Directory of commercial respondees

Responses from companies

Anonymous responses from companies

Responses from individuals

Anonymous responses from individuals

An additional 5 responses were received from individuals and companies who requested that their submission be used for no other purpose than in the overall analysis of responses.

UID
1916
Title
Dr
First Name
Thomas
Surname
Rooney
Category of respondee
Commercial researcher - Pharmaceutical
Company
SANOFI
Position
Head Translational Research, Neurodegenerative Diseases Group
Replying on behalf of organisation/company?
On behalf of my company
Country
France

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Strongly Agree
b) Improve education and training of healthcare professionals
Strongly Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Strongly Agree
d) Increase training for translational and clinician-scientists
Strongly Agree
e) Increase numbers of post-doctoral level researchers
Agree

Question 2: If you had to choose one priority from the points above what would it be?
d) Increase training for translational and clinician-scientists

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?
Multimodal Neuroimaging Biomarkers for patient selection, monitoring disease progression and therapeutic activity (POM/POC) Access to well characterised patient cohorts for clinical trials Improved animal models with better predictive value Regulatory Framework for NDD More interaction with patients to better assess unmet medical needs, evaluate risk factors for NDD and to encourage participation in clinical trials

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
Agree
b) Survey long-term care standards and provision across Europe
Agree
c) Research into the needs of carers
Strongly Agree
d) Research into care approaches including end of life decision-making
Agree
e) Rethink approaches to care
Strongly Agree
f) Understand and investigate influence of comorbidities
Strongly Agree
g) Conduct research into effects of nutrition and frailty
Agree
h) Determine cost-effectiveness of healthcare pathways
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Survey long-term care standards and provision across Europe
Second Priority
c) Research into the needs of carers
Third Priority
f) Understand and investigate influence of comorbidities on NDD

Question 3: How would you define "care"?

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

NDD patients have other needs than only treating their â€œdisease conditionâ€□. Nutritional, social and environmental aspects need to be also considered for maintaining a good QoL. Better understanding/assessment of cultural and socio-economic environment and their impact on the incidence of NDD (AD in particular).

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

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Strongly Agree
b) Promote development of non-pharmacological interventions
Strongly Agree
c) Conduct multi-centre primary prevention studies
Strongly Agree
d) Ensure better patient selection/stratification
Strongly Agree
e) Rethink approach to therapeutics
Strongly Agree
f) Support for high-risk projects
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
d) Ensure better patient selection/stratification
Second Priority
a) Increase involvement of individuals in research
Third Priority
f) Support for high-risk projects

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

More interaction and education with patients to better assess unmet medical needs and explain why research and participation to trials is important

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?

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
Strongly Agree
b) Improve understanding of disease stages
Strongly Agree
c) Improve understanding of disease mechanisms
Strongly Agree
d) Develop an improved understanding of the genetic basis for NDD
Agree
e) Determine the importance of genetic and environmental risk factors
Agree
f) Focus research on rare hereditary forms of disease
Agree
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Strongly Agree
h) Develop more representative animal and cell-based models of disease
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Improve understanding of disease stages
Second Priority
c) Improve understanding of disease mechanisms
Third Priority
h) Develop more representative animal and cell-based models

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

c) Mechanisms of neuronal death and dysfunction

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

Could be justified but would depend on what type of cellular model. Several laboratories are evaluating use of using iPS cell technology to take eg skin samples from NDD patients to differentiate into neurons (ie neurons with human NDD pathology). This could provide a better in votro model, but there are still questions regarding whether such neurons really show a full neuronal phenotype or whether they are a mixed phenotype. The challenges would be how to select doses for clinical trials (efficacy and saftey) and the lack of any in vivo pharmacology data to support the proof of mechanism or proof of concept for the hypothesis being tested.

Diagnosis, disease definitions and outcome measures

Question 1: Please indicate to what extent you endorse the following points

a) Redefine and standardise disease definitions and diagnosis

Strongly Agree

b) Redefine and harmonise clinical endpoints and outcomes

Agree

c) Develop new biomarkers

Strongly Agree

d) Consider regulatory approaches

Agree

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

b) Linking to treatment responses

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points

a) Improve access to, and sharing of, infrastructure and resources

Agree

b) Improve access to patient groups, samples and data

Strongly Agree

c) Improve data and sample collection

Strongly Agree

d) Develop a register of persons with cognitive impairment

Agree

e) Develop centres of excellence

Strongly Agree

Question 2: Please rank the suggestions in order of priority

First Priority

b) Improve access to patient groups, samples and data

Second Priority

e) Develop centres of excellence

Third Priority

a) Improve access to, and sharing of, infrastructure and resources

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

Sharing of clinical trial data to help improve future clinical trial design

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

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

Depends how they will be organised and funded eg by expertise already existing in different countries How would they be coordinated and ensure common goals/strategy to address needs in NDD Need to avoid over complicated administrative process

