

Biomedicine and Health Innovation

SYNTHESIS REPORT



OECD *Innovation Strategy*

Biomedicine and Health Innovation

Synthesis Report

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Table of contents

BIOMEDICINE AND HEALTH INNOVATION: SYNTHESIS REPORT	3
I. Introduction.....	3
II. The changing nature of health innovation.....	4
III. Lessons and policy recommendations.....	7
IV. New policy challenges from advances in biomedicine.....	14
Annex. Summary of OECD reports on biomedicine and health innovation	19

BIOMEDICINE AND HEALTH INNOVATION

SYNTHESIS REPORT

I. Introduction

Over the past few years, the OECD has done important work in a number of areas related to innovation in biomedicine, including on: genetics and genomics, intellectual property rights and collaborative mechanisms and knowledge markets, health research infrastructures, translational research, regulatory policies that affect the approval and uptake of new technologies, and new business models for bringing health products to market, etc. This synthesis report presents the main lessons and policy messages that emerge from this significant body of work and **identifies the gaps in today's understanding of the health innovation process.**

It was developed as a thematic contribution to the OECD Innovation Strategy, a comprehensive policy strategy to harness innovation for stronger and more sustainable growth and development, and to address the key societal challenges of the 21st century. The recommendations in this report aim, therefore, to serve as useful reference and resource to those governments, member or non-member, interested in fostering health innovation and improving the economic and health impacts of their biomedical research sector. As innovation is increasingly acknowledged as the main driver of sustainable growth, productivity, and wealth creation, governments have a strong stake in setting up conditions that encourage it.

The recent economic crisis – and ongoing efforts to bring about a sustainable recovery – bring many of the points made into this document into even sharper relief. The crisis has caused civil society and the governments that serve them to place renewed focus on the social welfare benefits of investment in innovation. Nowhere is innovation more relevant than in the context than human health. Thus, the recent economic worries have done much to bring the issues set out in this document even further up the political agenda and have placed health provision solidly in the cross-hairs of public scrutiny and expectation. Governments must more than ever ensure they harness innovation in health technology in an efficient and effective manner.

II. The changing nature of health innovation

General innovation trends

The health innovation ecosystem is in flux. A number of key drivers are pushing research organisations to think creatively about the way they manage and capture value from existing knowledge, be it in-house or external. Some of the major engines of change are identified in this paper. However, whilst most experts agree on what the drivers are, there is no consensus as to how innovators, funders, or governments are strategically positioning themselves, on what the future structure of the sector is likely to be or on what an appropriate future governance system looks like. These uncertainties need to be addressed.

Science and technology

The biomedical research system is under enormous pressure as it has become far more diverse, has accommodated many new players globally, has distributed its knowledge intensive resources widely, is awash in information, and has become increasingly costly to maintain. Biomedical research institutions are looking for new ways of working. The organisation of research is changing – driven by informatics and the relatively new notion that collaboration and sharing of knowledge can be more than a zero sum game. Of particular note are:

- Convergence of technologies and fields – biology, engineering, IT, synthetic biology, nanotechnology.
- Digitisation of biomedical and health data, information and knowledge and the increased importance of large databases for biomedical research and health decision-making.
- The need to access multiple sources of data and to make these interoperable.
- The shift towards knowledge-driven, evidence-based innovation in medicine and biology.
- Development of platform technologies/standards as globalisation accelerates.

What is especially new is the ease of communication amongst a very broad scope of distributed, virtual and diverse knowledge resources. Sophisticated use of information technologies enables users flexibly to interconnect these resources and to deliver research efficiencies.

But the explosion of promising new fields of research and technological opportunities will not translate into broad based improvements in health care without some sound policy work and a high level of consensus amongst the OECD countries.

Industry organisation

The system of health innovation – the very process by which new medicines, vaccines, and diagnostics are produced – is suffering from declining productivity and escalating costs. The failure or attrition rate in medicines development has stayed high, despite the promise of new technology. The market perceives a pipeline problem in several of the large pharmaceutical companies and is reflecting that in the way it values businesses. This is leading to a number of mergers and

acquisitions and alliances creating some structural flux. In short, there is uncertainty over the future structure of the health biotechnology and pharmaceutical (biopharmaceutical) industry. Some drivers of change in business strategies for knowledge management in the industry include:

- The productivity decline in pharmaceutical R&D.
- Technology, including the increasing role of biotechnologies in health innovation.
- The entrance of new players as research capacity globalises and the growth in competitive firms from the emerging economies.
- Uncertainty over new developments such as how personalised medicine will be delivered, and the role of diagnostics in delivery.
- The changing nature of demand and outcome expectations, particularly the growing role played by health consumers and patient advocacy groups.

The future organisational structure of the biopharmaceutical industry is a hotly debated topic. Some see the rise of outsourcing and knowledge management services as part of the move away from fully integrated pharmaceutical companies to a more networked pharmaceutical industry ecology. Others, on the contrary, believe that the complexity and difficult capital investment decisions in the health sector portend well for a return to a more vertically integrated structure.

Financial constraints

The search for funding in early stages of research and translation, the high costs of product development and regulatory approvals, the financial pressures due to valuation of biotechnology and pharmaceutical firms, as well as constraints on the public purse have been a constant in this sector. Particular issues are:

- Escalating costs of development and higher regulatory risks.
- Growing constraints on public health expenditures.
- Funding gaps, *e.g.* the so-called “valley of death”, financing for translational research, but also gaps in available funding for diseases/approaches, etc.
- Efforts to improve valuation of assets and enterprises based on the knowledge they hold.

What are some of the specificities of the health sector and health innovation?

Health innovation is an interactive and distributed process which involves five main phases: *i)* identification of need, *ii)* research and development, *iii)* commercialization, *iv)* delivery, and *v)* diffusion. These stages are increasingly understood to be circular, iterative and highly interconnected—unlike the traditional notion of a linear step-by-step process, as so often inbred in policy. Health innovation is tightly connected to the provision, uptake and use of new treatments: feedback from purchasers, providers and patients is essential in shaping the innovation process. Feedback mechanisms are built in throughout the innovation cycle, and are the source of modifications that improve

individual products and enhance innovative capacity as a whole as well as oftentimes the focus of research.

Health innovation is a notoriously complex and expensive undertaking which involves multiple actors from the public and private sectors. At each stage of the innovation cycle, many social and economic factors can affect the development, uptake or diffusion of new health technologies. Across the OECD, a number of studies have identified major health innovation bottlenecks in the cycle strategies for removing them. While the details vary, the roadblocks share similar characteristics across countries. Innovation in health care relies on (uncertain) technical and scientific advances; involves multiple players; requires large financial commitments, high risks and long time-frames; and is highly regulated.

Moreover, the provision of health care is considered a public good and in some countries a right. The government has an important role in funding both basic and clinical research; regulating safety and efficacy; determining availability, equity and access; and often pricing and/or reimbursement of health products and services.

Finally, public opinion matters greatly and there are numerous examples of public pressure on government health policies (to provide greater access, to maintain affordability of products/services, to increase/relax the burden of regulation).

Changing role of government policy

In 2004 the OECD held two consecutive Ministerial meetings, on Science and Technology and on Health, which both concluded that the OECD needs to help deliver on the vision that the remarkable advances in the biosciences should contribute fully to improving the health of society and to sustainable growth. Since, OECD Member countries, in close cooperation with a number of non-members, have been interested in exploring what can be done to re-invent the process of health innovation so that both the efficiency and effectiveness of the process is improved.

Government policies are deemed important in: communicating a vision of what are the public policy objectives in health and innovation and developing a roadmap for the future; creating incentives for R&D; providing sustainable financing for shared research infrastructures; fostering translation of public research; in fostering openness, cooperation and networking; educating researchers for an interdisciplinary scientific future; updating the regulatory environment; building dialogue and public trust; ensuring privacy and security of personal data and samples; promoting the development of standards.

Governments may be the only voices that can take a leadership role in championing the opportunity to deliver, over the course of the next couple of decades, innovative health products and services that are evidence based, personalized, and safer and more effective. Governments should create a roadmap for delivering such health innovations. Philanthropies, government and regulatory agencies (*e.g.* US FDA, US NIH, EMEA) and some non-governmental organisations and patient groups have been at the forefront of identifying what a more efficient route to market for health products could look like. The challenge is delivering the institutions, the industrial capacity, the financing and the regulatory environment needed for a more effective innovation system.

