Working Party on Biotechnology

Progress Report - Healthy Ageing and the Governance of Biomedicine and Health Innovation for Dementia and Alzheimer’s Disease

5 - 6 November 2013

The paper presents progress in the work of the Working Party on Biotechnology (WPB) on healthy ageing and the governance of biomedicine and health innovation for dementia and Alzheimer’s Disease.

Delegates should refer to document DSTI/STP/BIO(2013)9 for the project proposal discussed and agreed at the last WPB meeting in June 2013. The project will support the completion of Outputs Result 2.1 - Opportunities and options for public-private partnerships and 3.3 - Science, technology and innovation (STI) for healthy ageing, of the 2013-2014 Programme of Work and Budget.

Delegates to the WPB are invited to:

• Note progress in the project,
• Indicate their willingness to join the project Steering Group and participate in one or more of the project’s activities.

For further information, please contact:
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NOTE BY THE SECRETARIAT

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HEALTHY AGEING AND THE GOVERNANCE OF BIOMEDICINE AND HEALTH INNOVATION FOR DEMENTIA AND ALZHEIMER’S DISEASE: PROJECT UPDATE

Summary of progress

1. At its last meeting in June 2013, the Working Party on Biotechnology (WPB) agreed to proceed with the project on governance of biomedicine and health innovation for dementia and Alzheimer’s Disease as presented in document DSTI/STP/BIO(2013)9. The project is composed of different modules, as follows:

- Questionnaire on government perspectives and approaches for biomedical innovation in dementia and Alzheimer’s Disease;
- Case studies on public-private partnerships for facilitating the translation of science and technology for dementia and Alzheimer’s Disease;
- Socio-economic scenarios for new diagnostics and therapies for dementia and Alzheimer’s Disease;
- Workshops on the governance of biomedicine and health technologies for dementia and Alzheimer’s Disease; and
- Good practices in international co-operation for dementia and Alzheimer’s Disease.

2. The proposed activities to be undertaken in the different modules were refined through two workshops organised under the auspices of the WPB and in which WPB delegates participated, namely:

- An event on “Integrating Omics and Policy for Grand Challenges: Healthy Ageing” in Singapore on 13 April 2013, organised jointly between the WPB and the Human Genome Organisation (HUGO) (see DSTI/STP/BIO(2013)14); and
- A joint expert consultation between the Information, Communication and Consumer Policy Committee (ICCP) and WPB, organised in Oxford on 20-21 June 2013. This meeting was supported by the Global Coalition on Ageing and by Oxford University (Harris College) and provided an opportunity to discuss issues both relevant to ICCP and to the WPB and to identify complementarities between the projects of the two groups.

3. These two events reinforced the importance of addressing three particular topics within the project: i) mechanisms to support responsible innovation, in particular for the development of emerging fields of science and technology (e.g. omics technologies, nanotechnology, regenerative medicine, synthetic biology), that could lead to breakthrough innovation in research for dementia, ii) current regulatory frameworks, on a global scale, which could impede new technology-driven solutions reaching the patient, notably the use of new technological solutions in the context of clinical research; and iii) new cost-sharing and risk-sharing mechanisms for public-private collaborations and incentives targeted at (re-)engaging industry in the business of dementia research.

4. As a result, working with the Steering Group (composed of Australia, Canada, Finland, Germany, Mexico, the Netherlands, Spain, Sweden, Switzerland, the United Kingdom, the United States and BIAC), the Secretariat is ensuring that those three points remain central to the development of the different modules of the project. However, the completion of all aspects of the project will be dependent on delegations’ interest and willingness to contribute to the project through both in-kind and voluntary contributions.
5. Delegates should note that past and on-going activities of the WPB on the governance of biomedicine and health innovation for healthy ageing and dementia will contribute to preparation of a G8 summit on dementia that will be held in December of this year. As a possible input to this, the Secretariat is preparing a Synthesis Report bringing together the main conclusions from the work of the WPB in this area, to be completed and sent to delegates for comment in 2013.

Project achievements so far: two workshops on policy aspects of biomedical and health innovation for healthy ageing and dementia

6. Two workshops have been organised in the past few months: the OECD/HUGO workshop in Singapore in April 2013 and a joint ICCP/WPB Oxford workshop in June 2013. These two events have been used to refine the proposal for WPB work on Alzheimer’s disease and dementia and identify complementarities between the work undertaken by the ICCP and by the WPB.

OECD/HUGO workshop on “Integrating Omics and Policy for Grand Challenges: Healthy Ageing”, Singapore April 2013

7. The joint OECD/HUGO event was a 2.5 hour session and took place in the context of the Joint Conference of the Human Genome Meeting 2013 and the 21st International Congress of Genetics (see www.hgm2013-icg.org/scientific_programme.html). The objective of the workshop was to:

“discuss the latest policy developments, challenges and obstacles in omics technologies for healthy ageing, and most specifically for dementia and Alzheimer’s disease... focussing on the latest advances in omics technologies for healthy ageing and the policies and practices needed to facilitate their responsible development and integration into medical research and into innovation and health policy.”

