BIAC Comment on  
Health Technology Assessment (HTA) and the 
OECD Project on New and Emerging  
Health Related Technologies (NEHRT) 

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BIAC appreciates the opportunity to participate in and provide views on issues related to health related technology and innovation. In this paper we comment specifically on the OECD NEHRT Project (Part 1), as well as on the broader issue of Health Technology Assessment (HTA) (Part 2).

This paper conveys the consensus position of the constituencies of BIAC concerned with health related issues.

BIAC strongly supports the work undertaken at the OECD to study and promote innovation in general, and innovation in the health care sector - including biotechnology, in particular. Encouraging innovation as well as organising the appropriate reception of new medical products, medical devices and innovative services are key for the success of health care structures and funding in OECD countries.

**Perspective for a Collaborative Platform**

HTA is a major issue on the agenda of health care policy makers, and on that of the OECD. BIAC believes that those who provide health care technology (i.e. the private sector represented through BIAC), together with health care professionals, payers and political decision makers should engage in a collaborative dialogue.

In this respect, manufacturers should participate as an *equal partner* in any discussions organised under the auspices of OECD. At the same time, we recommend increased co-operation between Health, Social Affairs, and Science and Technology Ministries.
The collection of information on various methodologies and procedures used by countries around the world in HTA should help stakeholders to understand what health outcomes research can deliver, as well as the limitations of HTA in terms of economic evaluation and assessment of innovative health care technologies. BIAC would recommend that agreement on the terms of reference for further work involve all stakeholders from the outset.

BIAC considers that OECD discussions on HTA should be governed by a set of core policy principles, that should be agreed among stakeholders. It should be clear that the purpose of HTA is not to create another technical barrier to trade or simply to delay the entry of new technologies onto the market, but to ensure patient access to life saving and life enhancing medical technologies. HTA should assist this process of making a rational choice among different therapeutic alternatives. HTA should help improve the level of health care provided to patients.

BIAC is prepared to take part in discussions aimed at fostering the development of HTA. However, it will oppose initiatives that purport to assess the relative or added effectiveness or therapeutic value, or the cost effectiveness of a medicine at the global level. Such an approach is intrinsically linked to the particularities of a country’s health care environment and infrastructure, characterised by different morbidity and mortality patterns, treatment practices, prioritisation of health care resources, health care funding structures, direct and indirect cost impacts, etc. In short, the assessment will vary from one country to another depending on public health priorities and the clinical setting environment.

In line with this statement, BIAC will not support the establishment of a set of good practices or quality standards for the conduct of health technology assessments or economic evaluations of health care procedures, even if these good practice or quality standards do not specify protocol design and are restricted to a series of principles which lay out the quality requirements of a study instead of defining its contents per se.

Background

Medical progress is playing a major role in increasing life expectancy, improving the quality of life and eradicating diseases which were previously life threatening. Innovation brought about by those industrial sectors involved in health care have significantly improved the treatment of patients and will continue to do so in the future.

Scientific and technological breakthroughs are enabling researchers to target increasingly complex diseases. The R&D based industries’ key contribution to medical progress is to translate these fundamental research findings into innovative treatments for the benefit of patients.
The discovery and development process, however, is a risky business with no guarantees of success. Costs involved can only be sustained by companies if the economic climate encourages innovation by supporting and rewarding successful R&D, and allows fast and equitable access to the products of that research.

The dawn of the 21st Century illuminates an entirely new landscape, which is already providing entirely new approaches to many of the diseases of both the developed and the developing world. This has led – and will continue to lead – to the introduction of entirely new treatments against previously untreatable conditions.

There is a legitimate concern that these innovations provide adequate safety for the patient and that they are effective against the particular condition being treated and in a variety of patient populations. Equally, processes are in place that ensure that products are manufactured in a routine, reliable and reproducible quality. These concerns have been taken one step further when once an innovation has been made available, research will continue to establish the effectiveness of the new product in broader populations.

BIAC advocates the development of innovation in the health care sector and places the highest priority on innovation as central to the development of health care systems, whilst adhering to principles of social solidarity in access to health care. BIAC is convinced that innovations in health care will lead to greater productivity of the health care sector, and hence, will contribute to a higher level of welfare for individuals in the economy and a sustained economic growth.

Given the diversity of health care technologies and health care structures (including priority setting, organisation of health care provision, etc.), no single approach to assessment can suit all technologies or apply in all countries. For this reason, we believe that the focus of analysis in the OECD should be on methodologies such as described in the following section on Health Technology Assessment, rather than on a single approach such as suggested by the NEHRT project.

Part 1

New and Emerging Health Related Technologies (NEHRT)

The assessment of new technologies is becoming a major issue for those governing health care systems. It is BIAC’s position that OECD work in this area should proceed on the basis of a commitment to treat healthcare related innovation as central both to citizens’ aspirations to live healthier lives (i.e. their individual welfare) and to future economic development for OECD members and non-members alike.

