9th HTAi ANNUAL MEETING

"HTA in Integrated Care for a Patient Centered System"

Bilbao, 23rd-27th June 2012

PLENARY SESSIONS

Monday 25th June 2012

THE ROLE OF HTA IN PERSONALIZED MEDICINE

Clifford Goodman

The Lewin Group. USA.

Most health technologies, including pharmaceuticals, biologicals, devices, and medical and surgical procedures, have been developed for use across populations. In recent years, especially with the sequencing of the human genome and rapidly growing research findings of important differences in treatment response within subpopulations in many disease areas, personalized medicine is promising to take a greater role in health care. Personalized medicine involves tailoring of health care to the particular traits, circumstances, or other characteristics of a patient that influence response to a heath care intervention. These may include genetic, sociodemographic, clinical, behavioral, environmental, and other personal traits, as well as personal preferences. Rather than the creation of interventions that are unique to a patient, personalized medicine recognizes differences in how patient subgroups respond to particular interventions, and uses that information to help guide the screening, diagnosis, and treatment of individual patients. HTA must continue to adapt to account for personalized medicine. This will involve ongoing collaboration with regulatory and payment processes, as well as with researchers, innovators, clinicians, patients, and other stakeholders. Such efforts will address, e.g., development and interpretation of new evidence, identification and validation of biomarkers, methodological adaptations, and translation of findings and recommendations for use by different users. HTA and personalized medicine will be mutually influential. Along with regulators and payers, HTA should provide clear signals to innovators regarding evidence expectations and avenues for involvement in the HTA process. While remaining current with scientific and other advances and adapting accordingly, HTA must continue its mission of objective, evidence-based inquiry to meet the demand for informed health care decisions and policies.

ICT FOR THE FUTURE OF MEDICINE AND HTA

Hans V. Westerhoff

Synthetic Systems Biology, the University of Amsterdam. Molecular Cell Physiology, VU University Amsterdam and Manchester Centre for Integrative Systems Biology, the University of Manchester. UK.

Molecules act in networks *before* they affect biological function. Systems Biology's watchmaker approach makes computer replica of pathways and networks, which may function as 'flight simulators' for physicians in training. Iit may do the same for HT assessors, patients, and research scientists. Challenges include the enormous computing and storage capacities, as well as the Babylonian confusion of the different 'cultures' (genomics, biochemistry, medicine, economics, policy) that fail to integrate constructively. As one of the potential > 1G€ ICT flagships of the European Union, ITFoM (Information, Communication and computing Technologies for the Future of Medicine) calls modern and future ICT to the rescue. IT should be able to deal with the limitations to computation, and greatly enhance the required communications between the various stakeholders in medicine. ITFoM proposes to make millions (perhaps 7 billion) mathematical-ICT models of human individuals to enable truly individualized medicine; one man one therapy. Through social (?) networking, individual patients will be directly engaged. In this presentation I shall discuss the strategies of ITFoM, in particular those regarding interactions with patients, clinicians and HT assessors and the problems surrounding the clinical trials of the future.

HTA THE WAY TOWARDS PERSONALIZED MEDICINE. UNLOCKING THE FULL POTENTIAL OF PERSONALIZED MEDICINE – A ROLE FOR HTA?

Ansgar Hebborn

F. Hoffmann-La Roche AG. Base. Switzerland.

For patients, personalized medicine (PM) promises the opportunity to benefit from the most effective treatment that targets the fundamental driver(s) of their disease, while also potentially avoiding toxicity. For payers, PM is attractive as a means to avoid wasteful expenditure on treatments that are considered not sufficiently effective. Although the enormous potential of PM has been widely recognized, the pace of implementation has been less rapid than initially hoped. Next to a considerable degree of technological unpredictability, unreasonable evidence expectations, inadequate analytical standards, conflicting stakeholder incentives and misaligned reimbursement pathways, ethical considerations, as well as legal and organizational rigidities represent the source of considerable uncertainty and barriers on the translation pathway from basic PM research to point-of-care application. Given its already prominent and further growing role in third party payer coverage decisions HTA will play some role or another in the adoption of PM. But unlocking the full potential of PM requires more from HTA. It requires that the full potential of HTA is unlocked as well. Only a broad multi-disciplinary, inclusive, and constructive approach to HTA provides the adequate framework to effectively support the various stakeholders of PM in development and utilization decisions throughout a PM's lifecycle.

IS PERSONALIZED MEDICINE REALLY AN OPPORTUNITY FOR BIOTECH COMPANIES?

Antonio Martínez

Progenika Biopharma S.A. Spain.