8. The workshop was organised around expert talks and discussions on issues such as: the underlying molecular mechanisms and genetics of healthy ageing, the role of new omics tools (such as Genome Wide Association Studies (GWAS)) and their application in epidemiologic studies of ageing, in particular of brain ageing (e.g. twin studies). The workshop also addressed governance and policy issues linked to new developments in omics technologies for ageing, focusing on the regulatory challenges and the mechanisms to support effective translational research through, for example, new collaborative models of investment and risk-sharing initiatives. The workshop also examined the underlying principles of challenge driven innovation policy for healthy ageing, notably putting the emphasis on the central role of patient empowerment and user-driven innovation. The workshop concluded that important governance challenges and opportunities remain within the structures supporting research into age-related diseases and dementia, in particular:

- **Reinforcing strategic collaboration**: the strengthening of stakeholder collaboration is needed to overcome the challenges associated with the development and validation of new interventions for age-related conditions. Reinforcement of collaborations can include creating innovative partnerships between governments and public entities and the private sector in order to bring together the resources needed for efficient research and for risk sharing among the different actors. The need to support collaboration via new models of financing and investment for research, development and the implementation of innovation was also discussed. For example, the workshop highlighted the importance of developing new schemes of public-private research funding and pre-competitive consortia for shared risk management. As part of these collaborative mechanisms, issues surrounding data access and sharing were highlighted. Patients are becoming central actors within these collaborations.

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1. See https://www.gov.uk/government/news/uk-to-use-g8-to-target-global-effort-on-dementia
• **Supporting innovation**: addressing the biological complexity of certain age-related diseases will require not only an exceptional level of collaboration but also an environment that stimulates innovation, in particular in emerging fields of science and technology, from which breakthrough innovations could come. Innovation can be stimulated through supportive policies but also in the way that regulatory pathways are organised.

• **Modernising regulatory science**: many of the innovative solutions to address the challenges presented by the complexity of age-related conditions will be technology-driven, and will be dependent on rapid advances in emerging fields such as omics technologies, but also on regenerative medicine, nanosciences and synthetic biology. The workshop specifically pointed out the importance of adapting regulatory pathways in order that regulators are better able to navigate through an environment of uncertainty vis-à-vis emerging technology-driven solutions for ageing, in particular that regulation can support the responsible transfer of biomedical innovation to the point of care. This includes, for example, an early and continuous dialogue between the regulators and the innovators engaged in the development of technology-driven solutions for ageing. This should be supported by regulatory systems that are dynamic and future-oriented and have a strong scientific basis in order to facilitate innovation in emerging fields to take place.

9. The full workshop report is available at DSTI/STP/BIO(2013)14. The report is now with the WPB for comments, prior to being sent for declassification by written procedure to the Committee for Scientific and Technological Policy (CSTP).

**Expert Consultation on Unlocking Global Collaboration to Accelerate Innovation for Alzheimer’s Disease and Dementia, Oxford, 20-21 June 2013**

10. The OECD, together with the Global Coalition on Ageing, organised an Expert Consultation on Unlocking Global Collaboration to Accelerate Innovation for Alzheimer’s Disease and Dementia. The consultation was hosted by Harris Manchester College at the University of Oxford. The objective of the consultation was to begin to identify a framework for policy making and stakeholder engagement and it was organised around expert talks in the areas of (1) biomedicine and health innovation for Alzheimer’s disease and dementia, and (2) “big data” and data sharing challenges.

11. In Annex 1, delegates will find a summary of the key messages from the consultation with regard to the biomedicine and health innovation sessions. These sessions highlighted:

• **The need to re-examine the conceptual models of the disease and the regulatory pathway through translational research.** Alzheimer’s Disease is a complex human disease that has proved to be very difficult to replicate using animal models. Governance mechanisms are needed to facilitate the development of novel approaches in the drug development process based, for example, on emerging fields offering innovative technology-driven solutions, such as the development of in silico modelling (e.g. induced-pluripotent stem cells as predictive model for neurodegenerative diseases).

• **The importance of strategic collaboration between the public and private sectors.** The consultation highlighted the importance of developing new schemes for public-private research funding and pre-competitive consortia for shared risk management between public and private actors as an incentive to (re)-engage industry in the uncertain business of Alzheimer’s research.

12. A summary of the sessions linked to the data challenges will shortly be available from the ICCP and the WPB will be informed about that document when it is finalised.
Additional progress on the project to date

Questionnaire on government perspectives and approaches for biomedical innovation in dementia and Alzheimer’s Disease

13. A draft questionnaire has been developed for this module and is now with Steering Group members for comments (see Annex 2). The questionnaire for WPB delegations aims to develop an inventory of government approaches used in supporting the development of biomedical and health innovation for Alzheimer’s disease and dementia. The questions have been informed by the conclusions from the two workshops described above. It includes questions on policies to support research in Alzheimer’s and dementia, on the specific tools used to support emerging fields of science and technology, and on regulatory science programmes.

14. The questionnaire is short, with only nine questions, in order to minimise the burden on delegations and maximise the number of responses received, as well as the quality of those responses. The success of this module will heavily rely on the active participation of a sufficient number of delegations.

15. After review by the project Steering Group, the questionnaire will be circulated to WPB members with a deadline for completion of end January 2014. The responses will then be analysed between February and March 2014 and included in the final project report.

Case studies on public-private partnerships to facilitate the translation of science and technology for AD

16. This module is focusing on models for public-private partnerships (PPPs) in health biotechnology, most specifically on PPPs to facilitate the translation of science and technology for Alzheimer’s Disease and dementia. The work will feed into on-going work of the OECD Working Party on Innovation and Technology Policy (TIP) on Strategic Public-Private Partnerships for Key Enabling Technologies.

17. The information for the case studies on public-private partnerships for biomedicine and health technologies for Alzheimer’s disease and dementia will be gathered using a template prepared by TIP (see Annex 3). The case studies will have three main goals: 1) identifying the main initiatives that are being developed in countries to foster translational research for Alzheimer’s Disease; 2) identifying the factors that have affected progress through these initiatives; and 3) identifying good practice and drawing lessons for future use. The case study template will soon be sent to the WPB with guidance regarding its completion. WPB delegates will be able either to advise on interesting partnerships that are being developed in their country (and the Secretariat will conduct interviews with relevant people in these partnerships) or WPB delegates will be invited to fill in the case study template themselves.