In short, there is a need to articulate a vision of what is possible, a roadmap that identifies the individual steps that will make the vision reality, and a dialogue amongst stakeholders to agree how to go forward together. There is a significant level of OECD consensus on the steps that need to be taken, presented in the following section on Lessons and Policy Recommendations.

III. Lessons and policy recommendations

A. The future of biomedical innovation requires accessing and managing distributed networks of knowledge providers

- A more open architecture for innovation in health is emerging. Collaborations, public-private partnerships, consortia, innovation networks, brokerage facilities, prizes, and data sharing/exchange platforms are increasingly used to access dispersed sources of data, information, know-how, materials, compounds, software, methodologies, expertise, and patented innovations.
- User-driven and user-centric innovation is a new trend in biomedicine and health innovation. Formal networks of doctors and surgeons and other health professionals help provide data and feedback to innovators.
- Improved access to and exchange of biological samples and data is critically important to advancing the global scientific and medical knowledge base. The fusion and use of diverse sources of data, knowledge and technologies may require that both technical (*e.g.* for interoperability) and legal (*e.g.* intellectual property) solutions be found.
- As many knowledge assets are externally distributed, organisations derive value from the ability to access, manage and exploit knowledge from multiple sources. Open or networked health innovation requires organisation, frameworks, financing, good information and asset management and vision. These are important for both public and private research organisations.
- New research and business models more efficiently exploit such distributed networks of knowledge. However, there is no single model of firm or inter-firm organisation which has emerged as a clear success. Governments should tolerate and encourage experimentation and search for emerging best practices.
- Regulations have an impact on the incentives to participate in networks and consortia. There is a need to reduce bureaucracy and red tape, to make sure conflict of interest rules are reasonable, and to clarify anti-trust considerations.

B. Biomedical research infrastructures need to be accessible, high quality and sustainably financed

- High-quality biomedical research increasingly relies on biological and health-related infrastructures where large and diverse sources of biological, health and personal data and samples are stored, made interoperable and made accessible to a range of potential scientific users globally.

- In the life sciences, governments have a strong policy interest in developing and making accessible data, information, and knowledge to accelerate innovation. The circulation of knowledge is essential to innovation performance. New ideas emerge from the combination of existing knowledge from various sources. For example, within the scientific community, there is consensus that given the vast complexity of the human genome, progress in understanding disease will depend on the establishment, harmonisation and broad use of human biobanks and genetic research databases and in maintaining these scientific resources over the long term. But open or networked health technology innovation requires organisation, frameworks, financing, quality information, asset management and vision. Governments need to provide significant financial and human capital investments to assure the sustainability of research infrastructures. Lean economic times are when more not less attention needs to be paid to such infrastructure.
- IT infrastructures are the backbone that allow the networking of disparate databases and repositories. When building IT infrastructures and databases, **one should strive to “technology neutral” so that systems are adaptable and don’t limit the future scope of research or collaborations.**
- Quality assurance/management of the materials and data contained in research infrastructures is a prerequisite for high quality research and for facilitating exchange of data and samples. International guidelines or standards help establish good practices that can raise the quality of the materials and data and build trust and reputation locally and globally. Governments should work to ensure adherence to international guidelines such as the OECD Guidelines for Human Biobanks and Genetic Research Databases that facilitate wide access to data and materials for biomedical advances while ensuring that research is conducted in a manner respectful of participants, and that upholds human dignity, fundamental freedoms and human rights. They should endeavour to develop further guidance or standards where needed.

C. The intellectual property landscape is evolving to better leverage strategic intangible assets

- Intellectual assets, including in the form of intellectual property, are strategically key for the successful commercialisation of products and services in the health sector. The focus for governments should be on creating conditions that better leverage such assets to deliver optimal benefits both for health and for wealth.
- In the biomedical sector, the “own and protect” model for intellectual property (IP), whereby IP is mainly used “defensively” as a shield that locks innovation and protects innovators’ inventions from imitation, seems to be evolving gradually toward an “own and share” model where firms and research organisations make portions of their IP freely accessible in order to access collaborative networks, external sources of data, information and know-how.
- There is growing recognition that such shifts towards open innovation is resulting in more flexible IP management. Interest is accruing in the more efficient utilisation, valorisation and diffusion of intellectual property, knowledge and intellectual assets.

- New mechanisms for collaboration have emerged within this evolving intellectual property landscape. In order to create greater efficiencies in the use and exploitation of intellectual property, consideration has been given to the use of “collaborative mechanisms”. Intellectual property clearing-houses, exchanges, auctions, and brokerages; patent pools; model agreements and frameworks for intellectual property sharing; collaborative innovation arrangements; etc. are some of the approaches that have increasingly garnered attention. These mechanisms are used as means to improve efficiencies in the transaction of intellectual property and knowledge in order to stimulate innovation, foster R&D and promote the commercialisation of products and services.
- The establishment of such collaborative mechanisms raises a number of challenges as they normally involve very diverse areas. They may involve areas of intellectual property law and practice, competition and anti-trust law, business acumen, life sciences, and research and development in the life sciences. Often these mechanisms are put in place by a multi-disciplinary team covering these diverse areas. Governments may want to better understand the types of collaborative mechanisms available and to develop frameworks to aid in their establishment and governance.
- In their own collaborative research and consortia participation, public research organisations and universities should carefully consider the division between precompetitive and proprietary research outcomes to reflect this new reality. The management of IP in an open innovation context – as opposed to the traditional technology transfer approach of licensing out patents in one-to-one deals – remains a challenge. Universities may tend to overvalue or undervalue their IP, which can lead to difficulties in collaborating with industry as well as unnecessary costs and/or foregone rent benefits. New policies may be needed to help universities determine where on the value chain different strategies for sharing and/or licensing IP are most effective.
 - Governments can encourage a spirit of exchange and co-operative research which leads to improved access to biomedical technologies through a number of mechanisms. These include legislation, regulation, policies, guideline development, and conditions for funding or for providing grants and training and norms of practices. Policies vis-à-vis research organizations and grantees can encourage more sharing and access to publicly funded research outcomes in early stages of research.
 - Guidelines such as the OECD Guidelines for the Licensing of Genetic Inventions can encourage appropriate behaviour in the licensing and transferring of inventions and discourage the frequent use of restrictive, exclusive licenses that limit follow-on innovation and may impact access.
- New technologies – such as diagnostic biomarkers and synthetic biology – will involve a medley of different intellectual property rights (*i.e.* database protection, patents on software, algorithms, medical procedures and business methods). Differences across OECD countries in the type of intellectual property protection available will likely have an important impact on business models, industry strategies and the types of products that are ultimately commercialized. These new fields will present new challenges and require responses unlike the patent policies developed over

the last decade. Governments have a role in ensuring that the framework conditions are right to stimulate innovation in these new fields.

D. New research and business models are needed to meet economic and public health objectives

- The rapid pace of scientific and technological advancement in the life sciences, the complexity and heterogeneity of knowledge relevant to health innovation across multiple fields and subfields and the need to integrate the vast amounts both of scientific and clinical data all combine to create challenges for achieving the interoperability, knowledge integration and accumulation necessary to efficiently harvest the full benefits of the existing knowledge base.
- New models of health innovation and knowledge management are proving necessary for a number of objectives:
 - To improve the efficiency of biomedical research and facilitate incremental innovation (getting more use from knowledge and involving more organisations and individuals in research).
 - To improve the translation of research from academia to industry.
 - To increase evidence-based treatment options and deliver on the promise of personalised medicines and targeted therapies, to deliver better public health in general (across a broad range of disease groups and a broad range of the population) rather than just better private health for a select few.
 - To tackle new diseases and treatment paradigms, including high value added diagnostics, antibiotics, neglected infectious diseases.
- Some of the changes in business models are driven by technological opportunity. The move away from dependence on blockbuster drugs for treating whole populations and toward therapies that are tailored for treating individual patients, may be facilitated by a broader use of biomarkers to make early go/no go decisions in the developmental process and to better define diseases at the molecular and genetic levels.
- There is growing recognition, however, that vast amounts of data, information and knowledge in the health and biotechnology industries are held proprietary, though not part of the core business, but could be exchanged for the benefit of buyers and sellers (examples include precompetitive research data, data about research and clinical failures, in house materials and databases). One way to make more efficient use of such knowledge is the emerging practice of creating “knowledge markets”.
- “Knowledge markets” are formal mechanisms and institutions that facilitate access to and use of a wide variety of types of knowledge. Examples of such mechanisms include public private partnerships, consortia, innovation networks, brokerage facilities, prize mechanisms, and data sharing/exchange platforms. Knowledge markets make knowledge available, accessible, usable and sometimes tradable.

