The EU Joint Programme on Neurodegenerative Disease Research (JPND) has been established by 23 European countries to address the growing societal challenge presented by age-related neurodegeneration. This initiative spans the biomedical, healthcare and social science agendas, and seeks to improve the scientific understanding of neurodegenerative disorders, provide new approaches for their prevention, diagnosis and treatment, and ensure effective provision of health and social care and support, so that individuals can receive optimum care at all stages of their illness.

During 2011 the initiative will establish a joint ‘Strategic Research Agenda’ (SRA) for future research into neurodegenerative diseases. This work began by bringing together experts and other stakeholders from across Europe in order to identify common ground where JPND could consider action. The following overarching principles were established through a series of workshops:

- There should be wider availability and easier access to data and materials, as well as active dissemination of results
- Methodologies should be harmonised and technology platforms standardised wherever possible
- Education and awareness of neurodegenerative diseases (NDDs) should be promoted across all groups
- Ethical, legislative and regulatory issues related to this area need to be clarified and addressed

About this consultation

This web-based consultation comprises nine pages of questions, in addition to entry of personal contact details. Most users will complete the consultation in around 30 minutes.

The survey questions are based on discussion points and suggestions for progress identified to date through a series of expert workshops and meetings. Questions have been grouped into broad categories:

- Education, training and collaboration
- Health and social care issues
- Prevention/treatment strategies and trials
Disease cause, mechanisms and models
Diagnosis, disease definitions and outcome measures
Data, registries, repositories and centres
Policy, regulation and legislation
Funding and funding mechanisms

For each section you will be asked to:

- Indicate your level of support for individual statements
- Select those you think are most important or should be given priority
- Provide further input on specific points

Some categories are largely aimed at specific stakeholder groups, though anyone is free to respond to any section. You can skip any section by pressing the ‘Continue’ button at the foot of each page. If you do not have an opinion or are unsure of how to answer a specific point please leave blank.

A glossary containing definitions of several of the terms and categories used in the survey is available as an annex at the end of this PDF file.

As long as your web browser has cookies enabled and you do not clear your cache, once you have started the survey you have 10 days to complete it; your responses will be retained during this period and you can leave and return as many times as you like using the same computer. However, if you do not complete the survey within 10 days of starting it anything that you have already entered will be lost and will need to be re-entered on your return. Consultation responses can be submitted up to 11pm (GMT +1) on Sunday 18th September when the survey will close; it will not be possible to respond after this point.

**Next steps**

Responses to the consultation will be taken into consideration by the JPND when developing the Strategic Research Agenda which we plan to publish in early 2012. An analysis will be published alongside the SRA in early 2012. Where possible, we intend to post consultation responses on this website, listed by individual and by organisation.

**Troubleshooting and comments**

If you have problems accessing or using the survey please email

JPNDConsult(at)headoffice.mrc.ac.uk
Please select the category that best represents you or your organisation *

- Academic researcher
- Commercial researcher
- Charity organisation or patient group
- Funding agency, policy-maker or regulator
- Healthcare professional
- Patient with a neurodegenerative condition or their carer
- General public or other

May we include your / your organisation’s response on this website? *

- Yes, attributed to myself or my organisation
- Yes, anonymously*
- No, I/we do not wish my/our response to be used other than in the overall analysis of responses

*If you select this option, you should ensure that your name does not appear in the main text of your response. JPND cannot take responsibility for anonymising responses in which the individual or organisation is identifiable from the content of their response.
You will now be asked to complete you or your organisations contact details. Note that the information asked for is different for different types of respondees. The example given here is for a respondee who has classified themselves as being affected by a neurodegenerative condition, either as a patient or a carer.