The identification of new biomarkers (genetic polymorphisms, proteins and metabolites) associated with the diagnosis and prognosis

of complex human diseases promises to allow the tailoring of therapies for individual patients. To enable these developments to reach patients, it is now the responsibility of biotech companies to develop new, highly accurate, diagnostic methods that are financially sustainable for health systems. The development of these tools is accompanied by significant challenges that the companies have to face and solve, particularly at the level of technology, clinical validation, market acceptance, and the complexities of regulatory and intellectual property issues. Progenika is a Spanish biotech company established in 2000 with subsidiaries in the US (Boston, MA) and Mexico DF. Progenika has developed different tools in personalized medicine in the fields of blood transfusion, biological drug monitoring and familial hypercholesterolemia and has implemented screening programmeusing these tools in several countries. The presentation, will discuss how the company has overcome these challenges exemplified by our work in the field of familial hypercholesterolaemia.

HTA THE WAY TOWARDS PERSONALIZED MEDICINE

M. Lipucci di Paola[a] and Chris Sotirelis[b]

[a]AVLT/Eurordis. Italy. [b]UKTS/Eurordis. UK.

Personalized medicine (PM) is a new promising challenge to have access to innovative and effectiveness therapies removing obstacles and delay for the delivery to the patients. In the near future the "ACCESS" to the treatments will be strongly dependent on methodology used for cost-effectiveness analysis system (HTA) and on stratified patient populations for the same disease. Under the new approach of PM the access will be applied on the basis of individual or subgroup of patients. But the patients are not yet prepared to face the new opportunity or challenge arising from the PM approach. Currently the patients' organisations have the mission to provide care and treatments to all population affected by the same condition based on the principle of equitable access. The promise of PM is it will provide an effective treatment only for few patients appropriately selected with molecular biomarkers, comparators and companion diagnostic. We have to reflect how to solve the equity and ethical issue for PM and how to improve or adapt regulatory procedures to be more flexible to the innovative treatments and how to involve patients, regulators, industry, HTA bodies and Health Authorities in an early and transparent dialogue for the benefit of all patients and the society.

Tuesday 26th June 2012

HTA FROM INVESTMENT TO DISINVESTMENT

Iñaki Gutiérrez-Ibarluzea

Osteba, Basque Office for HTA. Spain.

All the health care systems in the world are facing the same problems, costs of health care, scarce resources, increasing demand and technological imperative to introduce innovation. Main issue is how to make them sustainable and maintaining safety, quality and efficiency as paradigms and return on investment and transparency as the best answers to the society. Researchers have recently claimed that cost containment,such as reductions in salaries of professionals, benefit structures and elegibility, is not the solution. The possible savings from systematic, comprehensive and accountable reduction in low added or no added value practices and technologies are much higher than irrational cuts in services and coverage. This process has been called disinvestment and although decisions are not part of HTA bussiness, HTA community has much to say in this field. Stakeholders involvement, methodological approaches to identify, prioritise and evaluate obsolete, outmoded or superseeded practices and common policies and recommendations to face this issue will be discussed in this session. The plenary will also refer to the human trend to maintain something that is in place for ages, even when there is evidence of no value.

HTA FROM INVESTMENT TO DISINVESTMENT

Chris Henshall

HTAi. UK.

Health systems face rising patient expectations and economic pressures, and decision makers are seeking to enhance system efficiency to improve access to appropriate care. There is international interest in defining and enhancing the role of HTA to support decisions to optimize the use of established health technologies, with particular interest in "disinvesting" from low-benefit uses. Optimisation involves assessment or re-assessment of a technology, a decision on optimal use, and implementation of that decision. This may occur within a planned, routine process to improve safety and quality and create "headroom" for new technologies, or ad hoc in response to financial constraints. The term "disinvestment" is not always the best way to describe these processes. HTA contributes to both routine and ad hoc optimisation processes, but there is scope to increase its role in many systems. This presentation will summarise the main points from an HTAi Policy Forum discussion on this topic, with particular attention to actions identified for health system leaders, politicians, HTA organisations and their partners in the health system, and industry.

A PAIRED-DÉGUSTATION OF DISINVESTMENT: CHALLENGES AND INTERNATIONAL INITIATIVES

Adam Elshaug

Harvard Medical School. USA.

Current day disinvestment initiatives can be likened to 'old wine in a new bottle' for it is true that related programs have emerged and re-emerged since the 1970s. The desire to minimize waste and deliver safe, effective and efficient health care is old wine. The new bottle is represented by ever-evolving HTA methods and dove-tailed policy processes. This presentation will examine how the science of HTA has listened to the successes and failures of the past and evolved to develop more robust methods moving forward. Many of the challenges faced are universal (e.g. sources of resistance to a potential loss function; burden of evidence requirements; levers to encourage optimal use) and these will be touched upon. Initiatives, on the other hand, tend to be context specific. Several brief case studies will highlight how certain countries are using HTA to engineer health technology 'reassessment' processes to support evidence-informed, high quality health care. The jury is out on their success but, the new bottle of HTA is creating an environment where old wine is softer on the nose and smoother on the palate.