- In areas where there is a strong public policy interest, governments can play a catalytic role in bringing diverse parties to the table to discuss new knowledge exchange and formation mechanisms. There is an immediate need clearly to identify the incentives driving participation in knowledge markets.

E. Intellectual Asset Valuation could facilitate trade in the under-exploited knowledge assets of the biomedical sector

- The market is steadily becoming better at valuing the intellectual assets (IA) of business – even when these have not yet turned into tangible products and services. And, equally, companies are realising the benefits from clearer reporting of their intellectual assets – though many still have a long way to go if they are really to reflect their value.
- The effective valuation and exploitation of intellectual assets can be facilitated by governments in a number of ways:
 - Encouraging the reporting of intellectual assets and associated business plans.
 - Developing and harmonizing IA reporting guidelines.
 - Developing and diffusing tools that allow patents and other IA to be valued.
 - Making patenting and licensing information readily available and searchable.
- Improved methodologies for the valuation of knowledge intensive IA will facilitate the emergence of knowledge markets. Brokerages, prizes and firms that provide outsourced knowledge intensive products and services already exist. These sorts of business models likely will become more common.
- “Warehouses” or safe brokerage services could act as intermediaries between buyers and sellers and independently value knowledge-intensive assets. They could in the future help potential sellers evaluate the opportunity costs of keeping a knowledge-intensive asset in house, and make available select information about these assets to potential buyers so that they can be traded.

F. Regulatory evolution, in consultation with industry, is critical to ensure the development and diffusion of breakthrough biomedical technologies and the innovative use of existing technologies

- The financial incentives to innovators to develop new technologies are influenced both by the regulatory environment and pricing policies. Measures need to be taken to develop stable and transparent regulatory frameworks that facilitate long-term planning, provide sufficient incentives for risk-taking investment, and enable market access for goods and services based on innovative technologies.
- Reducing the cost of health products by streamlining clinical trials to make them smaller and faster while maintaining the established standards for

both efficacy and safety is a shared government-industry goal. Work needs to be done to simplify, coordinate, and informatise the permissions needed for clinical trials; to make advice consistent and standardised; to create model contracts; and further to develop early warning systems for problems.

- Early and frequent interactions between regulators and companies are critical to build a supportive framework for the development of new clinically valid endpoints – including from biomarkers, clinical practice and genetic databases – to improve clinical trial performance.
- Government, industry and the medical community recognize that there is a need to constitute an evidence base for the evaluation of biomarkers. The existing evidence base, though vast, is fragmented and ill suited to determining clinical utility. Since building the evidence base presently falls outside the purview of either industry or government and will entail possibly large costs, further reflection is needed on how funding or other incentives could be deployed as there is at present a lack of consensus on options.
- Payers (*e.g.* governments and insurers) need to better understand the advantages and disadvantages (*e.g.* cost-effectiveness) of using biomarkers as diagnostic tests in establishing their payment and reimbursement plans.
- Regulatory agency – industry dialogue established under effective governance arrangements can help develop stable, predictable, transparent regulatory pathways; improve biomarker validation and pave the way for regulatory acceptance; take on the challenge of personalised medicines and targeted therapies; tackle methodologies for next generation clinical trial design; create safe havens for new approaches to knowledge sharing and risk sharing.
- It is critically important to be able better to measure the value of new technologies. New tools, frameworks, and processes for the evaluation of new technologies may need to be developed that capture elements such as increased effectiveness, cost effectiveness and increased specificity within patient populations.
- Pharmaco-economic assessment offers yet greater promise for arriving at socially optimal outcomes in terms of promoting the right level and type of R&D investment, by giving better signals to industry as to which innovations are most highly valued. It can also be used as a tool to establish market-based incentives for investment in treatments for rare conditions.

G. End users of new biomedical technologies have an increasingly strong impact on innovators and public policy

- Public acceptance and trust is a critical factor in uptake and diffusion. Establishing clear policies with regard to the privacy and security of personal data is fundamental and applies to a wide range of health technologies (*e.g.* genetics and genomics, electronic health records). But some access to **individuals' data is necessary for advances in research**. There is thus a potential tensions between securing and sharing some information. Governments have a central role to play in finding an appropriate balance between individual rights and public health/research priorities.

- New technologies may challenge the way health care is delivered by medical professionals and in health systems. Their uptake and diffusion may require changes to existing practices and relations that go beyond training or capital investment.
- Diagnostic biomarkers, for example, will require that physicians and health care providers be educated and also receive statistical training to understand tests and results. Moreover, information about the clinical utility of biomarkers will be needed at point of care as it may affect the process of care, and this will need to be addressed if there is to be effective implementation and there is to be widespread use of biomarker technologies and capturing of the benefits associated with this.
- Direct to consumer tests and services are increasingly available. There is no consensus whether and what oversight and governance should be in place though the OECD Guidelines on Quality Assurance of Molecular Genetic Tests do provide principles and best practices for some relevant aspects. This is a subject for further consideration by governments.
- End users also can have direct impact on what research is done, either through systems of feedback of patient outcomes or – increasingly – through patient-focused non-profits directly influencing or funding research.

H. New policy tools are available to spur innovation that addresses public health priorities and global challenges. The lessons learned in their use need to be better articulated and generalised.

- Meeting global health challenges has spurred innovative approaches to inter-firm collaboration, access and use of IP, and financing mechanisms. These experiments may yield lessons for a lower cost approach to innovation in health care. Governments should identify the lessons learnt from these innovative approaches to global health challenges and should try to apply these to health innovation more generally. This was the main aim of the Noordwijk Medicines Agenda.
- A variety of push and pull mechanisms are being used to bring new health technologies for neglected diseases onto the market. The mechanisms are complements, not competing models, and no individual tool (*e.g.* public-private partnerships for product development, advanced market commitment, prizes, etc.) is a panacea nor replaces traditional development programmes in health. Governments need to better understand the available mix of approaches in order to achieve different policy goals.
- Meeting public health priorities will require developing alternative approaches to forming capital for research and development, such as through bond issues or better uses of philanthropic programmes, in order to finance health innovation programs. Governments should be better aware of the options and their relative merits. The governance of international co-operation on STI to meet such global challenges is currently under scrutiny by the OECD.
- Integration and coherence of innovation policies amongst health, science, development, trade and industry policymakers would be of enormous help to addressing public health priorities. Such an integrated approach is difficult to implement, partly due to the institutional barriers to co-operation in policy

making and implementation. Governments should implement strategies for policy coherence in health in order to improve effectiveness and deliver greater mutual convergence between healthcare priorities and the direction of innovation in biomedicine.

- Governments may want to facilitate a more active role of patients and/or their organisations in innovation policy and policymaking related to clinical trials and access to new products. Patients are important sources of innovations which remain under-utilised. New modes of communication and networking between health systems, end users and innovators are emerging that may provide a better match between public health objectives and investment in R&D. These need to be better understood by governments.

IV. New policy challenges from advances in biomedicine

There remain a number of outstanding questions with reference to the development, delivery, and uptake of health innovations where further research might be pursued in order to help inform policymaking.

The thematic areas for possible future work include:

1. Progress in governance and the regulatory system.
2. Open innovation models in health.
3. Commercialisation of innovation derived from research and health infrastructures.
4. Financing models for health innovation.
5. Intellectual asset management.
6. Patient needs and demands.
7. Metrics and indicators of health innovation.
8. Policy challenges of disruptive technologies.