Other modules of the project

18. As there have not been any voluntary contributions or in-kind contributions from delegations and no other funding has yet been found for the work, the module on socio-economic scenarios for new diagnostics and therapies for Alzheimer’s and dementia will not be pursued at this time.

19. Discussions are on-going on the possibility of holding a funded workshop on the Governance of Biomedicine and Health Innovation for Dementia and Alzheimer’s Disease that will focus specifically on the challenges of modernising regulatory science to meet the challenges of Alzheimer’s Disease and dementia. The main objective of this workshop would be to propose good practices to strengthen regulatory science and support effective cooperation at a global level for the governance of biomedical innovation in Alzheimer’s and dementia.
ANNEX 1: EXPERT CONSULTATION ON UNLOCKING GLOBAL COLLABORATION TO ACCELERATE INNOVATION FOR ALZHEIMER’S DISEASE AND DEMENTIA:

BIOMEDICINE AND HEALTH INNOVATION: MAIN POINTS FROM THE CONSULTATION

Introduction to the Expert Consultation

20. The increase in the human life span is a testament to the economic, social and medical progress made in OECD countries over the course of the last century. However, an ageing population brings new social and economic challenges in terms of the increased manifestation of specific conditions primarily seen in the elderly. Age-related diseases, in particular those affecting the brain, represent a particular and very significant challenge to the health of the population, national prosperity, productivity and economic growth.

21. Dementia is today one of the most frequently seen degenerative conditions in the older part of the population and Alzheimer’s Disease is the most common form of dementia. Alzheimer’s is a complex multifactorial disease, which is being intensively investigated, but for which there are still very few effective treatments available. The growth in the prevalence of the disease is resulting in significant and increasing negative economic and social consequences. A number of complementary actions will be needed to help to tackle challenges associated with Alzheimer’s disease. For example, international measures will be necessary to accelerate innovation in areas of critical need, leveraging both advances in biomedicine and the sharing of data at a global scale.

22. The Organisation for Economic Co-operation and Development (OECD), together with the Global Coalition on Ageing, organised in June 2013 an Expert Consultation on Unlocking Global Collaboration to Accelerate Innovation for Alzheimer’s Disease and Dementia. The consultation was hosted by Harris Manchester College at the University of Oxford. The objective of the consultation was to begin to identify ways in which policy making and stakeholder engagement could be enhanced for AD. To this end, the consultation:

- Provided a space for country experts, policy makers and other stakeholders to share views on the main scientific, technological and policy challenges raised by Alzheimer’s Disease;
- Created an opportunity for multidisciplinary exchanges;
- Captured views of participants on how to move forward in an OECD context.

23. The consultation was organised around expert talks in the areas of i) biomedicine and health innovation, and ii) “big data” and data sharing challenges. This report summarises the key messages from the biomedicine and health innovation sessions of the consultation. The agenda for the consultative meeting can be found in the Appendix.

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The summary does not necessarily represent the views of the Global Coalition on Ageing, Harris Manchester College or the OECD or a consensus amongst participants.
Main conclusions from the Expert Consultation

24. Participants in the consultation highlighted that a number of complementary governance actions will be needed to tackle challenges associated with the scientific complexity of Alzheimer’s and dementia, making medical advances difficult and presenting obstacles in moving innovation from the lab bench to the bedside. A particular emphasis was made on:

- **The need to re-examine the conceptual models of the disease and the regulatory pathway through translational research.** Alzheimer’s is a complex human disease that proved to be very difficult to reproduce in animal models. Governance mechanisms should facilitate the development of other models in the drug development process based, for example, on emerging fields offering innovative technology-driven solutions, such as the development of *in silico* modelling (e.g. induced-pluripotent stem cells as predictive model for neurodegenerative diseases).

- **The importance of strategic collaboration between the public and private sectors.** The consultation highlighted the importance of developing new schemes for public-private research funding and pre-competitive consortia for shared risk management between public and private actors as an incentive to (re)-engage industry in the risky business of Alzheimer’s research. At the research, development and early commercialisation stages, more innovative approaches to sharing risk and knowledge should be developed (for example, large consortia comprising companies, SMES’s, CROs, public laboratories and academic institutions). Such collaboration would allow for risk sharing between public and private entities, but also risk sharing between companies themselves.

*The complex biology of the disease*

25. The consultation highlighted the complexity of Alzheimer's disease as being a multifactorial and multigenic neurodegenerative disease. While significant progress has been made in the past decades to characterise Alzheimer's and understand its biology, the diagnosis of the disease remains difficult and no solutions have been found to efficiently diminish its symptoms, slow its progression or cure the disease. So far the “anti-Alzheimer’s” drugs do not offer a sufficient benefit relative to the risk they raise. In addition, when the diagnosis of the disease is made, the patient’s condition is often already at an advanced and irreversible stage.

26. It was highlighted that investments in basic and applied research need to continue to enable the identification and clarification of the biological mechanisms associated with the disease and in particular the pathways that lead to the failure in the brain system. Research is, for example, concentrating on finding new biomarkers (e.g. molecular and imaging biomarkers) which could allow for a timely diagnosis of the disease at a stage at which a clinical intervention would be the most beneficial. The discovery of new biomarkers of the disease is also critical to the discovery of new therapeutic targets. In the past decades, the complexity of the disease and the difficulties in finding safely “reachable” therapeutic targets have led to a very high rate of drug failure in clinical trials and something of a disengagement of industry from Alzheimer’s research.