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points

a) Need for evidence-based policy

Agree

b) Ensure greater engagement with regulators

Strongly Agree

c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community

Agree

d) Encourage industry to adopt a pre-competitive approach to research

Strongly Agree

e) Rethink patent lifetime and conduct public-private clinical trials

Strongly Agree

f) Review and update legislation on treatment

Strongly Agree

g) Review and update legislation on privacy and data disclosure

Agree

Question 2: Please rank the suggestions in order of priority

First Priority

b) Ensure greater engagement with regulators

Second Priority

e) Rethink patent lifetime and conduct public-private clinical trials

Third Priority

d) Encourage industry to adopt pre-competitive approach

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points

a) Translational research needs to be promoted

Strongly Agree

b) Encourage open-access sharing of data and materials

Agree

c) Joint academic-industry funding models

Strongly Agree

d) Simplify funding application systems

Strongly Agree

e) Maintain capacity for 'bottom-up' innovative funding

Strongly Agree

Question 2: Please rank the suggestions in order of priority

First Priority

a) Translational research needs to be promoted

Second Priority

c) Joint academic-industry funding models

Third Priority

e) Maintain capacity for 'bottom-up' innovative funding

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

Concluding questions

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?

UID
1922
Title
Mr
First Name
Gareth
Surname
Maher-Edwards
Category of respondee
Commercial researcher - Pharmaceutical
Institution/Organisation/Company
GlaxoSmithKline
Position
Director, Clinical Development
Replying on behalf of organisation/company?
On behalf of my company
Country
United Kingdom

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Agree
b) Improve education and training of healthcare professionals
Neutral
c) Increase numbers of neurodegenerative disease (NDD) researchers
Agree
d) Increase training for translational and clinician-scientists
Agree
e) Increase numbers of post-doctoral level researchers
Agree

Question 2: If you had to choose one priority from the points above what would it be?
d) Increase training for translational and clinician-scientists

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
Agree
b) Survey long-term care standards and provision across Europe
Agree
c) Research into the needs of carers
Strongly Agree
d) Research into care approaches including end of life decision-making
Neutral
e) Rethink approaches to care
Neutral
f) Understand and investigate influence of comorbidities
Agree
g) Conduct research into effects of nutrition and frailty
Agree
h) Determine cost-effectiveness of healthcare pathways
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
c) Research into the needs of carers
Second Priority
h) Determine cost-effectiveness of healthcare pathways
Third Priority
b) Survey long-term care standards and provision across Europe

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 has both advantages and disadvantages, how do you suggest we change the balance to promote the advantages?

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Neutral
b) Promote development of non-pharmacological interventions
Neutral
c) Conduct multi-centre primary prevention studies
Agree
d) Ensure better patient selection/stratification
Agree
e) Rethink approach to therapeutics
Strongly Agree
f) Support for high-risk projects
Neutral

Question 2: Please rank the suggestions in order of priority
First Priority
c) Conduct multi-centre primary prevention studies
Second Priority
d) Ensure better patient selection/stratification
Third Priority
e) Rethink approach to therapeutics

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?

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
Agree
b) Improve understanding of disease stages
Strongly Agree
c) Improve understanding of disease mechanisms
Strongly Agree
d) Develop an improved understanding of the genetic basis for NDD
Strongly Agree
e) Determine the importance of genetic and environmental risk factors
Strongly Agree
f) Focus research on rare hereditary forms of disease
Neutral
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Agree
h) Develop more representative animal and cell-based models of disease
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Improve understanding of disease stages
Second Priority
c) Improve understanding of disease mechanisms
Third Priority
h) Develop more representative animal and cell-based models

Question 3: In relation to disease mechanisms which of the following do you think it is most important to investigate?
c) Mechanisms of neuronal death and dysfunction

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

Question 1: Please indicate to what extent you endorse the following points
a) Redefine and standardise disease definitions and diagnosis
Agree
b) Redefine and harmonise clinical endpoints and outcomes
Agree
c) Develop new biomarkers
Agree
d) Consider regulatory approaches
Agree