1. Progress in governance and the regulatory system

New work could analyze and compare the impact of current regulatory regimes on the innovation process of different health technology sectors (*e.g.* drugs, devices, diagnostics and transplants). Do the forms of health regulation already in existence suffice to deal with innovative developments in these various sectors? Can one tease out the impact of different regulatory approaches on innovative behaviour? Can one articulate more concretely how to create a dialogue between industry and government and other parties, in order to incite change in the regulatory environment. Is pricing policy and health regulation structured so as to complement each other? The OECD is currently considering various new models for governance of health innovation in order to address some of these needs.

2. Open innovation in health

New work could explore and more clearly define the meanings of open access, open science and open innovation in the life sciences. What methods are used by companies to source external knowledge? How widespread are open

innovation global networks for biomedical research? What are the implications for the protection and safeguarding of intellectual assets and intellectual property – patents, trademarks, trade secrets, etc? This work could choose to do one or more of the following:

- **Analyse how biopharmaceutical companies are embracing “open innovation”** and are collaborating with external partners, (*e.g.*, suppliers, customers or universities) to tap into new ideas
- Assess how structural policies, such as labour market and competition policies affect the open innovation strategies of biopharmaceutical companies?
- Identify what policies can foster or enable the development of world-class clusters and networks for innovation in biomedicine
- Look at commercial models of open innovation and interface between open science product development business models and more traditional ones.
- Articulate the crucial role of infrastructures, as a pool of data and information, in creating an evidence base for the clinical evaluation of biomedical innovations. For example, by looking at Cost/benefit studies of research infrastructures, and their role in advancing both science and public goals (*e.g.* as for the development of synthetic biology). Or by addressing barriers such as interoperability and data integration challenges – do they exist and where are the problems?

3. Commercialisation of innovation derived from research and health infrastructures

New work could aim to articulate what are appropriate transparent practices for the commercialisation of innovation derived from health and research infrastructures. For example, by:

- Assessing strengths and weaknesses of emerging early access mechanisms, identify when and where they can be used, complementarity versus trade-offs, and how these affect commercialization.
- Developing new methodologies and approaches for the early valuation of health innovations (*e.g.* genetic tests, biomarkers) in order to guide both investors and purchasers of innovation and to assist researchers and early stage product developers in licensing out technologies appropriately.

4. Financing models

Reliable financing is important for entrepreneurial activity and innovation. A feature of R&D for biomedical innovation is the degree of uncertainty associated with its output and related return on investment. New work could explore both financing gaps and new approaches to raising financing in health innovation. How do financial constraints affect the ability to bring products from bench to bedside? What are some of the new forms of financing that are emerging (securitization, new philanthropy)? How are they likely to affect the structure of industry or the process of innovation? Are there different financing sources for different stages in the innovation cycle? The work could also address how

macroeconomic uncertainty linked to the financial crisis has added to firm-level uncertainty.

From the public sector perspective, work could explore:

- Are financial factors influencing the orientation of the innovation process, possibly encouraging a bias towards specific areas of biomedical innovation?
- How can financing be used to encourage participation in more open innovation models or discovery networks?
- What tools can facilitate the identification of strong partnership opportunities for innovators and investors. Understand how difficulties in translational research, particularly for neglected infectious diseases are being addressed (or not) through new business models.

5. Intellectual assets management

Building on OECD's Collaborative Mechanisms project, work could explore how the management of intellectual assets (IP, human resources, networks and alliances) is changing in the health sector and what impact these strategies are having on health innovation. This work could be broadly defined and include some of the following elements:

- Structure of health industry sector: Articulate the role that players outside traditional bio/pharma industry – such as platform providers, IT – are having on organization of health industry and innovation. [Are there disruptive forms of innovation and where are they coming from?]
- Understand the impact of new global players on innovation strategy – technology acquisition, new markets.
- What is the range of potential business models in the health sector? What are some of the innovation benefits of different models? What might drive toward more networked open structures versus a return to integration? What models will deliver incremental innovations?
- Better articulate human resource needs for the post-market take up of a range of health innovations.
- Identify mechanisms that increase coordination amongst policy making bodies and document if it creates a more favourable environment for innovation.
- How can we ensure that knowledge is accessible and affordable so as to facilitate innovation and develop and market useful products?

6. Patients needs, demands and expectations

Given a number of trends, including the strength of patient groups in genomics and the democratisation of biotechnology through synthetic biology, new work on end users of health innovations may be warranted. How are patients and patient organisations influencing health innovation and its regulation? What are the feedback loops between clinical users, patients, upstream R&D and policy makers/regulators? How are countries addressing the tension between increasing public demands for new technologies and health care cost management? Can we better understand shifts in demand for health care, and how these influence the business models of health innovators?

7. Metrics and indicators of health innovation

Work could consider how to establish metrics to better evaluate the impact that policies have on health innovation, including for meaningfully measuring the impact of innovation on health systems. There is broad recognition that the metrics of innovative outputs, patents and licenses, do not capture the complexity of globally networked interactions that improve productivity or deliver new products and processes. OECD governments are struggling to understand how to describe or measure the change toward more open, interdisciplinary, collaborative and interactive innovative processes. And they are aware that their statistics and indicators do not adequately capture innovation in services and business models.

8. Policy challenges of new disruptive technologies

Work could explore the issues raised with the emergence of disruptive technologies (*e.g.* bio-nanotechnologies, stem cells and iPS, synthetic biology) and new powerful enabling platforms often based on converging technologies. The analysis could consider the impacts of these technologies and platforms along the health care value chain (see Figure 1) and address the following issues:

- Scientific and technological baseline: current status, trends and expected applications. How interdisciplinary is the technology? What are the potential impacts on biology/medicine/life sciences?
- Economic impacts: What are the actual and projected economic impacts of the new technologies and platforms?
- The innovator community: who is involved in research and development (governments, academic institution, private sectors and non profit organization), in what industries, with what levels of funding
- Networks of innovators: how is R&D organised? Is it vertically integrated or open? How interdisciplinary? Is there collaboration, consortia, public-private ties, technology transfer; multi-stakeholder involvement; information sharing? Does there need to be?
- Opportunities and roadblocks: Are there identified roadblocks or accelerators for the development of the technology? What are the financial needs, gaps? What are the business strategies vis-à-vis the technology?
- How is IP protected, accessed? Any perceived or real problems? What ownership arrangements have been created?
- Policy concerns – scientific, safety, industrial/commercial, ethical?
- Are existing regulatory mechanisms adequate to address the safety (worker or consumer safety, environmental) or security (bioweapons/bioterrorism) risks, if any?
- Is there a need to engage in dialogue with the broader public? On what issues? How does one define the terms of the debate?
- Is there a need to develop good practices, codes of conduct, self regulatory mechanisms for the researcher community, the private sector?

- What infrastructure is needed to facilitate research and product development? Who is developing it? How is it financed? How open is it to a broad user community?

The OECD will take forward a number of these challenges as part of its follow up to the 2010 Innovation Strategy. Advances in health technologies and biomedicine offer a great deal of promise for the improvement of the efficiency and effectiveness of health care. But to make optimum progress, countries – member and non-member of the OECD, will need to work together to develop the necessary policy environment. The Working Party on Biotechnology will continue to play an important role in this process.

ANNEX 1

Summary of OECD reports on biomedicine and health innovation

This annex lists the OECD documents analysed for this report. The documents are sorted according to four different themes or modules that are:

1. Access to knowledge and intellectual property
2. New business models: the creative fusion and exchange of knowledge
3. The governance of new research and health infrastructures
4. The demand and take-up of health innovations

They are also grouped by level of country consensus to the messages they contain. The classification is as follows and goes from high to low level of country consensus around the documents:

1. **Instruments.** OECD Instruments are adopted by the OECD Council at a whole-of-government level. While Recommendations are not binding, they represent a commitment on the part of countries to implement the Recommendations. Guidelines or Ministerial Declarations adopted or issued by particular Committees also represent a willingness to implement but have not been vetted at a whole-of-government level.
2. **Committee reports.** Reports from Committees or horizontal projects have been discussed by committees and, depending on the Committee practice, are either declassified by them by consensus or are released under the authority of the Secretary General.
3. **Workshops.** *Workshop summaries or rapporteur's reports* are simply a reporting on the outcomes of an OECD organised conference or workshop, they are usually discussed by Committees but may not represent a consensus view of Member countries.
4. **Consultant reports.** Consultancy reports are commissioned pieces of work which do not necessarily represent the views of the OECD. Committees may decide on their declassification, but the judgement is mostly about that of quality and accuracy of the work, rather than the political agreement with the content of the report. Similarly, the work from the Bioeconomy 2030 was done by a group of consultants outside a regular Committee.

