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Editorial

"Widespread orphan diseases" – A call for research, development strategies, and regulatory pathways for frequent diseases with multiple molecularly defined subgroups

Patrick Hunziker

Drug development has evolved into an endeavor associated with huge costs for single new drug, forcing the pharmaceutical industry towards development of "one-size-fits-all" blockbuster drugs for large patient cohorts. The advances in understanding of disease in the era of molecular medicine and "omics" technologies has opened our eyes to the broad variability in disease processes between individuals with the same nominal disease, e.g. breast cancer, splitting one frequent disease into multiple "molecular orphan diseases", each encompassing only small numbers of patients.[1] Distributing development cost of a single new drug among the small number of individuals with a narrowly characterized disease leads to very high cost per individual patient, costs that may be bearable for society if only a few individuals with very rare diseases are affected, but which will break the healthcare system if almost everybody will be affected at some point in his lifetime by a disease which is frequent in a conventional sense, but is a rare disease according to the new molecular definition.

Targeting – a common denominator of the future of medicine

Improving medicine requires implies delivering the right thing at the right location at the right time.

This fundamental strategy, valid for global health issues like the quest for the eradication of malaria, is likewise the key for improving healthcare economics in a national healthcare system, and at the scale of an individual patient, is a fundamental requirement to achieve better benefit/risk/cost ratios for pharmaceuticals. The key to such an improvement in efficacy while reducing toxicity and optimizing cost efficacy is targeting: understanding disease characteristics in an individual at a molecular level and using this information when establishing a therapy by targeting on the right patient group, delivering the drugs to the right tissue by targeted delivery, and targeting the right pathway by the suited drug molecule and eventually, allowing targeting in time and space by intelligent, switchable materials that deploy pharmaceutical and biological activity at specific times and location using external or internal triggers.

Shaping the medicine of the future to the benefit of patient and society

The goal is to deliver optimal therapies to the large number of patients affected by "frequent diseases" like cancer, cardiovascular disease, metabolic, neurologic or inflammatory diseases, which are or will be subdivided into a myriad of molecularly defined "frequent orphan diseases". How can we combine the evolving scientific insights in disease mechanisms, in nanocarriers, in intelligent materials, in targeted delivery strategies, and in pharmacologic blockage of critical biologic pathways into clinical reality?

The answer is neither found in the textbooks nor in the current guidelines for drug development. Academic researchers and industrial developers need to nurse a new culture of interaction between fields, of knowledge exchange and of frequent and thorough discourse in seminars, conferences and projects meetings. Funding agencies need to embrace and strengthen interdisciplinary research beyond the narrow limits of the scientific fields and need to participate actively in this community. The societal and political awareness to this new reality needs to be raised. Clinical and economic considerations need to go hand in hand with the evolution of regulatory pathways, which are currently often seen as a substantial impediment to progress. Patients desire, society demands, and ethics require new pathways that allow the economic development of targeted therapies directed at the new category of "frequent orphan disease".

The fundamental goal of this journal is to contribute to this new reality of medicine to the benefit of patients and society.

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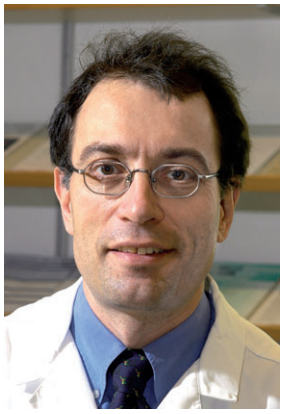
[1]The Cancer Genome Atlas Network. Comprehensive molecular portraits of human breast tumours. Nature 2012;490:61–70.



Patrick Hunziker has studied Medicine the University of Zurich, Switzerland. He received a doctoral degree based on thesis work in experimental immunology from the University of Zurich and did further research in experimental haematology at University Hospital in Zurich, Switzerland. He earned specialist degrees in Internal Medicine, Cardiology and Intensive Care Medicine. As a fellow at the Massachusetts General Hospital, Harvard Medical School, he worked on cardiac imaging in a joint project with the Massachusetts Institute of Technology, Cambridge. His professional activities in Europe, the U.S., Africa and China gave him a broad insight into the needs for the medicine of the future in a variety of settings. Hunziker became involved in medical applications of Nanoscience in the late 1990s and has been the pioneer physician in Nanomedicine in Switzerland since then. With improved prevention, diagnosis and cure of cardiovascular disease as his main research topic, he worked in the nanoscience fields of atomic force microscopy, nanoptics, micro/nanofluidics, nanomechanical sensors and polymer nanocarriers for targeting. He is the founding president of the European Society of Nanomedicine, cofounder of the European Foundation for Clinical Nanomedicine and coinitiator of the European Conference for Clinical Nanomedicine and is clinically active as deputy head of the Clinic for Intensive Care Medicine at the University Hospital Basel, Switzerland. In November 2008 Patrick Hunziker became professor for Cardiology and Intensive Care Medicine at the University of Basel.