Question 2: Which of the following do you think is most important in terms of biomarkers?
c) Providing an indicator of, and sensitivity to, disease progression

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points
a) Improve access to, and sharing of, infrastructure and resources
Agree
b) Improve access to patient groups, samples and data
Agree
c) Improve data and sample collection
Agree
d) Develop a register of persons with cognitive impairment
Agree
e) Develop centres of excellence
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Improve access to, and sharing of, infrastructure and resources
Second Priority
b) Improve access to patient groups, samples and data
Third Priority
e) Develop centres of excellence

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 forsee difficulties, how can we overcome these?

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

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
Agree
b) Ensure greater engagement with regulators
Strongly Agree
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community
Agree
d) Encourage industry to adopt a pre-competitive approach to research
Neutral
e) Rethink patent lifetime and conduct public-private clinical trials
Agree
f) Review and update legislation on treatment
Agree
g) Review and update legislation on privacy and data disclosure
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Ensure greater engagement with regulators
Second Priority
Third Priority

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points
a) Translational research needs to be promoted
Strongly Agree
b) Encourage open-access sharing of data and materials
Agree
c) Joint academic-industry funding models
Agree
d) Simplify funding application systems
Neutral
e) Maintain capacity for 'bottom-up' innovative funding
Neutral

Question 2: Please rank the suggestions in order of priority
First Priority
b) Encourage open-access sharing of data and materials
Second Priority
a) Translational research needs to be promoted
Third Priority

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

Concluding questions

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?

UID
2097
Title
Ms
First Name
Catherine
Surname
Bates
Category of respondee
Commercial researcher - Biotechnology
Institution/Organisation/Company
MERCK SERONO
Position
Manager, Patient Advocacy
Replying on behalf of organisation/company?
On behalf of my company
Country
Switzerland

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Agree
b) Improve education and training of healthcare professionals
Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Neutral
d) Increase training for translational and clinician-scientists
Agree
e) Increase numbers of post-doctoral level researchers
Agree

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?
Medicinal chemistry

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
Strongly Agree
b) Survey long-term care standards and provision across Europe
Strongly Agree
c) Research into the needs of carers
Strongly Agree
d) Research into care approaches including end of life decision-making
Agree
e) Rethink approaches to care
Agree
f) Understand and investigate influence of comorbidities
Strongly Agree
g) Conduct research into effects of nutrition and frailty
Neutral
h) Determine cost-effectiveness of healthcare pathways
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
c) Research into the needs of carers
Second Priority
f) Understand and investigate influence of comorbidities on NDD
Third Priority
b) Survey long-term care standards and provision across Europe

Question 3: How would you define "care"?
Many people with Parkinson's believe 'gold standard care' should involve a multidisciplinary team with care tailored to their specific needs and those of their family and carers. Such multidisciplinary treatment may involve a number of allied health specialists, including physiotherapists, occupational therapists and speech and

language therapists as well as support and advice from dieticians, social workers and sexologists to complement standard medical treatment in the management of both motor and non-motor symptoms. While the neurologist determines disease severity and optimises medical treatment to reduce symptoms, allied health therapists work to minimise the impact of the disease process and improve the person's participation in everyday activities. (Source: EPDA)

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

Pursue integrated approaches to healthcare including research, treatment and funding (i.e. to overcome challenges resulting from silos in government health care budgets)

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

Patients prefer to receive care at home. Access to technologies that facilitate home care should be promoted.

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points

a) Increase involvement of individuals in research

Strongly Agree

b) Promote development of non-pharmacological interventions

Strongly Agree

c) Conduct multi-centre primary prevention studies

Strongly Agree

d) Ensure better patient selection/stratification

Neutral

e) Rethink approach to therapeutics

Strongly Agree

f) Support for high-risk projects

Strongly Agree

Question 2: Please rank the suggestions in order of priority

First Priority

b) Promote development of non-pharmacological interventions

Second Priority

d) Ensure better patient selection/stratification

Third Priority

a) Increase involvement of individuals in research

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

-Currently, enrolling patients in clinical trials is complicated. Reducing the burden of time-consuming processes could help in this regard. -Changing the default option on donor cards such that one can donate more easily might also be an option.

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?