1. Access to knowledge and intellectual property

Explanation of the policy issue

There is a tension between the need to protect intellectual property rights and the need to ensure their availability and accessibility for further innovation. OECD countries are interested in understanding the mix of mechanisms and practices that incentivise R&D (be it through proprietary rights or other benefits to the inventor) and that facilitate access to and use of the fruits of research for follow-on innovation and translation into new products and services. This set of documents discusses current intellectual property regimes and their impact on innovation and access. Also explored are emerging trends, mechanisms, and best practices for ensuring access and exploitation of intellectual assets so as to maximize innovative capacity in the economy.

Two main questions were explored in the analysis:

- a. What are the “best practice” mechanisms/approaches for facilitating greater access to and value capture from knowledge and IP?
- b. What policies and infrastructures are needed to facilitate the use of IP management practices that enhance the innovative capacity of the economy as a whole? For example, what policies and infrastructures improve the ability of actors to cooperate and to improve knowledge fusion and exchange?

Related work done in the OECD

Instruments

OECD (2006), *Principles and Guidelines for Access to Research Data from Public Funding*, STI/TIP, www.oecd.org/document/55/0,3343,en_2649_34293_38500791_1_1_1_37417,00.html

This set of principles and guidelines was composed in response to a Ministerial meeting in 2004 that expressed the need “to develop a set of OECD guidelines based on commonly agreed principles to facilitate optimal cost-effective access to digital research data from public funding”. The Principles and Guidelines contained in this document should assist governments, research support and funding organisations, research institutions and researchers themselves in dealing with the barriers and challenges to the international sharing of, and access to, research data.

OECD (2005). *Recommendation of the Council on the Licensing of Genetic Inventions*. www.oecd.org/sti/biotechnology/licensing

These Guidelines offer principles and best practices for the licensing of genetic inventions used in human health care. They are targeted at all those involved in health R&D as well as those providing services in health, and particularly at those that involved in setting licensing norms and practices for such inventions (*e.g.* funding bodies, technology transfer offices). The Guidelines are intended to assist both OECD and non-OECD governments in the development of governmental policies as well as in their efforts to encourage appropriate behaviour in the licensing and transferring of genetic inventions.

Reports for Committees

OECD (forthcoming), *Knowledge Markets in the Life Sciences*; OECD (2011). “**Knowledge Markets in the Life Sciences: Issues Paper**”, www.oecd.org/document/55/0,3343,en_2649_34537_39407351_1_1_1_1,00.html

This is an issues paper prepared for an expert workshop on knowledge markets held in 2008. The workshop explored the factors that are influencing **the creation of new ‘markets’ for knowledge-based assets**, including intellectual property, information, data, goods and services; as well as identify what strategies different developers and users of knowledge are using to better identify, access, exploit, and create value from intellectual assets.

OECD (forthcoming) *Collaborative Mechanisms for Intellectual Property the Management in the Life Sciences*, www.oecd.org/document/9/0,3343,en_2649_34537_39406921_1_1_1_1,00.html

This report provides the context for the use of collaborative mechanisms, for the management of intellectual property exploring different types of collaborative mechanisms, as well as their nature, scope, structure and application. It also examines the possible employment of these mechanisms for stimulating innovation, encouraging access for R&D and for the diffusion of technology.

OECD (2008). “Open Innovation and Global Networks”. www.oecd.org/document/43/0,3343,en_2649_34273_41441387_1_1_1_1,00.html

This is a summary report of a conference hosted by the OECD and the Dutch **Ministry of Economic Affairs on “Globalisation and Open Innovation”** about the policy implications of the acceleration in the globalisation of research and **innovation and the shift towards “open innovation” strategies in companies**. The discussion centred around three main topics: *i)* the rise of open innovation and open business models in the context of globalisation; *ii)* the evidence for open innovation, drawing mainly on company case studies; and *iii)* the implications for science, technology and innovation policies.

OECD (2008), “**Intellectual Assets and Value Creation: Synthesis Report**”, www.oecd.org/dataoecd/36/35/40637101.pdf

This synthesis report follows up on the *Creating Value from Intellectual Assets* report of 2006. It was endorsed by Ministers. In order to deepen understanding of intellectual assets in relation to innovation and value creation, they are looked at on three levels: Macro-level (national accounts and estimations of investment in intellectual assets), regional-level (the regional dimension of innovation, firm location and linkage) and firm level (corporate reporting, value creation, SMEs).

OECD (2007). “**The Economic Impact of Counterfeiting and Piracy of Pharmaceuticals**” www.oecd.org/dataoecd/11/38/38704571.pdf

This report is the result of a horizontal OECD project on the “**Economic Impact of Counterfeiting and Piracy.**” It assesses the structure and scope of the market for counterfeit and pirated products and presents frameworks both for assessing the economic effects of such products and the effectiveness of policies and other initiatives to combat counterfeiting. Several industries are examined in detail, among which the pharmaceutical industry. Finally, suggestions are made for strengthening policies and practices that combat counterfeiting and piracy.

OECD (2006). “Research Use of Patented Knowledge: A Review”, OECD Science, Technology and Industry Working Paper 2006/2, <http://dx.doi.org/10.1787/18151965>

This STI Working Paper is a review of the issues related to research access to patented inventions. The key question addressed is how to ensure access to inventions for follow-on research, while simultaneously providing incentives for the original inventor. While several options for research exemptions are examined, the authors conclude that more research is needed to ascertain whether the absence of research exemptions is in fact having a deleterious effect on scientific inquiry.

OECD (2006). “Valuation and Exploitation of Intellectual Property.” www.oecd.org/dataoecd/62/52/37031481.pdf

This STI Working Paper addresses the valuation and exploitation of intellectual property, not just by developing products based on one’s own patents but also by licensing them to other firms or public research organisations (PROs), using them as bargaining chips in negotiations with other firms, and as a means of attracting external financing from institutional investors, banks, venture capitalists and other sources.

OECD (2004). “Intellectual Property Rights and Competition Policy with a Focus on Biotechnology,” roundtable materials and background note. www.oecd.org/dataoecd/61/48/34306055.pdf

This document presents the papers for a Roundtable discussion on Intellectual Property Rights held by the Competition Committee in June 2004 (a Background Note, Executive Summary, individual country contributions). The papers consider the interdependence of competition and intellectual property policy, and the role of competition and IP agencies in assuring that IP does not impede fair competition.

OECD (2003). “Genetic Inventions, Intellectual Property Rights, and Licensing Practices: Evidence and Policies”. www.oecd.org/dataoecd/42/21/2491084.pdf

This publication is based on a 2002 expert workshop which discussed the challenges raised by the proliferation of patents on genetic material (e.g. blocking patents, patent thickets, freedom to operate), and the licensing practices of public and private actors. It discusses the advantages and disadvantages of various policy measures which could be used to address access issues for genetic inventions.

Workshop summaries

OECD (2007). “Report of the High Level Forum on the Availability of Medicines for Neglected and Emerging Infectious Diseases.” www.oecd.org/document/45/0,3343,en_2649_34537_39163757_1_1_1_1,00.html

This document reviews the outcomes of the OECD High-Level Forum on Medicines for Neglected and Emerging Infectious Diseases: Policy Coherence to Enhance the Availability which was held in the Netherlands in 2007. The High Level Forum issued the Noordwijk Medicines Agenda, (NMA) which summarises the main actions participants at the Forum agreed were necessary to accelerate the development and delivery of new medicines, vaccines and diagnostics for the emerging and neglected infectious diseases that primarily affect developing countries.