In this context, BIAC has expressed its concerns regarding the goals and the methodology of the NEHRT project. We wish to take the opportunity to reiterate these concerns below.
The NEHRT project is characterised by **complex specifications and multiple goals** relating to assessment of innovations in the health care sector and their impacts in the management of health care systems. We feel that the project might be interpreted as leading to a conclusion that incentives for the development of innovative treatments have to be regulated due to concerns over the rising costs of new medical products and services. Moreover, OECD countries might be led to consider HTA cases a panacea for limiting health care expenditures in a “rational” way.

The NEHRT project proceeds by raising a series of **questions**, centring how HTA is conducted, how decisions are made how HTA (or other evidence) was used in that decisions, and how decisions are implemented for a sub-set of decisions. These HTA evaluation techniques are based on a piecemeal evaluation of innovations against standard treatments, often focusing on a limited range of outcomes (including costs).

It would be better to proceed with a more **holistic approach** that considers the contributions of innovations to the existing “portfolio” of interventions currently available. Here the contribution can be assessed from the perspective of all stakeholders of the national health systems.

In this respect, as is explained in greater detail below, BIAC believes that HTA should be considered within the general framework of the enabling conditions for innovation, thus supporting innovation as a contributor to societal welfare including economic growth, development, improved patient health and autonomy. Based on experience gained within industrial sectors specialising in health related R&D, which have contributed to critical progress in provision of new treatments. BIAC offers its views on HTA as a contribution to an open debate. This debate should lead to consensus among all interested parties, including providers of innovative products and services, citizens and patients, health care professionals, managers of health care services and payers.

The conclusions of the NEHRT project could have potential impacts, some negative or even harmful on every stakeholder in health systems, including:

- Investors in innovative companies
- Providers of innovative products and medical devices
- Practitioners
- Patients
- Managers of health services

Any future project could carefully consider such a range of potential impacts in the framework of enabling conditions for innovation as well as individual and social welfare.

Mindful of potential exposures, BIAC believes that any recommendations arising from the NEHRT project should be made with the following considerations:
1. The impact of the message on investors in the innovative companies

The message that OECD sends to the financial community that funds innovative companies might be influenced.

Policy makers must consider how and in what respect OECD work can encourage investment in innovative companies of the health sector.

Governments should avoid creating the perception that they are posing new barriers by raising major uncertainties for investments in innovative health sector projects.

Recommendations must be mindful of the need to maintain the flow of funds necessary for research and development by emerging innovative start-ups in the health sector.

2. The practical requirements of researchers and producers

Policy makers must recognise that major innovations are often the product of incremental or minor discoveries. Moreover, the path of innovation is never smooth or linear. It often entails failures, fumbles and uneven progress, and the full potential of an innovative product might become evident after a considerable period of practical use only.

Thus, attempting to apply existing methods of cost effectiveness to every innovation could limit the incremental innovation and reduce choice for patients and competition for innovators.

In their approach to networks of assessment and transferability, policy makers should also avoid undertaking reforms that could undermine the functioning of markets. The set up of networks of assessment should not lead to a standardisation of decisions and to the creation of de facto monopolies and bias in the purchase of the products that could lead to:

- Reduction of choice to one or two products
- Elimination of so called minor products
- Fatal market reduction for emerging products.

3. The role of physicians

Practising physicians are at the centre of the healthcare system. It is their professional duty to act independently for the benefit of their patients. Guidance most often is gained through education and training as well as experience gained among peers.

Physicians know that data from scientific research establish the principal utility of a novel treatment. However, they are not sufficient to provide guidance for the
treatment of an individual patient. Multi-morbidity, allergies or intolerance against
certain substances, cultural and psychological factors, social factors, compliance
and other parameters need to be considered and require an individual interpretation
of scientific knowledge. This is the reason why physicians must not be limited by too
restrictive formulas in choosing what is appropriate for their patients.

4. The potential for innovation by health facilities managers

In the creation of rules or institutions, policy makers should focus on creating an
environment that promotes development of innovative services and facilities across
the health care systems – both private and public. Barriers to such innovation should be minimised to the greatest extent possible.

Part 2

Health Technology Assessment

Health Technology Assessment (HTA) refers to a variety of activities applying
systematic methods of scientific inquiry to the evaluation and use of new or existing
health care technologies. The evaluation can focus on all impacts of a particular
health care technology, including its clinical, ethical, social, legal and economic
implications. It is also conducted by innovators at various stages of analysis before a
particular project is selected for further research and not abandoned. The overall
objective of HTA is to provide robust and objective information for decision-making in
health care at different levels.

BIAC supports market-based structures and approaches for the purchase and
delivery of integrated health care within the frameworks of regulation deemed
effective in health care. Transparency, choice and competition are the best way to
promote the quality of health care and help contain its costs. If HTA is used as a
method to increase transparency, it should be based on some basic principles which
are outlined below.

Choice of HTA methodology

The choice of study designs should be realistic, flexible, contingent on available data,
and allow for the continuing evolution of the field of health outcomes research. Just
as research principles have developed over time to ensure the objectivity and
reliability of clinical research studies undertaken by industry, governments and
academia, similar approaches and standards should apply to research on health
outcomes.