INCLUDING PATIENTS AND CITIZENS IN DISINVESTMENT DECISION-MAKING

Jackie Street

University of Adelaide. Australia.

In developing policy, it is clear that the social meaning and promise of a new technology may conflict with the evidence for potential health benefit and cost-effectiveness. Similar conflicts may occur with disinvestment initiatives and therefore, it would seem wise to

incorporate the views of citizens and patients. This presentation draws on two case studies conducted at the University of Adelaide concerning potential disinvestment from government funding, namely i) ophthalmology services (Adelaide Health Technology Assessment (AHTA)) and ii) assisted reproductive technologies (The ASTUTE Health Study). These case studies demonstrate that including community perspectives can reveal community concerns, flag problem areas, highlight otherwise hidden consequences and present opportunities for developing solutions acceptable to most people. In considering disinvestment from an entrenched and valued technology, community perspectives, although essential, can be difficult to canvass. In the Australian context, barriers may include ethical considerations, the policy context and the broader political environment. This presentation will explore who can best represent the patient or citizen voice, the role and impact of partisan voices (those with strongly held beliefs) and strategies for including community perspectives in disinvestment decision-making.

OPTIMIZING THE INTRODUCTION AND USE OF INNOVATIONS IN A HOSPITAL UNDER A CRISIS ENVIRONMENT

Josep M. Piqué

Hospital Clínic. University of Barcelona. Spain.

In the past ten years when budgets in healthcare were incremental, the decisions to introduce new technologies were basically based on effectiveness and mostly driven by physicians will. Now, budget constrains have completely changed this scenario and more rigorous process is needed either introducing new products or technologies or evaluating its performance when in use. Chances of success reducing costs are scarce if you do not have commitment of the professionals. The best way to approach the issue is by raising the awareness among professionals of the need to identify routine process that can be easily eliminated without impairing the quality of the final outcome because they are not providing a real added value. Reducing costs by means of this procedure, both managers and health professionals can create windows of opportunity to incorporate new techniques or products without increasing the total budget of the institution. In such context, accurate selection of technologies to be incorporated becomes critical since the decision have to compete among different options from different areas or departments. Therefore, the use of a methodological and a well accepted approach in assessing cost-benefit or cost-opportunity for new technology is much more crucial in this context of budget constrains.

Wednesday 27th June 2012

SIXTEEN QUESTIONS THAT CAN KILL AN INNOVATION. HTA AND e-HEALTH: AIMING FOR SYNERGY

Persephone Doupi

National Institute for Health and Welfare. Finland.

Health-IT or eHealth is an umbrella term, encompassing a variety of applications, among others Electronic Health Records (EHRs), ePrescription, Decision-Support Systems (DSS) and Telemedicine/Telehealth. Big hopes and claims have been placed on eHealth as a major driver of changes that would make healthcare practices and systems better and safer, in an efficient and cost-effective manner. Over a couple of decades and several million euros of investments later, the promises have not been quite delivered and evidence is found to be lacking or limited. With financial pressures rising, populations and work forces both aging, but also becoming increasingly mobile, the need for validated, proven eHealth solutions becomes an imperative. Could HTA hold the key to improving the quality, reliability and cost-effectiveness of eHealth? HTA namely aims at informing decision-makers about the consequences and implications of technology use, ensuring that decisions can be based on the best available evidence. At the same time, the HTA research process, as well as dissemination of findings is becoming progressively more dependent on eHealth tools. While the benefits at the intersection of the two disciplines appear to be evident, an extensive collaboration is yet to materialize. In this plenary session we intend to explore at least some of the known challenges that lie ahead: 1. In ehealth, innovation and speed are desirable properties resulting in continuously changing technologies. As a result, any form of evaluation and assessment activity has been often perceived as a hindering factor. Moreover, HTA methodology traditionally comes into play only after a technology has matured enough and evidence has been gathered that can inform decision making. How to align these needs and traditions in the best way in order to achieve the desired development and implementation of evidence-based Health-ITs? 2. In the view of many, eHealth applications are socio-technical systems. There is a constant interplay between the technology and human/social factors in the environment of implementation, which brings about changes in all involved (systems, humans, organizations, services). Further, Health-ITs are usually a combination of technologies and services, or a means supporting innovative service provision. How well does the HTA approach transfer to a domain with the features of eHealth? 3. eHealth developers and scientists, as well as the HTA community have each in their own ways approached the subject of the patient taking up a different, more defining and determining role in modern healthcare delivery. Is there a shared view and vision of the role of the patient between the two communities? What has been done in practice to achieve it? What remains to be done?