27. The consultation included much discussion about the complex interaction between genetic and environmental factors (e.g. the local environment, poor nutrition, excessive stress) in the onset and development of Alzheimer’s. Both types of factors are involved in the ageing process and in the pathways that lead to the development of age-related conditions. It was put forward at the consultation that the mechanisms of the disease involve genetics, gene expression, epigenetics and a multitude of environmental factors.
Tools and techniques: integrating emerging technologies for breakthrough innovation

28. The consultation strongly pointed out that the validity of scientific tools, models and methods currently used in translational research for Alzheimer’s is being questioned and may need to be revisited. In particular, animal models of the disease are under scrutiny. Animal models have played a critical role in recent finding about the impact of genetics on ageing and the development of Alzheimer’s disease. However, questions remains about the accuracy of the models used and the extent to which they sufficiently relate to lesions similar to those in the human form of the disease. Alzheimer’s has been very difficult to reproduce in animal models so far. This leads to doubts around their absolute value in giving an appropriate picture of the mechanisms of the disease but also doubts about the likely effectiveness of candidate drugs ahead of the clinical research stage.

29. In recent years, new research paths have developed to include emerging fields such as nanosciences, synthetic biology, regenerative medicine and, in particular, genomics. The spectacular advances in genomics in the last few years have resulted in the discovery of a number of genes that can be associated with the disease. It should be noted that a very large number of alleles are likely to be involved in the development of Alzheimer’s and in the ageing process more generally and studies need to be conducted on a very large scale. Genome Wide Association Studies (GWAS) have greatly contributed to the discovery of some of the underlying pathologic mechanisms associated with Alzheimer’s. The genes that have been discovered are essentially linked to functions of repair but they also respond to both damage and metabolic regulation (e.g. the role of the amyloid precursor protein, and its impact on the regulation of brain cholesterol turnover). The challenge now lies in the difficulty of assigning pathogenicity to each genome variant and of identifying its role in the onset and progression of the disease.

30. The consultation highlighted that all stages of research are considered to be important - from basic research to more applied research - covering the great need both to understand the molecular mechanisms of the disease and to rapidly find clinically-applicable ways of managing the disease. A particular emphasis was placed on the importance of blue skies-research, which has the potential to open up new avenues leading to breakthrough innovations in the field of neurodegenerative diseases. Many such innovations are likely to be technology-driven and dependent on rapid advances in emerging fields of science and technology. The consultation also pointed out that, in this context, the modernisation of regulatory mechanisms may be necessary for regulators to be better able to deal with the uncertainties around these emerging technology-driven solutions.

Global research and collaboration in the fight against dementia and Alzheimer’s Disease

31. The consultation emphasised the need to better use existing resources at local, regional, national and international levels by aligning the needs of the research community working on Alzheimer’s Disease and national policy strategies and, for example, avoiding fragmentation of research. This need is being addressed in part through financing of research, the development of a number of programmes and initiatives at the national and global level, and associated policies. Examples of investment in and coordination of research were presented during the meeting, in particular some programmes set up through the European Commission, with the specific example of the country-led Joint Programming Initiative on Neurodegenerative Diseases (JPND, see below).

32. The presentations highlighted that understanding the human brain and its associated diseases is one great scientific challenge being addressed at European Union level through support for brain research under the Seventh Framework Programme (FP7). Research on neurodegenerative diseases, including Alzheimer’s Disease, is a priority in this programme. A number of initiatives have been developed to foster basic and applied research for the disease, such as MEMOLOAD (which aims to identify the molecular level mechanisms by which accumulation of amyloid beta in the brain results in impaired synaptic
plasticity and memory loss) and PHARMA-COG (working on the prediction of cognitive properties of new drug candidates for neurodegenerative diseases in early clinical development). The US initiative on the brain mapping was also mentioned.

33. The European Joint Programming Initiatives are structured and strategic processes whereby Member States agree, on a voluntary basis, on the definition, development and implementation of common strategic research agendas, based on a common vision to address major societal challenges. The Joint Programming Initiative on Neurodegenerative Diseases (JPND) aims to make national research more strategic, focused and effective, and to enable it to have a higher impact. The JPND has facilitated an alignment of research strategies and national activities and is also partnering with industry (for example, through their membership of the scientific advisory board of the JPND). A specific JPND Industry Action Group has also been established to develop new public-private partnerships.

34. The meeting brought to the fore the importance of fostering innovative partnerships between governments and public entities and the private sector in order to bring together the resources needed for efficient and effective research. For example, the consultation highlighted that new collaborations are being engaged in that aim for all partners to share equally both the “risks” and “benefits” of the entire enterprise.

**Conclusion**

35. The findings of the *Expert Consultation on Unlocking Global Collaboration to Accelerate Innovation for Alzheimer’s Disease and Dementia* are informing the project of the WPB on healthy ageing and the governance of biomedicine and health innovation for dementia and Alzheimer’s Disease. That project includes modules on the following issues which will build on the findings of the Oxford meeting:

- Government perspectives and approaches for biomedical innovation in dementia and Alzheimer’s Disease; and
- Public-private partnerships for facilitating the translation of science and technology for dementia and Alzheimer’s Disease,
APPENDIX: EXPERT CONSULTATION AGENDA

Expert Consultation

20-21 June 2013

The Harris Manchester College, Oxford, United Kingdom

The aim of this consultation is to stimulate discussion and the highest possible level of expert engagement in setting out an agenda for OECD action to accelerate innovation for Alzheimer’s disease and dementia. Specifically, the objectives are to:

- Provide a space for country experts, policy makers and other stakeholders to share views on the main scientific, technological and policy challenges Alzheimer’s and dementia raise
- Create an opportunity for multidisciplinary exchange
- Capture views on how to move forward and develop concrete ideas for OECD action

The consultation will be held on 20-21 June 2013 at The Harris Manchester College (HMC), Oxford University in collaboration with the Global Coalition on Aging and Oxford’s HMC. It will see the participation of policy makers, academic and private sector researchers, clinicians, health economists, NGOs and technical experts (e.g. from the bio-nano-technology and IT sectors) who lead efforts on Alzheimer’s and dementia research. For further information, please contact elettra.ronchi@oecd.org.

