

Joint Programme on Neurodegenerative Disease

Online Consultation



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](mailto: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 *

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)

Education, training and collaboration

(Page 3 of 11)

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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree Strongly Disagree

c) Increase numbers of neurodegenerative disease (NDD) researchers

More researchers are needed especially in fields such as health economics and bioinformatics

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree Strongly Disagree

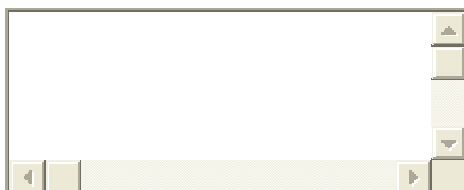
e) Increase numbers of post-doctoral level researchers

Efforts should be directed to facilitate recruitment of good researchers at the post-doctoral level

Strongly Agree Agree Neutral Disagree 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?



Health and social care issues

(Page 4 of 11)

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

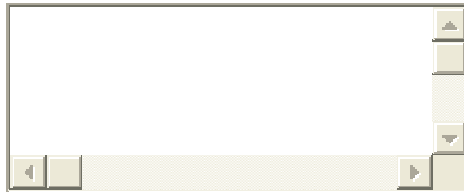
Question 2: Please rank the suggestions in order of priority

First Priority

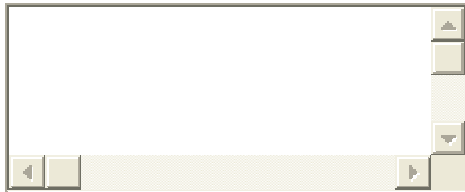
Second Priority

Third Priority

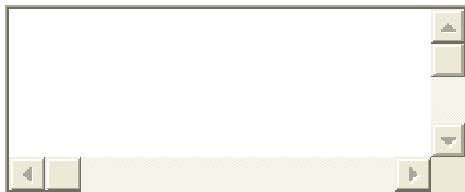
Question 3: How would you define 'care'?

A rectangular text input field with a light beige background and a thin border. It features a vertical scrollbar on the right side and a horizontal scrollbar at the bottom, indicating it is a scrollable text area.

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

A rectangular text input field with a light beige background and a thin border. It features a vertical scrollbar on the right side and a horizontal scrollbar at the bottom, indicating it is a scrollable text area.

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

A rectangular text input field with a light beige background and a thin border. It features a vertical scrollbar on the right side and a horizontal scrollbar at the bottom, indicating it is a scrollable text area.

Prevention/treatment strategies and trials

(Page 5 of 11)

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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree Strongly Disagree

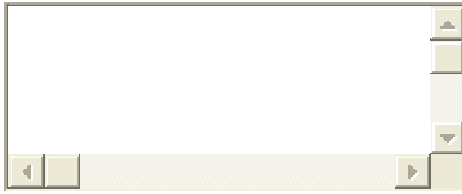
Question 2: Please rank the suggestions in order of priority

First Priority

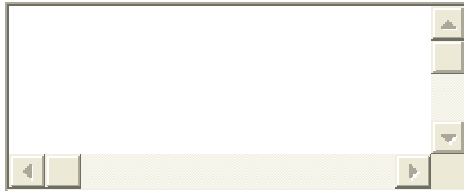
Second Priority

Third Priority

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

An empty text input box with a light beige background and a thin border. It features a vertical scrollbar on the right side and a horizontal scrollbar at the bottom. There are small navigation icons: a left arrow and a right arrow on the bottom left, and a top arrow and a bottom arrow on the right side.

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?

An empty text input box with a light beige background and a thin border. It features a vertical scrollbar on the right side and a horizontal scrollbar at the bottom. There are small navigation icons: a left arrow and a right arrow on the bottom left, and a top arrow and a bottom arrow on the right side.

Disease cause, mechanisms and models

(Page 6 of 11)

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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree Strongly Disagree

Question 2: Please rank the suggestions in order of priority

First Priority

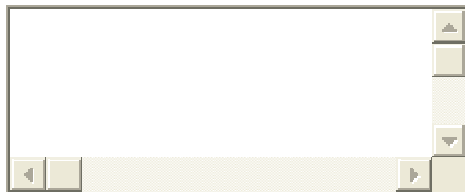
Second Priority

Third Priority

Question 3: 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.