Risto Roine

Helsinki and Uusimaa Hospital District.

The rapid developments in information and communications technology have aroused growing interest also in health care which faces a constant challenge of meeting increased demands with limited budgets. Especially telemedicine and the Internet have been seen as potentially cost-saving tools for providing fair and equitable services.

Like all other health care technologies, also the new ICT technologies should, before adoption into routine use, be proved to be better or cheaper than the technologies they intend to replace. Although the scientific literature on ICT-technologies has during the last decades been growing at a fairly fast pace, the vast majority of the studies have been pilot projects that provide preliminary information about the feasibility of new technologies but rarely solid scientific information demonstrating the value of ICT applications. Such pilot studies are of limited value only for decision makers faced with the question of whether or not to invest in a service replacing traditional means of providing health care services with those based on the use ICT-technology. The limited number of good quality studies may be one of the main reasons for the fact that some of the promising new ways of delivering services, e.g. the use of Internet self-help programs, have been adopted fairly slowly. Some of these problems could perhaps be abated if the HTA community would to take a more proactive role in the assessment of ICT-technologies by reviewing and compiling results of original studies. Some efforts taken into this direction so far will be discussed in the plenary session.

SIXTEEN QUESTIONS THAT CAN KILL AN INNOVATION

Carl May

Professor of Healthcare Innovation. Faculty of Health Sciences. University of Southampton. UK.

The value of formal methods for Health Technology Assessment, demonstrated over two decades, has been to help us understand the clinical and cost effectiveness of therapeutic interventions in ways that are transportable between healthcare services. Randomised controlled trials, systematic reviews, meta-analyses, and economic modelling have played a central methodological role in the development of the field. During the same period, however, the methods of HTA have been extended to increasingly complex interventions that are recalcitrant in the face of Trials. Implementation of ICTs is often inspired by political decision-making processes; worked out through complex relationships between different agencies and organizations; and experienced as terrain changers for collaborative relationships and work amongst healthcare professionals and service users. My paper in this plenary session discusses important aspects of these shifts, and discusses the role of Normalization Process Theory (www.normalizationprocess.org), in asking the most difficult evaluation questions – can an innovation survive once the protective structure of the evaluation study is taken away?

HTA AND e-HEALTH: AN HEALTH INFORMATICS PERSPECTIVE

Jan Talmon

FACMI. Maastricht University. Maastricht. The Netherlands.

In the last couple of years, concerns have grown in the Health Informatics (HI) community about the value eHealth. In the past, the HI community has developed and implemented various eHealth applications assuming that they would be beneficial for both patients and health care providers. Both anecdotal and observational evidence has indicated that in many cases the implementation of the eHealth "solution" caused problems, in some cases even harm. In the HI community the need for proper evaluations is now well recoginized. Still the evidence base is rather weak. There are challenges that make evaluation of eHealth difficult. RCTs are not always possible, in particular for applications like PDMS in ICUs or EHRs in hospitals. Most trials are for rather specific components that can only be evaluated when the larger systems are in place. So new models are needed to evaluate and assess the impact of eHealth applications. In addition eHealth applications do not necessarily address directly a patient's condition (like a drug), but often influences the organization and workprocesses. It often results in situations where those that perceive the burden of the systems are not those that receive the benefits. As to assess the value eHealth applications a broad view has to be taken, not only from the perspective of those that are likely to benefit of the application, but from all stakeholders perspectives. There is evidence that the way the applications are implemented plays an important role as well. In the presentation some of the developments from the HI community to address these points will be presented.

ACTIVE PATIENT MANAGEMENT AND CITIZEN ENGAGEMENT

Simon Stone

Chief Clinical Architect. Oracle Corporation.

Simon Stone has been working with international clients on initiatives to use "off the shelf" software to interact with patients in the ways that they are becoming used to with banks and other service providers. This use of multi-channel communications – from phone, text, e-mail as well as paper and face to face meetings, developments in providing tele-conferencing and so on are all changing the dynamics of the relationship between healthcare providers and patients. People with long term conditions are often highly motivated to take greater responsibility for their care but have lacked access to accurate and timely information, again the rapid growth of the Internet and more recent efforts to "kitemark" sites to indicate trusted content are changing the way we look at healthcare as consumers. Similarly, as we become more comfortable with technology in the home the use of monitoring tools and other devices to measure a person's health status is becoming more acceptable and cheaper to implement. Underlying these changes however is a need to re-examine the basic relationship between the "patient" and the "provider"!