Background

The OECD workshop on Anticipating the Special Needs of the 21st Century Silver/Ageing Economy: From Smart Technologies to Services Innovation, hosted by WASEDA University on September 12-14, 2012 with the support of the Japanese government (MIC and METI) and involving APEC countries concluded that innovation will be needed to meet the challenges and opportunities of global demographic change and mitigate the health, social and economic impacts of ageing. In particular, participants agreed that international action is required to accelerate innovation in areas of critical need such as Alzheimer’s and dementia by leveraging advances in biomedicine and the sharing of data at a global scale. Experts called for the development of an urgent and global Alzheimer’s strategy that includes critical re-evaluation of well-accepted traditional concepts of healthcare services, expanded research investments, coordinated strategies to address the big data challenges, and recognition of the massive implications of Alzheimer’s for economic growth.
### Thursday, 20 June 2013

**Alzheimer’s and dementia research today**

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<th>Time</th>
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<tr>
<td>13:30-14:00</td>
<td>Registration and coffee</td>
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<tr>
<td>14:00-14:15</td>
<td><strong>Opening and Introductory Remarks</strong></td>
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<td>• <strong>Ralph Waller</strong> - Principal of Harris Manchester College; Pro-Vice-Chancellor elect of the University of Oxford</td>
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<td>• <strong>Elettra Ronchi and Jacqueline Allan</strong> (OECD)</td>
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<tr>
<td>14:15-14:45</td>
<td><strong>Why accelerating innovation for Alzheimer’s and dementia is a global priority</strong></td>
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<td>• <strong>Michael Hodin</strong> - Executive Director, Global Coalition on Ageing, United States</td>
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<td>Alzheimer's Disease is a global social and economic challenge. It will significantly increase with demographic change. This session will discuss the strategic goal of this initiative: to foster the expansion of 1) new knowledge, 2) research and development resources and 3) technical capabilities, which will enable countries to reduce the prevalence of Alzheimer's disease (modest delay of five years in the onset of brain disability can reduce the cost and prevalence of this chronic condition by half; this view is widely shared by experts in the field).</td>
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<tr>
<td>14:45-15:30</td>
<td><strong>Biomedical Innovation for Alzheimer’s disease and dementia: latest advances</strong></td>
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<td>• <strong>Jean-Noël Octave</strong> - President Institute of NeuroScience, Catholic University of Louvain, Belgium.</td>
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<td>• <strong>Takaomi Saido</strong> - Senior Team Leader, Laboratory for Proteolytic Neuroscience, RIKEN Brain Science Institute, Japan.</td>
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<td>This session will review latest advances in biomedicine for Alzheimer’s and other neurodegenerative disease (e.g. innovative diagnostics, targeted therapies, regenerative medicine, gene therapy). It will highlight the main scientific and technological challenges to the diffusion of these therapies/diagnostics to point of care. It will open the discussion on the link between scientific and technological challenges and governance.</td>
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<td>15:30-16:15</td>
<td><strong>From Bench to Bedside: meeting the needs of patients, academia, and industry</strong></td>
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<td>• <strong>Richard Johnson</strong> - CEO, Global Helix LLC; National Academy of Sciences Board on Life Sciences; Advisory Council, Global Coalition on Aging, United States</td>
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<td>• <strong>Martin Rosser</strong> - Vice-Chair, JPND Scientific Advisory Board.</td>
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<td>This session will address the main challenges in moving innovation in biomedicine for Alzheimer’s disease from research to point of care; the new tools and techniques and platforms of research; the knowledge exchange models between public and private sector. It will discuss the need for interdisciplinary research, the emerging new platforms for collaboration, and the promise of technology convergence in the development of diagnostics and therapies for Alzheimer’s disease.</td>
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<td>16:15-16:30</td>
<td>Coffee break</td>
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16:30-17:15 **Addressing the Big Data Challenges**

- [Donald Stuss](#) - President, Ontario Brain Institute, Canada
- [Robert Simpson](#) - Researcher and Developer Zooniverse, Oxford, UK

The multi-factorial nature of the neurodegenerative diseases requires the collection, storage and processing of increasingly large and very heterogeneous datasets (behavioural, genetic, environmental, epigenetic, clinical data, brain imaging, etc.). No one nation has all the assets to pursue this type of research independently. This session will look at the large scale data collection, storage and analysis problems that need to be solved over the next decade in order to effectively harness technological progress and ensure that data will be turned into useful and actionable health information. It will discuss lessons learned from other sectors on the power of citizen science and crowd-sourcing in addressing complex science problems.

17:15-18:00 **What data can be shared today? Thinking about the tomorrow.**

- [Robert Cook Deegan](#) - Director, Center for Genome Ethics, Law and Policy, Institute for Genome Sciences & Policy, Duke University, United States
- [Simon Lovestone](#) - Professor Old Age Psychiatry, Director NIHR Biomedical Research Centre for Mental Health, King’s College, UK

The development of new diagnostics and treatments for Alzheimer’s disease requires large science and a new approach to clinical trials. Access linkage and sharing of data on Alzheimer’s disease and other dementias remain, however, a serious challenge. This session will discuss what can be shared today /should be shared to accelerate innovation. It will review the major technical and policy challenges to international sharing of data (interoperability, linkage, privacy and security challenges, IPRs) and how advances in information technology is transforming the clinical trial paradigm. It will consider the emerging new role of social networks as patients become agents of innovation.