In this respect, the same decision criteria should apply symmetrically to old and new
interventions, and health outcomes research should be used consistently within
therapeutic areas without discrimination (i.e. they should not be applied arbitrarily to some interventions and not others, or only target innovations without also questioning existing treatments).

The choice of study methodologies and the decision making process must be objective, verifiable and transparent. A given technology can be used in different settings, the outcomes of which not only depend on the performance of the procedure itself, but also on a variety of additional factors, such as training and experience. In such a complex environment it is therefore crucial to clearly define the research questions addressed through HTA. A close interaction between all stakeholders involved (including manufacturers and intended users of the technology) will help avoiding unnecessary confusion.

Because HTA applications are being continually refined, decision makers should have realistic expectations for their use. A health outcome research methodology that works in one setting may not be appropriate to another, particularly if it is impossible to gather requisite data. In general, a diversity of approaches to HTA for a given health care intervention will lead to better results by framing the context for understanding the technology’s appropriate and effective use.

**Timing of the assessment**

Health care technologies are characterised by a constant flow of incremental product improvements. Development times will vary with the technology (e.g., 18-24 months for medical devices, compared to 8-10 years for medicinal products). These times also impact product life cycles.

There is an ongoing debate on when to assess a product innovation. Assessing an innovation early in its product life cycle could provide answers for decision makers and insurers on the issue of funding the new technology. On the other hand, there might be limitations to meaningful interpretations that can be made from HTA in the early phase of the product life. The early assessment of a technology might ignore both the learning curve phenomenon, and the fact that the process of innovation is one of continuous – and often incremental – improvements in close interaction with the users of the technology (both prescribers and patients).

The effectiveness of a new procedure (be it a device, a medicinal product, or any medical procedure) depends to a large degree on the user’s experience with the procedure. Too early an assessment could give an unrepresentative impression of the long term value of the innovation.

Because broadly based data on real-world use will not be available at the time of first marketing, HTA should not be used as an additional criterion at initial market access. If so, HTA would operate as a “fourth hurdle”, which would create an immediate disincentive for investment into innovation in health care. Marketing authorisation decisions must be based only upon safety, efficacy and quality of new health
technologies. Through long experience, this principle has provided the appropriate balance between safety and rapid market access, thereby benefiting patients and providers worldwide.

Health outcomes research is best applied to health care technologies after they have been used in a variety of clinical settings in the real world and over a long enough period to observe outcomes. How well a technology performs in a clinical trial setting is highly related to the protocol under which the product is studied. These trials focus on effectiveness and safety in a narrowly defined patient population as a basis for product approval. It would be unrealistic to include complex outcome measures for cost-efficiency parameters at this stage.

**Optimal resource allocation**

Health outcomes research may – once there is sufficient experience with new interventions in clinical practice – offer additional guidance for decisions regarding resource allocation priorities. The value of a new health technology is best demonstrated when its performance relative to other health care interventions is assessed through the collective experience of patients, health care providers, innovators and payers.

HTA should best be approached from a societal perspective, acknowledging competing uses for society’s resources. Under a societal perspective the analyst should consider all resources involved – whether they are paid for by the collective purse or the individual patient, and all health effects – including both benefits and harms.

The means for such comprehensive analysis are not always available, and therefore HTA is mostly restricted to the “health service perspective”. This can only be seen as a second-best solution which would consider all costs and benefits that occur within the national health care setting. However, if limited to considering only costs in certain subsections of the health systems (motivated by “silo-mentality”), HTA would be worthless. Moreover, decisions that are based on costs will ultimately fail patients who depend on access to life saving and life enhancing innovative technologies.

The focus of HTA is on identifying the relative value of a given medical procedure as opposed to other interventions, i.e. the identification of direct, indirect and non-tangible benefits of a product/service, including its short and long-term impact on health. Health outcomes results can change over time.

**Diversity in health care decision making**

Because health care is local, and costs of different aspects of health care vary from country to country, without clear and convincing evidence of relevance across different settings HTA findings should not be extrapolated or interpreted as having universal application.
The components of health care costs will differ according to such factors as the nature of local health care systems and clinical practice patterns. In addition, the comparative indicators used within health outcomes research studies (products and services, as well as the direct and indirect costs involved) vary from market to market, and moreover, are subject to varying interventions in the market by national governments. Thus, comparing costs across countries without accounting for these differences can introduce further distortions into the analysis.

The appraisal should ideally take into consideration variations in country-specific unit costs and national resource use patterns. However, modelling from international study data can yield valuable information, but not to the extent that it neglects the local setting. Actual market experience should guide research in the HTA area.

The “buyer-seller” relationship

HTA is used to support negotiations between purchasers (national or local) and providers (companies). Companies use these studies to demonstrate the value of their products to the health care professionals and to payers.

HTA is aimed at reviewing the effectiveness and value added of (new) health care technologies. Health outcomes research may be used to inform some types of clinical decisions and supplements other information in relation to patients’ needs and conventional practice from the disease concerned.