Maastricht University

Personalised medicine and drug development

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Summary

Short summary of the chapters:

Chapters 1-3 set the scene for the development of personalised treatments and analyse the gaps and needs for its wider implementation, as well as the current barriers. In chapter 4 we describe the available regulatory tools that aim to facilitate earlier patient access to promising medicines, showing that these tools are already available but not optimally used. Chapter 5 outlines the need to have flexible approaches at the HTA level in order to guarantee early patient access through flexible MAA processes. The last two chapters, focus on tools that can be used for decision making: in chapter 6, we discuss big data from the perspective of drug development and public health laying out the needs to ensure that it fulfils its promises for healthcare; whereas in chapter 7, we propose a framework to apply game theory to drug development.

Summary of the topic of the thesis:

Rapid advances in 'omics' sciences and technologies have facilitated the development of personalized medicine. However, there are some areas that need revision in order to reach the full implementation of personalised medicines. These gaps and needs are described in detail in the thesis, one of the most important aspects is the need for multi-stakeholder dialogue and collaboration across scientific fields and across regions. One of the aspects that have been evaluated more in detail is how the current drug development process can address the approval of personalised medicines with approaches that are more flexible than the standard drug approval process. The innovative methods developed by national and international medicines agencies to expedite promising new medicines to the market and facilitate early patient access, such as the conditional approval and the adaptive pathways, are described. We conclude that the flexible tools are available but the way we use them has to improve. However, the efforts from medicines agencies are welcome but will be in vain if health technology assessments do not follow with similar adaptive and flexible initiatives, and the European 'postcode' lottery will continue. One decision-supporting tool that can be used by decision makers is big data. Data analytics can be used across the entire healthcare value chain, from drug discovery and development through epidemiology to informed clinical decision for patients or policy making for public health. Big data has the potential to improve general health outcomes if we learn how to exploit it in the right way. Game theory is another decision supporting tool that can be applied for rational decision-making. In this work we describe a framework for its application in drug development and we give an example of how it could be applied. This application has an enormous unravelled potential. Finally, a framework is proposed, which explicitly puts together all tools that are available

to increase the flexibility of the drug regulatory system and makes it adaptive to the best available evidence. Tools such as early multi-stakeholder dialogue, iterative dialogue, implementation of control mechanisms, successive rounds of HTA with the new evidence and the introduction of MEAs and adaptive pricing mechanisms are part of the framework.