**Title**
e.g. Mr, Mrs, Ms, Dr, Professor

**First Name**

**Last Name**

**Country**
-- None --

**Contact email address**

**Are you a patient with a neurodegenerative condition or a carer?**
- Patient
- Carer

**Please indicate which condition you, or the person you care for, is affected by**
- Alzheimer's Disease or other dementias
- Parkinson's Disease
- Huntington's Disease
- Spinal Muscular Atrophy
- Spinocerebellar Ataxia
- Prion related disorders
- Other (please specify below)
**Question 1:** Please indicate to what extent you endorse the following points

**a) Improve dialogue between researchers and the wider population**
We need to achieve better general awareness and knowledge of research and increase understanding of how participation can improve research quality and progress

O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**b) Improve education and training of healthcare professionals**
Training of healthcare professionals with respect to patient needs and healthcare options needs to be improved and to be based on the latest research

O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**c) Increase numbers of neurodegenerative disease (NDD) researchers**
More researchers are needed especially in fields such as health economics and bioinformatics

O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**d) Increase training for translational and clinician-scientists**
There needs to be more emphasis on developing good translational and clinician-scientists and on developing the skills of clinicians who can assist with trials

O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**e) Increase numbers of post-doctoral level researchers**
Efforts should be directed to facilitate recruitment of good researchers at the post-doctoral level

O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**Question 2:** If you had to choose one priority from the points above what would it be?

**Question 3:** Are there any other specialist areas which you think need promoting or should be given greater emphasis?
In this section we would particularly like to hear from researchers and individuals involved in health and social care, organisations representing patients and carers, individuals with NDD or their representatives and the wider public, however all are welcome to respond.

**Question 1: Please indicate how far you endorse the following points**

**a) Define the term ‘care’**
There is no agreed standard definition of the term ‘care’
- Strongly Agree
- Agree
- Neutral
- Disagree
- Strongly Disagree

**b) Survey long-term care standards and provision across Europe**
Uncertainties surround the level and standards of care provision across Europe
- Strongly Agree
- Agree
- Neutral
- Disagree
- Strongly Disagree

**c) Research into the needs of carers**
The needs of carers are often overlooked and research is needed to understand more closely how carers can be supported in an effective way
- Strongly Agree
- Agree
- Neutral
- Disagree
- Strongly Disagree

**d) Research into care approaches including end of life decision-making**
Care approaches and decision-making in relation to the end of life need to take more account of how NDD affect physical and mental capacity
- Strongly Agree
- Agree
- Neutral
- Disagree
- Strongly Disagree

**e) Rethink approaches to care**
Insufficient emphasis has been placed on providing care at home and how care can be aided by the implementation of assisted-living technologies. Innovative approaches are needed as part of systems that aim to minimise the demands on family and carers and to improve quality of life across the stages of NDD
- Strongly Agree
- Agree
- Neutral
- Disagree
- Strongly Disagree

**f) Understand and investigate influence of comorbidities on NDD**
The effect that co-existing medical conditions such as diabetes have on NDDs is not sufficiently well understood
- Strongly Agree
- Agree
- Neutral
- Disagree
- Strongly Disagree

**d) Conduct research into effects of nutrition and frailty**
How nutrition and frailty influence the development and progression of NDD is not well understood
- Strongly Agree
- Agree
- Neutral
- Disagree
- Strongly Disagree

**g) Determine cost-effectiveness of healthcare pathways**
The cost-effectiveness of pathways to diagnosis, intervention, care and support needs to be determined and the results factored into healthcare system approaches
- Strongly Agree
- Agree
- Neutral
- Disagree
- Strongly Disagree
Question 2: Please rank the suggestions in order of priority

First Priority

Second Priority

Third Priority

Question 3: How would you define ‘care’?

Question 4: Have you any (additional) suggestions as to how care systems should be revised?