Anti-amyloid strategies in Alzheimer's Anti-inflammatory strategies in AD, PD, possibly others

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points

a) Understand relationship between neurodegenerative disease and ageing

Agree

b) Improve understanding of disease stages

Strongly Agree

c) Improve understanding of disease mechanisms

Agree

d) Develop an improved understanding of the genetic basis for NDD

Agree

e) Determine the importance of genetic and environmental risk factors

Agree

f) Focus research on rare hereditary forms of disease

Neutral

g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups

Neutral

h) Develop more representative animal and cell-based models of disease

Agree

Question 2: Please rank the suggestions in order of priority

First Priority

b) Improve understanding of disease stages

Second Priority

h) Develop more representative animal and cell-based models

Third Priority

a) Understand relationship between NDD and ageing

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

c) Mechanisms of neuronal death and dysfunction

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

Probably not but needs careful case-by-case consideration. We do not have great animal models that encompass all the prominent aspects of many neurodegenerative diseases, and we must not let that prevent trials. However the animal model to be used may look at only a narrow and very specific biochemical effect (e.g. reduction in alpha synuclein, altered subcellular localization of a marker, etc.).

Diagnosis, disease definitions and outcome measures

Question 1: Please indicate to what extent you endorse the following points

a) Redefine and standardise disease definitions and diagnosis

Strongly Agree

b) Redefine and harmonise clinical endpoints and outcomes

Agree

c) Develop new biomarkers

Strongly Agree

d) Consider regulatory approaches

Strongly Agree

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

b) Linking to treatment responses

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points

a) Improve access to, and sharing of, infrastructure and resources

Strongly Agree

b) Improve access to patient groups, samples and data

Strongly Agree

c) Improve data and sample collection

Strongly Agree

d) Develop a register of persons with cognitive impairment

Disagree

e) Develop centres of excellence

Strongly Agree

Question 2: Please rank the suggestions in order of priority

First Priority

b) Improve access to patient groups, samples and data

Second Priority

e) Develop centres of excellence

Third Priority

c) Improve data and sample collection

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

-Current restrictions could be changed -Sharing of data could be incentivized

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

Definitely difficult. - Competition is a marked feature of the current system and a powerful driver for creativity and quality; in many cases data sharing is incompatible with such competition. - IP issues also are often the driver for maintaining strict confidentiality and would have to be addressed in a way to reduce the risk of certain types of disclosures. - Guidelines, such as those governing posting of clinical trials on ClinicalTrials.Gov, but more specific, could be developed and adopted, to define clearly the expectations and best practices for data sharing.

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

Developing centres of excellence by integrating current activities in a given city, London or Tuebingen for example, would probably be a win-win. Taking resources away from smaller centres to build up a few big ones would be win-lose and might result in average quality groups taking resource away from excellent groups not part of larger centres.

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
Neutral
b) Ensure greater engagement with regulators
Strongly Agree
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community
Strongly Agree
d) Encourage industry to adopt a pre-competitive approach to research
Strongly Agree
e) Rethink patent lifetime and conduct public-private clinical trials
Strongly Agree
f) Review and update legislation on treatment
Strongly Agree
g) Review and update legislation on privacy and data disclosure
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
e) Rethink patent lifetime and conduct public-private clinical trials
Second Priority
a) Need for evidence-based policy
Third Priority
b) Ensure greater engagement with regulators

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?
Expand IP capabilities for the protection of research data- Promote risk-Sharing agreements and other new approaches to fair-pricing of medicines to encourage innovation -Promote NGO partnerships -encourage global collaboration with the US and China.

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points
a) Translational research needs to be promoted
Strongly Agree
b) Encourage open-access sharing of data and materials
Strongly Agree
c) Joint academic-industry funding models
Agree
d) Simplify funding application systems
Agree
e) Maintain capacity for 'bottom-up' innovative funding
Disagree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Translational research needs to be promoted
Second Priority
b) Encourage open-access sharing of data and materials
Third Priority
c) Joint academic-industry funding models

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

Concluding questions

Question 1: Are there strategies you think we have overlooked, if so please suggest below (up to three suggestions)
No

Question 2: Do you have any comments on how to implement the above suggestion(s)?
No

Question 3: Is there anything else you would like to tell us?
Increasing opportunities for training must be carefully balanced with the opportunities for long-term careers that are available. Is it acceptable to ask students to commit to 7-10 years of postgraduate training to have at the end a 25%(?) chance of a sustained career in the field? It would be valuable to develop a communication

package highlighting the progress that medical research has achieved in the NDD area, not just the large unmet need.