Consultant reports

Gold, R., M. Herder, M. Trommether, (2008). “The Role of Biotechnology Intellectual Property Rights in the Bioeconomy of 2030,” *BioEconomy 2030*.
www.oecd.org/futures/bioeconomy/2030

This paper predicts the role that intellectual property will play in the Bioeconomy of 2030. It argues that while it will remain important, IP will not shape the Bioeconomy. If countries, industry and public institutions manage to develop collaborative platforms for sharing and disseminating knowledge and innovation, then we can expect a dynamic Bioeconomy with reduced regulatory costs in 2030. If such platforms are not created, we can expect increased transaction costs, increased regulatory costs and less innovation.

Herder, M. and R. Gold (2008), “Intellectual Property Issues in Biotechnology: Health and Industry,” *Bioeconomy 2030*, www.oecd.org/futures/bioeconomy/2030.

This paper tackles the question of whether the current IP system incentivises innovation or impedes it, and how this affects health and industrial outcomes. The conclusion is that we still lack knowledge and appropriate indicators to assess how IP rights are driving or impeding innovation.

Chris Dent, Paul Jensen, Sophie Waller and Beth Webster (2006). “Research Use of Patented Knowledge: A Review”, www.oecd.org/dataoecd/15/16/36311146.pdf

This working paper reviews issues related to research access to patented inventions, with a particular focus on the role of research exemptions (or experimental use exemptions) in protecting such access. It outlines factors that may affect the ability of researchers to access patented inventions for legitimate research purposes, it reviews evidence of current and anticipated limitations on access, and explores different options for the formulation of research exemptions that balance research use and patent holders’ rights.

2 .New business models: the creative fusion and exchange of knowledge

1. Explanation of the policy issue

The private sector has the central role in the process of developing and delivering new health products and services. Business strategies, knowledge sourcing and management, interfirm relationships in the health sector have been the subject of many OECD documents. As a short hand, the OECD refers to these topics as research and business models.

This module focuses on the processes of knowledge creation, diffusion and exploitation in the health sector. Some of the documents explore the health research system architecture: who are the different actors and enterprises involved in innovation and what are the channels they employ for accessing and commercialising knowledge and innovations (*e.g.* academic or government research, inter-business or public-private partnerships, internal investment in R&D, non technological and user-driven innovation). Some of the documents explore how and why inter-firm and inter-organisation relationships are changing. New types of partnerships, new business models, the integration of different technologies and sectors, new way of thinking, are driving a new vision of how to apply science and technology for innovations in health. Some of the documents focus on the impact of new biomedical technologies on business models (and vice versa).

2. Related work done in the OECD

Committee reports

OECD (forthcoming), *Knowledge Markets in the Life Sciences*; OECD (2011). “*Knowledge Markets in the Life Sciences: Issues Paper*”, www.oecd.org/document/55/0,3343,en_2649_34537_39407351_1_1_1_1,00.html

This is an issues paper prepared for an Expert Workshop on Knowledge Markets held in 2008. The workshop explored the factors that are influencing **the creation of new ‘markets’ for knowledge-based assets**, including intellectual property, information, data, goods and services; as well as identify what strategies different developers and users of knowledge are using to better identify, access, exploit, and create value from intellectual assets.

OECD (2008). “Open Innovation and Global Networks”. www.oecd.org/document/43/0,3343,en_2649_34273_41441387_1_1_1_1,00.html

This is a summary report of a conference hosted by the OECD and the Dutch **Ministry of Economic Affairs on “Globalisation and Open Innovation”** about the policy implications of the acceleration in the globalisation of research and **innovation and the shift towards “open innovation” strategies in companies**. The discussion centred around three main topics: *i)* the rise of open innovation and open business models in the context of globalisation; *ii)* the evidence for open innovation, drawing mainly on company case studies; and *iii)* the implications for science, technology and innovation policies.

OECD (2009). *Pharmacogenetics: Opportunities and Challenges for Health Innovation and Care*.

Pharmacogenetics offers new ways of understanding how drugs work and how this affects both their safety and efficacy in individuals. The potential opportunities for both drug development and clinical care are considerable. In drug development, pharmacogenetics can improve both the research and development process and the quality and efficacy of the products delivered. Pharmacogenetics can help identify those individuals most likely to benefit from a therapy, optimising treatment strategies for both common and complex disorders. Research in pharmacogenetics is proceeding rapidly. A number of scientific, regulatory, and economic challenges need to be overcome if pharmacogenetics is to be taken up more widely by healthcare systems. This report examines the challenges facing pharmacogenetics at different stages in the health innovation cycle and in the clinic. The report concludes that **governments have a role to play in creating an “enabling” environment for pharmacogenetics**. In particular it argues that building infrastructures for large-scale association studies is necessary to identify and validate the biomarkers that underpin the use of pharmacogenetics. This publication is based on commissioned consultant papers and an expert workshop.

OECD (forthcoming). *Emerging Research Models for Biomedicine and Health Innovation*.

This report takes a systemic view of innovation systems. It identifies four different approaches to improving the translational research process, all of which share the objective of facilitating the process of discovery research, development and delivery and of bringing biomedical innovations from invention to market faster. The report presents four examples initiatives (Innovative Medicine Initiative, Top Institute Pharma, MaRs, etc.) The report asks: (1) How do these initiatives differ from traditional approaches? (2) Are disparate initiatives and approaches above part of a movement toward the development of a/or several new research and business models that could revive drug discovery and health innovation? (3) What will make the emergence of such new business models possible? The report also identifies measure governments can take to influence health innovation processes and facilitate the development and diffusion of needed new products and services.

OECD (2006). *Innovation in Pharmaceutical Biotechnology: Comparing National Innovation Systems at the Sectoral Level*, www.oecd.org/dataoecd/29/59/36446831.pdf

This report is based on eight country studies (Belgium, Finland, France, Germany, the Netherlands, Norway, Japan, and Spain). It describes the structure and dynamics of national biopharmaceutical innovation systems in each country and their capacity to develop, produce and deliver biopharmaceutical products and services. It identifies the factors which influence different business models: *i)* the openness of innovation systems -- trade, alliances, foreign ownership, *ii)* demand side factors -- size and configuration of lead markets, type of health care systems, ethical consideration, *iii)* framework conditions – financial, regulatory, employment, *iv)* the structure and dynamics of the biopharmaceutical business ecosystem, *v)* the science base. The Report identifies a number of policy recommendations which are meant to improve the performance of the national systems for pharmaceutical biotechnology.

Workshop summaries

OECD (forthcoming), “Policy Issues in the Development and use of Biomarkers in Health”, see also expert analytical papers, www.oecd.org/document/48/0,3343,en_2649_34537_39405168_1_1_1_1,00.html

This document presents the outcomes of a workshop in October 2008 about how to improve the development and use of biomarkers in health care. Topics included: data and knowledge sharing for biomarker research; the creation of an evidence base and the clinical evaluation of biomarkers; the regulatory and policy framework for safe and efficient development of biomarkers; and business models for biomarker discovery, development and commercialization. Policy conclusions to governments focussed on how to create a common evidence base for evaluation, business strategies, regulatory and IP challenges, changes to medical practice.

OECD (2007). *Report of the High Level Forum on the Availability of Medicines for Neglected and Emerging Infectious Diseases*, www.oecd.org/document/45/0,3343,en_2649_34537_39163757_1_1_1_1,00.html

The Noordwijk Medicines Agenda is a summary of the main themes of the OECD High Level Forum. It identifies actions to stimulate R&D and radically accelerate the development and delivery of medicines for neglected and emerging infectious diseases. The NMA calls on governments to demonstrate political leadership and join a range of interested stakeholders to intensify collaborative efforts and promote coherent policies. It underlines the promise of new models for R&D that help overcome failures in the global health innovation system and draw on collaborative mechanisms for access and use of IPRs, as well open innovation approaches for R&D. It discusses collaborative drug development R&D networks and and new financing mechanisms for R&D.

The background papers to the HLF focus on the health needs of developing countries (Sachs and Sachs) and on the different mechanisms that are being proposed to address the dearth of investment, development and delivery of needed new medicines (Kremer and Williams). The later paper details push and pull mechanisms for incentivising R&D in for neglected and emerging infectious diseases such as support for public-private partnerships, global research funds, targeted R&D tax credits, advanced market commitments, patent extensions. If these mechanisms become widespread they will likely influence health industry business models.

