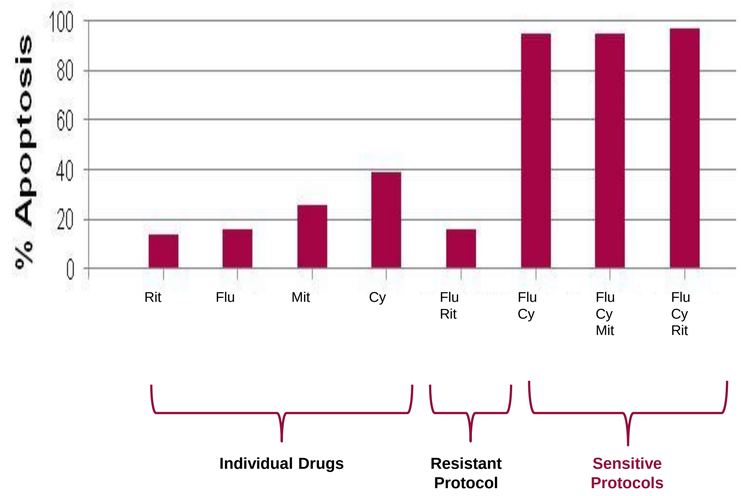
An example of Ex vivo variation in the efficacy of 4 drugs in samples from 9 CLL patients. The ability of Doxorubicin, Chlorambucil, Mitoxantrone and Vincristine to induce apoptosis in samples from 9 patients (x-axis) is analyzed. The effectiveness of the drugs varies greatly from patient to patient. The stars identify two patients in which Doxorubicin is the only effective drug.



Example of Vivia´s procedure to analyze patients sample in our PM test in Hematology.



Example of how the reports will looks like after Vivia´s PM Test



Vivia Personalized Medicine test in a CLL sample assessed ex vivo with individual drugs and polytheraphy protocols used in clinical practice. The sample is resistant to individual drugs (left) and one treatment protocol (centre). However, there are three very effective drug combinations in treatment protocols that kill more than 95% of malignant cells (right).We would recommend these protocols to the haematologist. Flu (Fludarabine), Cy (Cyclophosphamide), Mit (Mitoxantrone), Rit (Rituximab).

FP7 Phases of the work (working plan)

Objective 1. Setting up Committee with representatives of the stakeholders that would supervise the whole process of defining and setting up the guidelines for clinical trials in PM.

Objective 2. Definition of parameters in the area of: biomarkers, bioinformatics, clinical trials methodology and pharmaco-economics.

Objective 3. Consensus on a cost-effective standardized Personalized Medicine approach.

Objective 4. Observational study: The different approaches will be applied on the same patient samples without affecting patient treatment. Standardized methods for integrating and presenting the results will be devised, leading to a proposed clinical protocol for the next phase.

References

Stakeholders:

SME Companies
Clinical CRO
Clinical Society
Patients Associations
Public Health Systems
Regulatory Agencies
Specialized Media
Other participants (Reference and Support)
The Biomarkers Consortium. Foundation for the National Institute of Health. USA
EHA (European Hematology Association)