Question 5: Provision of care at home carries both advantages and disadvantages, how do you suggest we change the balance to promote the advantages?
**Prevention/treatment strategies and trials**

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**Question 1: Please indicate how far you endorse the following points**

a) Increase involvement of individuals in research
   Currently too few people with NDD are enrolled in research studies or are registered to donate brain material
   O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

b) Promote development of non-pharmacological interventions
   There is a need for more non-pharmacological interventions – to include psychosocial interventions - and strategies to promote social inclusion in order to prevent and treat NDD
   O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

c) Conduct multi-centre primary prevention studies
   Studies aimed at preventing NDD should take as their basis what we currently know about factors that increase or decrease the risk of developing disease
   O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

d) Ensure better patient selection/stratification
   Clinical trials should involve more specific (sub)groups of participants or be tailored to those participants who are most likely to respond
   O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

e) Rethink approach to therapeutics
   It needs to be recognised that when there is no cure, treatments that improve quality of life are important for people with NDD and this information is relevant when determining whether clinical trials are a success
   O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

f) Support for high-risk projects
   Where there are potential future benefits for themselves or other people with NDD, patients are likely to be willing to participate in trials that carry more personal risk than regulatory guidance currently allows
   O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**Question 2: Please rank the suggestions in order of priority**

**First Priority**

**Second Priority**

**Third Priority**
Question 3: How can we encourage more people to take part in research and/or register to donate brain material?

Question 4: If you think we are in the position to begin multi-centre primary prevention trials, what measures do you think should be trialled?
In this section we would particularly like to hear from academic and industry researchers working in basic and clinical science, regulators and policy makers and organisations representing patients and carers, however all are welcome to respond.

**Question 1: Please indicate how far you endorse the following points**

**a) Understand relationship between neurodegenerative disease and ageing**
More research is needed into ageing, and the relationship between NDD pathology and 'normal' ageing
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**b) Improve understanding of disease stages**
Understanding of the various stages of disease progression needs improvement, and there is a need to determine how patients’ quality of life can be improved especially at the mid-stage of their illness
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**c) Improve understanding of disease mechanisms**
A more complete understanding of the biological mechanisms of disease across the various NDDs is needed particularly as these conditions are increasingly seen to share common features
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**d) Develop an improved understanding of the genetic basis for NDD**
With regard to genetic factors, the predictiveness of disease markers for early diagnosis needs to be determined
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**e) Determine the importance of genetic and environmental risk factors for NDD**
The factors associated with developing NDD, their importance, and how they interact with each other needs to be fully understood
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**f) Focus research on rare hereditary forms of disease**
To achieve early gains, research should focus on defined hereditary forms of disease before progressing to investigation of other forms
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high-risk groups**
Large pan-European studies are needed to better understand the causes and development of NDD; studies should be designed so that subjects could easily participate in clinical trials
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**h) Develop more representative animal and cell-based models of disease**
It is important to recognise that existing animal models mimic mechanistic pathways rather than human diseases. Innovative models are required that more closely replicate human disease as well as human-derived cell-based models that can be used in drug development and for drug testing
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree
Question 2: Please rank the suggestions in order of priority

First Priority

Second Priority

Third Priority

Question 3: In relation to disease mechanisms which of the following do you think it is most important to investigate?

- a) Interactions between cells and their surrounding intra- and extracellular environment
- b) Neuronal inflammation
- c) Mechanisms of neuronal death and dysfunction
- d) The biological basis of behavioural and psychological symptoms

Question 4: Do you think there could be justification for progressing research from cell-based models straight to humans (i.e. missing out animal models)? If so please explain under what circumstances this could be acceptable.
In this section we would particularly like to hear from academic and industry researchers working in basic and clinical science, regulators and policy makers and organisations representing patients and carers, however all are welcome to respond.