UID
2149
Title
Dr
First Name
Wally
Surname
Landsberg
Category of respondee
Commercial researcher - Pharmaceutical
Institution/Organisation/Company
Bristol Myers Squibb
Position
European Medical Lead Neuroscience
Replying on behalf of organisation/company?
On behalf of my company
Country
France

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Strongly Agree
b) Improve education and training of healthcare professionals
Strongly Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Strongly Agree
d) Increase training for translational and clinician-scientists
Agree
e) Increase numbers of post-doctoral level researchers
Agree

Question 2: If you had to choose one priority from the points above what would it be?
b) Improve education and training of healthcare professionals

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?
Need to establish stronger academic-industry collaboration in the ND R&D space to bring treatments to the market faster

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
Strongly Agree
b) Survey long-term care standards and provision across Europe
Strongly Agree
c) Research into the needs of carers
Strongly Agree
d) Research into care approaches including end of life decision-making
Agree
e) Rethink approaches to care
Strongly Agree
f) Understand and investigate influence of comorbidities
Agree
g) Conduct research into effects of nutrition and frailty
Agree
h) Determine cost-effectiveness of healthcare pathways
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Survey long-term care standards and provision across Europe
Second Priority
e) Rethink approaches to care
Third Priority
h) Determine cost-effectiveness of healthcare pathways

Question 3: How would you define "care"?

To optimally address the needs of the patient and his/her support system (social ,family ,health) during all stages of the disease and especially during key decision points.

Question 4: Have you any (additional) suggestions as to how care systems should be revised?
Minimum desired and acceptable care levels should be standardised and communicated in conjunction with what optimal care should look like.

Question 5: Provision of care at home has both advantages and disadvantages, how do you suggest we change the balance to promote the advantages?
Provide training and incentivise carers at home .Also provide sufficient professional support to carers.

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Strongly Agree
b) Promote development of non-pharmacological interventions
Agree
c) Conduct multi-centre primary prevention studies
Agree
d) Ensure better patient selection/stratification
Strongly Agree
e) Rethink approach to therapeutics
Strongly Agree
f) Support for high-risk projects
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Increase involvement of individuals in research
Second Priority
d) Ensure better patient selection/stratification
Third Priority
e) Rethink approach to therapeutics

Question 3: How can we encourage more people to take part in research and/or register to donate brain material?
Better educate people on the unmet needs in research and how their participation could support science and ultimately patient care.

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?
nutrition ,education , geography , co- morbid medical conditions and treatment for those ,co-morbid psychiatric conditions ,genetics, age, gender, environmental factors

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
Agree
b) Improve understanding of disease stages
Agree
c) Improve understanding of disease mechanisms
Strongly Agree
d) Develop an improved understanding of the genetic basis for NDD
Strongly Agree
e) Determine the importance of genetic and environmental risk factors
Strongly Agree
f) Focus research on rare hereditary forms of disease
Disagree
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Strongly Agree
h) Develop more representative animal and cell-based models of disease
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
c) Improve understanding of disease mechanisms
Second Priority
h) Develop more representative animal and cell-based models
Third Priority

d) Develop an improved understanding of the genetic basis

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

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
Not sure if we should bypass the phase of testing in animal models.. in fact the effort should be able to come up with better animal models that would relate more closely to the effect one could expect to see in humans .

Diagnosis, disease definitions and outcome measures

Question 1: Please indicate to what extent you endorse the following points
a) Redefine and standardise disease definitions and diagnosis
Strongly Agree
b) Redefine and harmonise clinical endpoints and outcomes
Strongly Agree
c) Develop new biomarkers
Strongly Agree
d) Consider regulatory approaches
Disagree

Question 2: Which of the following do you think is most important in terms of biomarkers?
b) Linking to treatment responses

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points
a) Improve access to, and sharing of, infrastructure and resources
Agree
b) Improve access to patient groups, samples and data
Strongly Agree
c) Improve data and sample collection
Agree
d) Develop a register of persons with cognitive impairment
Agree
e) Develop centres of excellence
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Improve access to patient groups, samples and data
Second Priority
e) Develop centres of excellence
Third Priority
c) Improve data and sample collection

Question 3: What can be done to facilitate increased sharing of data?
Improved communication and building of trust between academia and industry.

Question 4: What are your views on making data open access? If you forsee difficulties, how can we overcome these?
It is a good idea in principle but clear ethical /financial rules will need to be agreed upon by anyone who would like to make use of the open data.The legal aspects of data sharing will also need to be addressed.