Consultant reports

McKelvey, M. (2008), “Emerging Business Models and Institutional Drivers” *Bioeconomy 2030*. www.oecd.org/futures/bioeconomy/2030

This paper is about biotechnology business models in the human health sector. It describes existing and emerging health biotech business models and some key trends. It touches on the relationship between internal firm resources and external actors. It estimates future investment into private and public R&D. It identifies four external institutional drivers that will influence emerging business models: *i)* technological advances; *ii)* public research and the public-private interface; *iii)* policy and institutions; and *iv)* regulation; *v)* demand and consumers. The report describes what policies are needed for a better balance and harmony across both industry actors and fields of technologies and calls for

measures that take into account the sectoral or technological characteristics of biopharmaceutical innovation systems.

Dukes, M.N.G. (2008). “**Biotechnology** Regulation in the Health Sector,” *Bioeconomy 2030*. www.oecd.org/futures/bioeconomy/2030

This paper focuses on the study of regulatory systems which are key factors influencing the business models of the health industry. It analyses both existing and proposed regulation of health products emerging from biotechnology in a number of countries. It tries to determine to what extent existing regulatory approaches are adequate in the field of biotechnology, especially with regard to clinical challenges that will emerge as new technologies come on line (*e.g.* high throughput technologies, tissue engineering, regenerative medicine, cellular therapies, *etc.*) It suggests what sorts of policies may be needed to palliate to the likely regulatory gaps, including changes to intellectual properties protection norms, legal frameworks for data submission, licenses for medical products issued from biotech, and public dialogue on ethics.

Tait, J. Wield, D. Bruce, A. Chataway, J. (2008). “**Health Biotechnology to 2030**”. www.oecd.org/futures/bioeconomy/2030

This paper discusses the main factors to influence health care from now to 2030. It describes two scenarios, or possible futures, for health care development and delivery. The first scenario is the virtuous combination of circumstances and actors that are needed to stimulate innovation in health, generate profits and meet consumer demands. The second scenario shows what could happen if the different systems become increasingly dysfunctional with regard to advances in biotechnology and change too difficult to apprehend. The goal of this paper is to determine what behaviours stakeholders in health innovation systems, including governments, should adopt in order more nimbly adapt to the coming changes in medical research and healthcare. Some drivers of change are discussed including: technology and research, industry innovation systems, governments policies and regulation, health care delivery systems, public and stakeholders attitudes, global economics, demography and human resources, climate change, security, development in animal health and their influence on human health care systems.

3. The governance of new research and health infrastructures

1. Explanation of the policy issue

Biological and health information research infrastructures are important resources that underpin all biological science. They provide information and source material for scientific investigation, and underpin many of the discoveries on which biotechnology is founded. The WPB has extensive experience in the development and promotion of governance infrastructures needed for fostering research in the life sciences and especially for removing the disincentives to the sharing and use of data, knowledge and technologies. OECD work supports Countries in their efforts to establish and maintain research infrastructures; to ensure quality, security, accessibility of the data and materials contained therein; to build public trust and understanding of these infrastructures; and finally to ensure they adequately address policy concerns. Notably the OECD has established Best Practice Guidelines on Biological Resource Centres and is in the process of negotiating a Recommendation on Human Biobanks and Genetic Research Databases.

This module identifies lessons learned about how to set up new organizational arrangements, infrastructures, and governance systems that facilitate knowledge creation, access and use. It identifies challenges such as the difficulty of collective action, and the need for interoperability of data and the promotion of innovation at the interface different disciplines.

2. Related work done by the OECD (In this module the publications are grouped by theme)

Instruments

OECD (2007). *OECD Best Practice Guidelines for Biological Resource Centres*. DSTI/BIO. www.oecd.org/dataoecd/7/13/38777417.pdf. See also: *Guidance for the Operation of Biological Resource Centres: Part 1 - General Requirements for All BRCs* and *Guidance for the Operation of Biological Research Centres: Certification and Quality Criteria for BRCs*, www.oecd.org/document/36/0,3343,en_2649_34537_38777060_1_1_1_1,00.html

The Guidelines and supporting documents address the establishment, management and governance of BRCs. BRCs are considered to be one of the key elements of a sustainable international scientific infrastructure necessary to underpin the delivery of benefits of biotechnology, whether within the health, industrial or other sectors. BRCs are repositories and service providers of the living cells, organisms, genomes, and information relating to heredity and the functions of biological systems. Such biological resources provide the source materials for scientific investigations. Ensuring the proper maintenance and supply of biological resources is essential. Four sets of best practice guidelines are described here, dealing with *i)* general quality aspects, *ii)* biosecurity-related issues, *iii)* specific guidelines for BRCs holding and supplying microorganisms, and; *iv)* specific guidelines for BRCs holding human-derived materials. The best practice guideline for BRCs target quality assurance issues that should be addressed to ensure the supply of high quality materials. In order to improve BRC quality assurance, the CSTP agreed the BRC Best Practice Guidelines in 2007.

Reports for Committees

OECD (Forthcoming). “Policy Issues in the Development and use of Biomarkers in Health”, see also expert analytical papers:

www.oecd.org/document/48/0,3343,en_2649_34537_39405168_1_1_1_1,00.html

This document presents the outcomes of a workshop in October 2008 about how to improve the development and use of biomarkers in health care. Topics included: data and knowledge sharing for biomarker research; the creation of an evidence base and the clinical evaluation of biomarkers; the regulatory and policy framework for safe and efficient development of biomarkers; and business models for biomarker discovery, development and commercialization. Policy conclusions to governments focussed on how to create a common evidence base for evaluation, business strategies, regulatory challenges, changes to medical practice.

OECD (2008). “Draft Recommendation on Human Biobanks and Genetic Research Databases.” www.oecd.org/sti/biotechnology/hbgrd

The WPB is developing Council Guidelines on human biobanks and genetic research databases (HBGRDs). The establishment, harmonisation and broad use of research involving data and samples from human biobanks and genetic research databases analysed in conjunction with personal or health data is important for research and will be increasingly important for not only for healthcare but also for drug discovery. HBGRDs are structured resources that can be used for the purpose of genetic research and which include: a) human biological materials and/or information generated from the analysis of the same; and b) extensive associated information. The draft Guidelines are intended to assist governments in the development of policies applicable to HBGRDs and to provide guidance for private and public sector HBGRDs. The Guidelines, developed by a group of member country experts, cover the establishment, governance, management, operation, access, use and discontinuation of HBGRDs. They also cover governance structure and oversight mechanisms; privacy and confidentiality; terms of participation; access; funding mechanisms; benefit sharing, intellectual property and commercialisation; protection and security of human biological materials and data; the qualifications, education and training of staff; disposal of materials and data and the discontinuation of a HBGRD.

OECD (2007-2008). *Creation and Governance of Human Genetic Research Databases*. STI/BIO, www.oecd.org/dataoecd/61/29/37647338.pdf and www.oecd.org/dataoecd/61/29/37647338.pdf

This book summarises the proceedings of a 2005 conference on human genetic research databases. Large-scale study of populations may contribute **significantly to science’s understanding of the complex multi-factorial basis of disease** and to improvements in prevention, detection, diagnosis, treatment and cure. The book examines what these databases are and provides a number of examples. It looks at how they have been established, governed, and funded. And it looks at how they are managed and commercialised, exploring what the policy implications are for governments. The book identifies the international policy challenges associated with the establishment, management and governance of human genetic research databases.

OECD (2009), *Pharmacogenetics: Opportunities and Challenges for Health Innovation and Care*.

Pharmacogenetics offers new ways of understanding how drugs work and how this affects both their safety and efficacy in individuals. The potential opportunities for both drug development and clinical care are considerable. In drug development, pharmacogenetics can improve both the research and development process and the quality and efficacy of the products delivered. Pharmacogenetics can help identify those individuals most likely to benefit from a therapy, optimising treatment strategies for both common and complex disorders. Research in pharmacogenetics is proceeding rapidly. A number of scientific, regulatory, and economic challenges need to be overcome if pharmacogenetics is to be taken up more widely by healthcare systems. This report examines the challenges facing pharmacogenetics at different stages in the health innovation cycle and in the clinic. The report concludes that governments **have a role to play in creating an “enabling” environment** for pharmacogenetics. In particular it argues that building infrastructures for large-scale association studies is necessary to identify and validate the biomarkers that underpin the use of pharmacogenetics. This publication is based on commissioned consultant papers and an expert workshop.