**Dinner reception for speakers**

- George Vradenburg – Chairman USAgainstAlzheimer's

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**Friday 21 June 2013**

**Creating an enabling policy environment for global collaboration**

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<td>08:00- 08:30</td>
<td>Coffee and registration</td>
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<tr>
<td>08:30-08:45</td>
<td><strong>Opening of Day 2</strong>&lt;br&gt; <em>A brief presentation will be given of Day 2 goals and structure.</em></td>
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<tr>
<td>08:45-09:30</td>
<td><strong>Major policy trends and new regulatory paradigms for fostering the translation of biomedical innovation for Alzheimer’s disease</strong>&lt;br&gt; - <a href="#">Philippe Cupers</a> - Head of Sector, Neuroscience, DG Research and Innovation, European Commission&lt;br&gt; - <a href="#">Mario Romao</a> - Senior Policy Manager, Intel Corporation, Belgium</td>
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This session will look at the main and most recent trends globally, and those that are country specific, in policies to support the safe and efficient translation of biomedicine and health technology for Alzheimer disease, including initiatives relating to the ethical and social impact of...
biomedical research for Alzheimer’s. Speakers will discuss the advances that have been made as a result of these policies and what further progress is needed. This session will also look at whether multidisciplinary research and the combined use of technologies raise any specific policy challenges for the translation of biomedical innovation to patient care. It will discuss how convergence is being addressed in governance models at present.

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| 09:30-10:15  | Fostering Open Access for Alzheimers and dementia research | Walter Kukull - Director, National Alzheimer's Coordinating Center, United States
Giovanni Frisoni - Neurologist and Deputy Scientific Director, IRCCS Fatebenefratelli, The National Centre for Alzheimer's Disease, Brescia, Italy |

This session will discuss opportunities and challenges of open access and open data platforms. It will review the open access policies emerging across OECD countries and their implications. It will look at lessons learned from concerted multidisciplinary efforts to collect and process large scale national and global data and how these can be applied to Alzheimer’s and dementia research (for example, the Brain Activity Map (BAM); the neuGrid project; the recent 100,000 Human Genome Sequencing Project in the UK). It will discuss what is needed to reach international agreement on standards and best practices for data deposit, management, access and sharing.

<table>
<thead>
<tr>
<th>Time</th>
<th>Session Title</th>
<th>Speakers</th>
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</table>
| 10:15-11:00  | Towards an integrated data ecosystem for new smart models of care and research | Tia Powell - Professor of Clinical Epidemiology & Population Health, Albert Einstein College of Medicine Yeshiva University, United States
Mehdi Khaled - Vice President, Healthcare & Life Sciences, Oracle, Singapore |

This session will discuss the challenges in designing and implementing an integrated data ecosystem for new smart models of care and to sustain new research platforms. It will consider the challenges of real-time global research information exchange and what is needed to make it happen. It will discuss the major ethical challenges as the range of research focus is extended to healthy people who are merely at risk for the disease but could benefit from preventive therapies. It will also discuss the opportunity costs to governments and to patients of not making best use of the large streams of health and social care data available, and what frameworks are needed to make progress.

<table>
<thead>
<tr>
<th>Time</th>
<th>Coffee Break</th>
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<tbody>
<tr>
<td>11:00 – 11:15</td>
<td>Coffee Break</td>
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</table>

| Time         | Innovative partnerships to facilitate the translation of biomedical innovation for Alzheimer’s disease | Lefkos Middleton - Professor of Neurology, Neuroepidemiology and Ageing School of Public Health, Imperial College London, United Kingdom
Zaven Khachaturian – President, PAD2020, United States |

This session will look at partnerships in place or being developed at global and national levels to facilitate the translation of biomedical innovation (e.g. national and international public-private collaborations for discovery and validation of biomarkers, for the development of global clinical trials; but also private/private interactions and new models of products development and commercialisation). The session will discuss potential work on case studies on innovative partnerships which would, in a policy context, aim to identify any common characteristics of these initiatives, their mode of knowledge exchange, the main challenges and opportunities they are facing and how policy is or might address those challenges and opportunities.

<table>
<thead>
<tr>
<th>Time</th>
<th>How can the OECD contribute to moving the international agenda forward?</th>
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<tr>
<td>Time</td>
<td>Session</td>
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<td><strong>Moderators:</strong> Elettra Ronchi and Jacqueline Allan (OECD)**&lt;br&gt;This session will take stock of the main messages from the Consultation, highlighting priorities for an international research and policy agenda and setting out a roadmap for OECD action.</td>
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<td>13:15</td>
<td><strong>Concluding remarks</strong></td>
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ANNEX 2: DRAFT QUESTIONNAIRE ON GOVERNMENT APPROACHES FOR BIOMEDICAL INNOVATION FOR ALZHEIMER’S DISEASE AND OTHER NEURODEGENERATIVE DISEASES

Introduction

Human life span and age-related disease continue to increase, leading to rising concern about our ability to provide appropriate health care. The ageing population is putting unprecedented pressure on social and economic systems. Ageing, and ensuring that ageing is healthy, has become a priority for many governments for reasons including economic productivity, financial stability and sustainability, social engagement, human rights and ethics.

Today, dementia is one of the most frequently seen degenerative conditions in the older part of the population, with Alzheimer’s disease being its most common form. Despite the spectacular medical and technological advancements in recent decades, there are no approved treatments to prevent, slow down or cure Alzheimer’s. Biomedicine has an important role to play in enabling people to live longer and healthier lives.

Biomedicine and health technologies are used in the discovery and development of new solutions for the prevention, diagnosis, monitoring and treatment of age-related diseases, notably through the discovery of new biomarkers. An improved environment for innovation and for facilitating the transfer of technology-driven discoveries from the laboratory to the point of care is needed. However, translating scientific and technological advances into innovation in the clinical setting still presents a number of governance challenges.