**Question 1: Please indicate how far you endorse the following points**

a) **Redefine and standardise disease definitions and diagnosis**
   For better, earlier and more consistent diagnosis, effort should be placed on redefining and standardising disease definitions/parameters and diagnostic procedures

   O Strongly Agree  O Agree  O Neutral  O Disagree  O Strongly Disagree

b) **Redefine and harmonise clinical endpoints and outcomes**
   To ensure applicability across large and varied populations, clinical endpoints/outcomes need to be redefined and harmonised and to incorporate measures that reflect quality of life for the patient

   O Strongly Agree  O Agree  O Neutral  O Disagree  O Strongly Disagree

c) **Develop new biomarkers**
   New and improved biomarkers must be developed and validated to enable early diagnosis, disease stratification and measurement of disease progression across large study populations

   O Strongly Agree  O Agree  O Neutral  O Disagree  O Strongly Disagree

d) **Consider regulatory approaches**
   Researchers should take greater account of the requirements of regulatory agencies earlier in their research, for example when designing biomarker studies

   O Strongly Agree  O Agree  O Neutral  O Disagree  O Strongly Disagree

**Question 2: Which of the following do you think is most important in terms of biomarkers?**

O a) Linking to the mechanism of disease and functional endpoints

O b) Linking to treatment responses

O c) Providing an indicator of, and sensitivity to, disease progression

O d) Facilitating back-translation to models of disease
In this section we would particularly like to hear from academic and industry researchers working in basic and clinical science, regulators and policy makers and organisations representing patients and carers, however all are welcome to respond.

**Question 1: Please indicate how far you endorse the following points**

**a) Improve access to, and sharing of, infrastructure and resources**
Scientists working on NDD need better access to infrastructure and resources
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**b) Improve access to patient groups, samples and data**
To facilitate and speed the pace of research we need to improve access to larger patient groups, patient samples and data, especially for scientists from industry. Additionally, academic scientists need better access to industry data, particularly trial results, as this would help inform and influence their research
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**c) Improve data and sample collection**
Common methodologies to share data and samples should be developed wherever possible
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**d) Develop a register of persons with cognitive impairment**
To conduct effective studies on how NDD develops, understand disease subtypes and facilitate clinical trials and studies it would be helpful to have a register of people who may show early signs of NDD
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**e) Develop centres of excellence**
Centres of excellence that concentrate resources and expertise in one place should be developed and combine multidisciplinary research, treatment and education. Dedicated translational research centres should also be developed.
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**Question 2: Please rank the suggestions in order of priority**

**First Priority**

**Second Priority**

**Third Priority**
Question 3: What can be done to facilitate increased sharing of data?

Question 4: What are your views on making data open access? If you foresee difficulties, how can we overcome these?

Question 5: Relating to point (e) do you see any risk(s) in developing centres of excellence?
This section is aimed primarily at policy makers, regulators and representatives from funding agencies, but is open to all to respond.

**Question 1: Please indicate how far you would endorse the following points**

**a) Need for evidence-based policy**  
Evidence-based policy should be promoted wherever possible.  
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**b) Ensure greater engagement with regulators**  
As for biomarker studies researchers need to work more closely with regulators to ensure greater, and earlier, input and remove (unnecessary) barriers or impediments to conducting research  
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community**  
There is a need to understand what is happening throughout the healthcare system and throughout society in general  
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**d) Encourage industry to adopt a pre-competitive approach to research**  
Drug development in large pharmaceutical companies may be best served by industry sharing information and resources during early stage development then reverting to a competitive structure  
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**e) Rethink patent lifetime and conduct public-private clinical trials**  
Currently patents relating to therapeutic drugs are granted for a fixed period of time. Industry representative suggested this was too short for commercial drug development in neurodegeneration to be viable. Systems similar to those for conducting joint public-private clinical trials may encourage companies to continue working in this area  
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**f) Review and update legislation on treatment**  
Treatment of individuals with asymptomatic, at risk and early stage NDD raises ethical issues that may require existing regulatory frameworks to be updated or new legislation developed  
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree

**g) Review and update legislation on privacy and data disclosure**  
Legislation into privacy and data disclosure should take into consideration the needs and wishes of patients and carers with reference to NDD  
O Strongly Agree O Agree O Neutral O Disagree O Strongly Disagree
Question 1: Please rank the suggestions in order of priority

First Priority

Second Priority

Third Priority

Question 2: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?
This section is aimed at **policy-makers, regulators and representatives from funding agencies**, but is open to all to respond.