OECD (2007). “Intellectual Property Rights in International R&D Collaboration: Background and issues. www.oecd.org/dataoecd/60/61/34305040.pdf

This report describes the potential barriers to IP in international collaboration in R&D and innovation. It tries to provide solutions for improving intellectual property and knowledge sharing, such as by placing IP in the public domain, developing patent pools, developing patent portfolio, developing copyright licensing models, *etc.*

4. The demand and take up of health innovations

1. Explanation of the policy issue

Governments are searching for opportunities to improve health care and pave the way to high value-added production and services from health technologies. Achieving this requires an adaptation of policies and institutions, including: the regulatory system, the way new technologies are priced/valued, medical practice and health systems, and the alignment of research and health policies.

Work in this area has focused on the following set of questions:

- Is there a match between health innovations and public health needs? If not, how can the situation be improved?
- What are the framework conditions or factors that influence incentives for the development of health technologies? And can these be modified to encourage the development of health innovation for the public good?
- How is the demand for health innovation changing and influencing incentives firms face?
- Can one better value health innovations so as to guide investment by innovators and purchasers? What are emerging approaches and methodologies?
- Do innovations improve health outcomes and the quality of health care?
- How does innovation impact health expenditures?
- What are the barriers in the health system receiving environment to the uptake and diffusion of more efficient and effective health technologies? And how can these be addressed to improve the diffusion of efficient and effective health technologies?
- How are new technologies influencing the operation of health systems? What are their impacts on human capital and other resources?
- What are the opportunities and challenges for assessing the effectiveness of innovative biomedical products/processes?

2. Related work done in the OECD

Instruments

OECD (2007). *Guidelines on the Quality Assurance for Molecular Genetic Testing*. www.oecd.org/sti/biotechnology/licensing

The Guidelines address genetic testing for variations in germ line DNA sequences or products arising directly from changes in heritable genomic sequences that predict effects on the health, or influence the health management, of an individual. They focus on molecular genetic testing for the diagnosis of a particular disease or condition and predictive genetic testing often carried out before any clinical signs of the disease or condition appear. They are also relevant to tests for heritable DNA variants that predict the response profile

of an individual to a drug or course of therapy and that affect susceptibility to disease, patient prognosis, counselling, treatment and family planning. They do not address testing carried out only for research purposes.

Guidelines focus on the provision of clinical genetic services, in particular on: quality assurance systems for the tests offered, result reporting requirements, proficiency testing of laboratories performing tests, and the education and training standards for laboratory personnel.

Reports to Committee

OECD (2009). *Pharmacogenetics: Opportunities and Challenges for Health Innovation and Care*.

Pharmacogenetics offers new ways of understanding how drugs work and how this affects both their safety and efficacy in individuals. The potential opportunities for both drug development and clinical care are considerable. In drug development, pharmacogenetics can improve both the research and development process and the quality and efficacy of the products delivered. Pharmacogenetics can help identify those individuals most likely to benefit from a therapy, optimising treatment strategies for both common and complex disorders. Research in pharmacogenetics is proceeding rapidly. A number of scientific, regulatory, and economic challenges need to be overcome if pharmacogenetics is to be taken up more widely by healthcare systems. This report examines the challenges facing pharmacogenetics at different stages in the health innovation cycle and in the clinic. The report concludes that governments have a role to **play in creating an “enabling” environment for pharmacogenetics**. In particular it argues that building infrastructures for large-scale association studies is necessary to identify and validate the biomarkers that underpin the use of pharmacogenetics. This publication is based on commissioned consultant papers and an expert workshop.

OECD (2008). “Chapter 6. The Impact of Pharmaceutical Pricing Policies on Pharmaceutical Innovation”. *Pharmaceutical Pricing Policies in a Global Market*. www.oecd.org/health/pharmaceutical

Pharmaceutical pricing policies are designed with national objectives in mind, but are the transnational implications always taken into account? This report assesses how pharmaceutical pricing and reimbursement policies have contributed to the achievement of certain health policy objectives. It examines the national and transnational effects of these policies, in particular, their implications for the availability of medicines in other countries, the prices of these medicines, and innovation in the pharmaceutical sector. This publication presents an analysis of comparative price levels, making use of a unique dataset to construct the most comprehensive pan-OECD pharmaceutical price index to date. It also draws upon original case studies of pharmaceutical pricing and reimbursement policies in six OECD countries to provide specific examples of the impacts of policies on health system performance.

Chapter 6 focuses on pharmaceutical R&D investment and the ways in which pricing and reimbursement practices contribute to trends in innovation.

OECD (2008). “Health ICT Use and Adoption: Case studies.” www.oecd.org/els

This is an ongoing project which will end in 2009. It aims to provide OECD governments with advice concerning a range of policy options, conditions and practices that may help achieve efficiency improvements in the health sector

through more widespread adoption of ICTs. There are two work streams to identify and analyse: *i)* the most common indicators used across OECD countries for benchmarking the use and adoption of ICTs; and *ii)* drivers and incentives for ICT adoption within the health sector. The study includes a synthesis of information in the published literature and in national studies, and a more focused set of in-depth case studies in selected countries.

OECD (Forthcoming). “Uptake and Diffusion of Health Related Biotechnologies”.

This report is based on a paper by a consultant. It discusses five health-related biotechnologies and identifies the range of incentives and barriers that affect their uptake and into the health care sector. The selected technologies differ in their stage of development, including innovations that are relatively mature to those that are still very new, but all have products on the market. The five case study technologies are: monoclonal antibodies as diagnostics and as therapeutics; molecular genetic testing; DNA micro-arrays; and the convergence of bio- and nano-technologies. For each technology, the report identifies the potential clinical utility and the factors identified through a literature survey that appears to be either stimulating or inhibiting its diffusion into the market and eventually the health care system.

OECD (2007). “Promoting Innovation, Promoting Health”, www.oecd.org/els

This document is based on expert interviews by a consultant in eight OECD countries about how each country is approaching the challenge of delivering greater convergence between healthcare priorities and health innovation. The purpose was to explore how to better meet the dual policy objectives of investing in and encouraging innovation while maintaining the affordability, quality and sustainability of healthcare systems. The study, which includes seven country chapters, discusses how various governments, government departments and other actors define innovation in health care; it documents key government programs to promote innovations which meet public health needs; it identifies initiatives that coordinate health care and innovation policies and discusses motivations behind such initiatives.

OECD (2005). *Health Technologies and Decision Making*.

www.oecd.org/document/55/0,3343,en_2649_34537_35589431_1_1_1_1,00.html

This publication focuses on the policy challenges around how to encourage the uptake of the most efficient and effective health-care technologies. The study is based on an eight country survey of decision-making processes for 5 technologies. Comparative information was collected on decision-making processes for five case study technologies: positron emission tomography, hepatitis C genotyping and viral load testing, telemedicine, prostate cancer screening, and technologies for dealing with stroke patients. The focus of the study is on how evidence, primarily in the form of health technology assessment (HTA), was produced and subsequently used in decision making. In terms of policy, it considered options for dealing with uncertainty in the evidence base, consideration of the transferability of evaluations between different situations, and analysis of how biomedicines challenge decision makers.

Workshops

BIAC, (2007). “A Turning Point for ICTS in Health Care? Key Points from the BIAC–LMP Consultation.”

www.oecd.org/document/41/0,3343,en_2649_34489_37981993_1_1_1_1,00.html

The Business and Industry Advisory Committee (BIAC) convened an expert group in November 2007, which included leading representatives for doctors, patients, health programs, the employers, the pharmaceutical industry and ICT system providers. The group was asked to discuss ICTs in health care in November 2007, and specifically addressed: What is the status of ICT in health care today? How is the market evolving? What challenges lie ahead? And, which new business models are emerging? The second paper presents a summary of the conclusions from this meeting.

Figure 1: The Health Care Value Chain and areas for policy planning