Diagnosis, disease definitions and outcome measures

(Page 7 of 11)

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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree Strongly Disagree

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

- a) Linking to the mechanism of disease and functional endpoints
- b) Linking to treatment responses
- c) Providing an indicator of, and sensitivity to, disease progression
- d) Facilitating back-translation to models of disease

Data, registries, repositories and centres

(Page 8 of 11)

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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree Strongly Disagree

c) Improve data and sample collection

Common methodologies to share data and samples should be developed wherever possible

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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.

Strongly Agree Agree Neutral Disagree Strongly Disagree

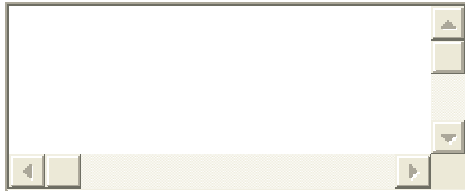
Question 2: Please rank the suggestions in order of priority

First Priority

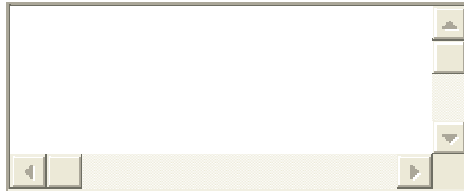
Second Priority

Third Priority

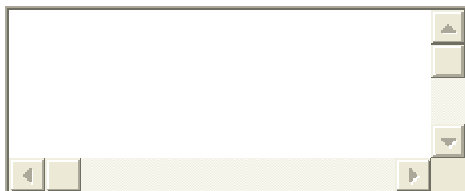
Question 3: What can be done to facilitate increased sharing of data?

An empty text input box with a light beige background and a thin border. It features a vertical scrollbar on the right side and a horizontal scrollbar at the bottom, both with small square handles.

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

An empty text input box with a light beige background and a thin border. It features a vertical scrollbar on the right side and a horizontal scrollbar at the bottom, both with small square handles.

Question 5: Relating to point (e) do you see any risk(s) in developing centres of excellence?

An empty text input box with a light beige background and a thin border. It features a vertical scrollbar on the right side and a horizontal scrollbar at the bottom, both with small square handles.

Policy, regulation and legislation

(Page 9 of 11)

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.

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree 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

Strongly Agree Agree Neutral Disagree Strongly Disagree

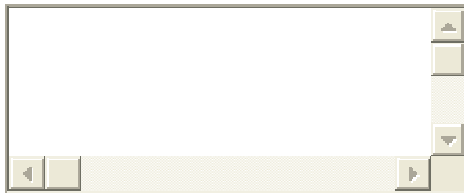
Question 1: Please rank the suggestions in order of priority

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?



Funding and funding mechanisms

(Page 10 of 11)

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

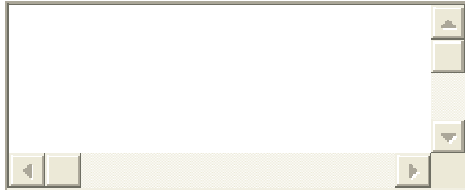
Question 3: Please expand on point (b) if you have any experience or suggestions for alternatives or that may help us achieve this goal

Concluding questions

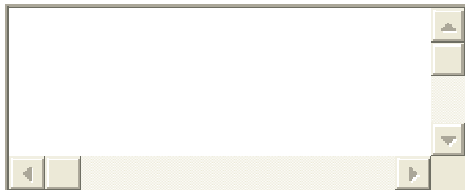
(Page 11 of 11)

Here, we would like to hear from all respondents

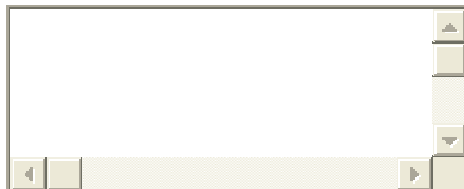
Question 1: Are there strategies you think we have overlooked, if so please suggest below (up to three suggestions)

A rectangular text input field with a light beige background and a thin border. It contains several small, light beige buttons: a left arrow, a right arrow, and a vertical stack of three buttons (up, down, and a small square) on the right side.

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

A rectangular text input field with a light beige background and a thin border. It contains several small, light beige buttons: a left arrow, a right arrow, and a vertical stack of three buttons (up, down, and a small square) on the right side.

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

A rectangular text input field with a light beige background and a thin border. It contains several small, light beige buttons: a left arrow, a right arrow, and a vertical stack of three buttons (up, down, and a small square) on the right side.

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

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