

Personalised medicine and drug development

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Propositions accompanying the thesis

Personalised Medicine and drug development: analysis of the current landscape, flexible regulatory pathways and decision supporting tools

Lada Leyens

1. *"(...) far more important to know what person the disease has than what disease the person has"* Hippocrates (c. 460-370 BCE)
2. In the same way that many diseases will fit into the definition of rare diseases due to the molecular classification of diseases and our better understanding of the biology of disease, many therapies will become targeted and personalised therapies.
3. We should no longer focus in claiming a change in regulatory pathways or new ones: they are already available. We need to invest our energy in using the available tools appropriately and developing new evidence generation tools in order to have the right tool-box for each potential drug candidate.
4. Flexible regulatory pathways developed by medicines agencies can only reach their full potential if HTA bodies stop criticizing these approaches and RWD, but try to find the best tools to make the best use of it for the benefit of the patients.
5. Dynamic biomarkers, integrating the epigenome, the changing environment, the microbiome, tumor heterogeneity and other non-static aspects that affect health, should become an essential part of diagnosis, treatment and healthcare in general.
6. "Without the right analytical methods, more data just gives a more precise estimate of the wrong thing" [Ellenberg 2015].
7. The new ground-breaking technology for editing genes, CRISPR-Cas9, opens the door to truly personalised medicine and treatment of genetic diseases; however, we need to analyse the many related ethical concerns and possibilities for miss-use.
8. In flexible regulatory approaches, where the sum of its parts is greater than the whole, it is not important if the sceptics criticize the whole but embrace the application of the parts. The final result will be positive.
9. "Il vaut mieux penser le changement, que changer le pansement". Francis Blanc. (it is better to think the change than changing the dressing –on the wound.)
10. "As complexity rises precise statements lose meaning and meaningful statements lose precision" (Lotfi A Zadeh 1965)
11. *Critics are loud but success is louder. Matshona Dhlwayo / Visionaries will always meet opposition from weak minds but the seeds they plant always save the World.* Bangambiki Habyarimana.