The Working Party on Biotechnology (WPB) of the Organisation for Economic Cooperation and Development (OECD) is currently engaged in a project on “Healthy Ageing and Biomedical Innovation for Dementia and Alzheimer’s disease”. It aims to identify good practices for the governance of biomedical innovation and health technologies in Alzheimer’s and dementia. This short questionnaire will form the basis of an inventory of government approaches used to support the development of biomedicine and health innovation for Alzheimer’s disease and other neurodegenerative diseases.

The questionnaire should be completed by representatives of national governments, ministries or their agencies responsible for national policy in this area of biotechnology for health policy and returned to the OECD Secretariat not later than 31 January 2014.

Please return your questionnaire to:
Hermann Garden
OECD/DSTI/STP
email: hermann.garden@oecd.org

DEADLINE FOR RESPONSES: 31 January 2014
QUESTIONNAIRE

Responding country: ____________________________

Question 1: Which of the dementia listed below are recognised as priority areas in your country?

☐ Alzheimer’s disease
☐ Parkinson’s disease
☐ Vascular dementia
☐ Other, please specify……..

______________________________________________________________________________________
______________________________________________________________________________________

Question 2: Do you have an integrated national plan on dementia?

☐ Yes
☐ No

If yes, please specify (name, partners, website, etc.)

______________________________________________________________________________________
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Question 3: What are the main fields of research/investment in the priority areas selected in question 1? In each case please specify to which type(s) of dementia they apply

☐ Basic research (for dementia……__________________________)
☐ Translational research (for __________________________)
☐ Clinical research (for __________________________)
☐ Research infrastructures (for __________________________)
☐ Funding of individual research groups (for __________________________)
☐ Funding of international networks (for __________________________)
☐ Funding of Public Private Partnerships (for __________________________)
☐ Other, please specify………..

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**Question 4:**

a. In meeting the challenge of Alzheimer’s and dementia, what types of policy are relevant for your country? Please give a score from 1 to 5 for each proposition according to their importance in your country (1 = not important, 5 = very important).

<table>
<thead>
<tr>
<th>Challenge-driven investment for healthy ageing (e.g. tackling diseases of ageing populations)</th>
<th>1</th>
<th>2</th>
<th>3</th>
<th>4</th>
<th>5</th>
</tr>
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<tbody>
<tr>
<td>Strengthening the science base (e.g. support for the development of tools and techniques that underpin biomedical innovation for Alzheimer’s, such as omics technologies, the development of animal models)</td>
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<tr>
<td>Strengthening business R&amp;D and innovation capacity</td>
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<td>Linking public research and industry (e.g. innovative public-private partnerships)</td>
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<td>Linking the research community with policy makers</td>
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<td>Supporting public engagement and patients’ involvement</td>
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<td>Developing translational research (e.g. new schemes of public-private research funding, pre-competitive consortia for shared risk management)</td>
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<tr>
<td>Developing human resources and skills</td>
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<tr>
<td>Improving physical infrastructure</td>
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<tr>
<td>Improving framework conditions (e.g. IPRs, competition)</td>
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<tr>
<td>Investment in regulatory science</td>
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<tr>
<td>Internationalisation (e.g. international partnerships and collaboration)</td>
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<tr>
<td>Other(s): ____________</td>
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</table>
b. Please list examples of initiatives, support programmes and policies in your country for each topic for which you have chosen a grading of 4 or 5.

______________________________________________________________________________________
______________________________________________________________________________________
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Question 5: Please indicate the major policy tools used in your country to support biomedical research addressing dementia:

______________________________________________________________________________________
______________________________________________________________________________________
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Question 6: What changes have been introduced in national Science, Technology and Innovation policy in your country in the past 10 years, as a response to the ageing population and the growing issue of dementia?

______________________________________________________________________________________
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Question 7: In your opinion, what are the main barriers for innovation in dementia?

☐ Inadequate policies

☐ Financial constraints

☐ Lack of awareness

☐ Inadequate coordination of research; lack of networks

☐ Other: please specify………

______________________________________________________________________________________

______________________________________________________________________________________
Question 8: How is your country engaged in supporting innovative biomedical research approaches for dementia (e.g. non-invasive imaging techniques, omics technologies, induced-pluripotent stem cells, nanosciences, synthetic biology)?

______________________________________________________________________________________

______________________________________________________________________________________

______________________________________________________________________________________

Question 9: Regulatory science represents the basis for rational decision-making of approval processes for clinical research, marketing authorisation, medicines labelling. What are the activities in your country to support regulatory science for emerging biomedical research techniques?

☐ Establishment of a strategic plan for regulatory science within the national authority

☐ Hiring scientific experts, consultancies

☐ Establishment of partnerships

☐ Specific staff training

☐ Establishment of dedicated department for regulatory science

☐ Other, please specify

______________________________________________________________________________________

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ANNEX 3: CASE STUDIES ON PUBLIC-PRIVATE PARTNERSHIPS FOR BIOMEDICINE AND HEALTH TECHNOLOGIES FOR ALZHEIMER’S DISEASE AND OTHER NEURODEGENERATIVE DISEASES

Background: The Organisation for Economic Cooperation and Development (OECD), through its Working Party on Biotechnology, is undertaking a project on “Healthy Ageing and Biomedical Innovation for Dementia and Alzheimer’s disease”. Its aim is to identify good practices to strengthen effective cooperation at a global level for the governance of biomedical innovation and health technologies in Alzheimer’s and other neurodegenerative diseases. One component of this project is the investigation of innovative partnerships (public-private partnerships) to foster biomedicine research in Alzheimer’s and other neurodegenerative diseases.