**Question 1: Please indicate how far you endorse the following general points**

**a) Translational research needs to be promoted**
Cross disciplinary research is needed to make the process of turning biological discoveries into therapeutics more efficient and effective.
- [ ] Strongly Agree
- [ ] Agree
- [ ] Neutral
- [ ] Disagree
- [ ] Strongly Disagree

**b) Encourage open-access sharing of data and materials**
Collaborative studies should include a requirement for open-access sharing of data and materials.
- [ ] Strongly Agree
- [ ] Agree
- [ ] Neutral
- [ ] Disagree
- [ ] Strongly Disagree

**c) Joint academic-industry funding models**
There is a need for funding mechanisms to encourage industry and academia to work together to facilitate the collaborative development of new treatments.
- [ ] Strongly Agree
- [ ] Agree
- [ ] Neutral
- [ ] Disagree
- [ ] Strongly Disagree

**d) Simplify funding application systems**
European funding application systems should be simplified and standardised.
- [ ] Strongly Agree
- [ ] Agree
- [ ] Neutral
- [ ] Disagree
- [ ] Strongly Disagree

**e) Maintain capacity for 'bottom-up' innovative funding**
Funding and funding mechanisms should maintain capacity for bottom-up and innovative research.
- [ ] Strongly Agree
- [ ] Agree
- [ ] Neutral
- [ ] Disagree
- [ ] Strongly Disagree

**Question 2: please rank the suggestions in order of priority**

**First Priority**

**Second Priority**

**Third Priority**

**Question 3: Please expand on point (b) if you have any experience or suggestions for alternatives or that may help us achieve this goal**
Here, we would like to hear from all respondents

**Question 1: Are there strategies you think we have overlooked, if so please suggest below (up to three suggestions)**

**Question 2: Do you have any comments on how to implement the above suggestion(s)?**

**Question 3: Is there anything else you would like to tell us?**

**Do you want to be included in the JPND stakeholder database?** *

- [] Yes
- [] No
- [] I am already on the database
Annex – Glossary of terms

The following definitions are used by the JPND initiative:

**Animal models** - can reproduce all aspects of a condition or part of a disease pathway. They are used both to test our understanding of disease pathways, and to provide the tools for developing and assessing therapeutic approaches.

**Back-translation** – the process of taking a research finding along the translational pathway for clinical research and using it to inform or validate an earlier stage (in the process).

**Biomarker(s)** – short for biological marker; a characteristic that is measured or evaluated as an indicator of a biological state e.g. to evaluate the presence or progression of disease or a response to treatment.

**Clinical trial** – a scientifically controlled study that is carried out in consenting human beings to evaluate the safety and effectiveness of a therapy.

**Comorbidity** - the presence of one or more disorders in addition to (in this case) neurodegenerative disease.

**Mechanistic pathway** – the components involved in a physiological process and their relationship and interactions with each other.

**NDD** – neurodegenerative diseases. Included in the initiative are Alzheimer's disease and other dementias, Parkinson's disease (PD) and PD-related disorders, Prion disease, Motor neurone diseases, Huntington's disease (HD), Spinocerebellar ataxia (SCA) and Spinal muscular atrophy (SMA).

**Non-pharmacological interventions** - prevention strategies or treatments such as physical or behavioural therapy that do not involve the use of chemical agents.

**Psycho-social interventions** - can be to treat or prevent a condition using educational, behavioural and/or cognitive approaches.

**Stratification** – the process of dividing patients into more specific classes or groups with the idea that their response to treatment will be more uniform (and so easier to assess).

**Translation** – the integration of research across disciplines especially basic and clinical science in order to turn biological discoveries into drugs or medical devices that can be used in the treatment of patients.