Objectives: There are three main objectives to this part of the project: 1) identifying the main initiatives that are being developed that help foster research for Alzheimer’s disease and other neurodegenerative diseases; 2) identifying the factors that have affected progress in these initiatives; and 3) identifying good practice and drawing lessons for future use.

Instructions for completion: You are being asked to identify and provide information about one or more national and/or international Public-Private Partnerships (PPPs) for biomedicine and health technologies for Alzheimer’s and other neurodegenerative diseases.

Please note that many European countries are involved in the EU’s Joint Programming Initiative on Neurodegenerative Diseases (JPND). If you are involved in the JPND, we ask you to identify other PPS and to mention only in your email response to us that you are also engaged in the JPND.

Please return the completed template to the OECD Secretariat to the WPB at:

Hermann Garden: hermann.garden@oecd.org
Marie-Ange Baucher: marie-ange.baucher@oecd.org
A. General Guideline

(1) Participating delegations are requested to nominate up to 3 flagship Public-Private Partnerships (PPPs) for biomedicine and health technologies for Alzheimer’s and other neurodegenerative diseases.

(2) Participating delegations can choose PPPs at the programme level, project level or both.

(3) Participating delegations are invited to address the 8 themes presented in the below template in their case studies (PPPs) and to provide charts and statistics where available. Participating delegations are invited to highlight the key features of their PPPs such as governance arrangements, modes of financing, IPRs, internationalisation and evaluation. Please provide links to websites of relevant sources of information.

- **Length:** approximately 20-25 pages per case study, including references and appendices.
- **Submission deadline:** 31 January 2014
- **Contact point:** hermann.garden@oecd.org; and marie-ange.baucher@oecd.org

B. Case Study Template

The template provides a framework for the development of the case studies on public-private partnerships (PPPs) for biomedicine and health technologies for Alzheimer’s and other neurodegenerative diseases. It is composed of eight different sections, which are:

<table>
<thead>
<tr>
<th>1. Overview of programme/project objectives</th>
<th>2. Rationale, motives and key drivers</th>
</tr>
</thead>
<tbody>
<tr>
<td>3. Financing issues</td>
<td>4. The management of IPRs</td>
</tr>
<tr>
<td>5. Governance issues</td>
<td>6. International context</td>
</tr>
<tr>
<td>7. Evaluation issues</td>
<td>8. Lessons and challenges ahead</td>
</tr>
</tbody>
</table>
1. Overview of programme/project objectives

**Basic information to collect**

- Provide a summary of the cases, including key elements such as project name, responsible Ministry or agencies, private partners.
- Describe the objectives of the programme (what was the problem that it is trying to solve).
- Describe the research focus.
- The type of programme (e.g. a public grant programme on a co-investment basis, a strategically-initiated programme on a contract basis between public and private partners).

2. Rationale, motives and key drivers

**Possible issues to explore**

- The underlying motives for initiating the PPP (e.g. sharing of risk and resources, private expertise, developing breakthrough or bottleneck technology, commercialisation of public R&D, building innovative infrastructure, creating job and income).
- The underlying motives for joining the PPP from a business perspective. (what instigates participation of private partners in the PPP despite growing risk, like high growth potential, advantages in early market, access to IPRs, cost-sharing, and procurement incentive).
- The key drivers of the PPP and the reason why they are the key drivers, based on the experiences in the cases.

3. Financing issues

**Possible issues to explore**

- The financing arrangements in the PPP project, including financing structure, total project cost, cost-sharing rate, funding instruments (e.g. cash, in-kind, loan, and equity).
- De-risking or the way cost-sharing rate is decided (e.g. depending on the risk undertaken or growth potential to be created), and the most important factor affecting the decision of cost-sharing.
- The actors who fund the PPPs (e.g. governments, universities, foundations, public entities or firms, or both), and their funding stage (e.g. seed, early, late stage or full stage).
- The firm size of the participants who received financially support in the PPPs (e.g. small start-ups, SMEs, or large-scale enterprises).
- The fiscal incentives that the participants enjoy (e.g. tax incentives on investments or capital gains or losses, government guarantee).
4. The management of IPRs

Possible issues to explore

- The management of IPRs, the role of open innovation approaches, IPR sharing mechanisms, etc.

5. Governance issues

Possible issues to explore

- Type of governance model (e.g. managed by an independent organisation such as dedicated PPP unit, or on a project basis or contract basis, operated by centrally or fragmentally, at local or regional or national level or beyond), and the reasons why that type is introduced.
- The legal status of the PPPs project.
- The ways to monitor the progress of the PPPs project (e.g. performance evaluation, feedback system, organising a review team).
- The mode of collaboration under the PPP scheme (e.g. carrying out research at a central lab or dispersed labs to which the PPP participants belong).
- The level of cooperation among the participants (e.g. the facilitators and barriers to increasing collaboration).
- The co-ordinator who can play a moderating role when conflicts of interest occur among the participants concerned.
- The measures to improve transparency in the management of the PPP projects.
- PPP governance model considered to be the best in terms of efficiency based on the experiences.

6. International context

Possible issues to explore

- The critical barriers to the global PPPs (e.g. IPR issues or laws and regulations).
- Regarding open innovation, the impact that PPPs bring on the economy at large on a global context (e.g. positive, negative, or both).

7. Evaluation issues

Possible issues to explore

- The agency that evaluates the performance of the PPPs project, including the timing of evaluation, management of fairness and transparency.
• The criteria and key indicators for evaluating PPPs. Whether the indicators reflect the performance of the PPPs adequately.
• The feedback mechanism following the evaluation.
• The incentives and disincentives as a consequence of the evaluation (e.g. increased budget or award as an incentive, reduced funding or suspension of the project).

8. Lessons and Challenges ahead

Possible issues to explore

• Identify good practices from the PPP case study.