Question 5: Relating to point (e) do you see any risk(s) in developing centres of excellence?
Centres of excellence may attract all funding which leaves smaller centres/ researchers potentially without support. But if you want to look at it from a positive standpoint.. COEs can be the central point for a well established multidisciplinary care system where smaller institutions are an integral part of the network .

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
Strongly Agree
b) Ensure greater engagement with regulators
Strongly Agree
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community

Agree

d) Encourage industry to adopt a pre-competitive approach to research

Strongly Agree

e) Rethink patent lifetime and conduct public-private clinical trials

Strongly Agree

f) Review and update legislation on treatment

Strongly Agree

g) Review and update legislation on privacy and data disclosure

Agree

Question 2: Please rank the suggestions in order of priority

First Priority

b) Ensure greater engagement with regulators

Second Priority

d) Encourage industry to adopt pre-competitive approach

Third Priority

e) Rethink patent lifetime and conduct public-private clinical trials

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Engage payers/policy makers in discussions around need for data generation and disease strategy partnerships early on.

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points

a) Translational research needs to be promoted

Agree

b) Encourage open-access sharing of data and materials

Agree

c) Joint academic-industry funding models

Strongly Agree

d) Simplify funding application systems

Strongly Agree

e) Maintain capacity for 'bottom-up' innovative funding

Strongly Agree

Question 2: Please rank the suggestions in order of priority

First Priority

a) Translational research needs to be promoted

Second Priority

c) Joint academic-industry funding models

Third Priority

b) Encourage open-access sharing of data and materials

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

Concluding questions

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?

UID
1895
Category of respondee
Commercial researcher - Pharmaceutical
Country
France
Replying on behalf of organisation/company?
On behalf of my company

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Strongly Agree
b) Improve education and training of healthcare professionals
Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Agree
d) Increase training for translational and clinician-scientists
Strongly Agree
e) Increase numbers of post-doctoral level researchers
Agree

Question 2: If you had to choose one priority from the points above what would it be?
a) Improve dialogue between researchers and wider population

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
Disagree
b) Survey long-term care standards and provision across Europe
Agree
c) Research into the needs of carers
Neutral
d) Research into care approaches including end of life decision-making
Agree
e) Rethink approaches to care
Agree
f) Understand and investigate influence of comorbidities
Neutral
g) Conduct research into effects of nutrition and frailty
Agree
h) Determine cost-effectiveness of healthcare pathways
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Survey long-term care standards and provision across Europe
Second Priority
h) Determine cost-effectiveness of healthcare pathways
Third Priority
e) Rethink approaches to care

Question 3: How would you define "care"?
all processes involved in the health care management of one patient , including relatives, the medical personnel, facilities, medical ressources, ...

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

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

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Agree

b) Promote development of non-pharmacological interventions
Agree
c) Conduct multi-centre primary prevention studies
Agree
d) Ensure better patient selection/stratification
Strongly Agree
e) Rethink approach to therapeutics
Agree
f) Support for high-risk projects
Neutral

Question 2: Please rank the suggestions in order of priority
First Priority
a) Increase involvement of individuals in research
Second Priority
d) Ensure better patient selection/stratification
Third Priority
e) Rethink approach to therapeutics

Question 3: How can we encourage more people to take part in research and/or register to donate brain material?
drive a transparent, objective, well balanced communication around clinical research

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?

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
Agree
b) Improve understanding of disease stages
Agree
c) Improve understanding of disease mechanisms
Agree
d) Develop an improved understanding of the genetic basis for NDD
Agree
e) Determine the importance of genetic and environmental risk factors
Agree
f) Focus research on rare hereditary forms of disease
Neutral
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Neutral
h) Develop more representative animal and cell-based models of disease
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Understand relationship between NDD and ageing
Second Priority
c) Improve understanding of disease mechanisms
Third Priority
h) Develop more representative animal and cell-based models

Question 3: In relation to disease mechanisms which of the following do you think it is most important to investigate?
c) Mechanisms of neuronal death and dysfunction

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

Question 1: Please indicate to what extent you endorse the following points
a) Redefine and standardise disease definitions and diagnosis
Agree
b) Redefine and harmonise clinical endpoints and outcomes
Strongly Agree
c) Develop new biomarkers
Agree

d) Consider regulatory approaches
Agree

Question 2: Which of the following do you think is most important in terms of biomarkers?
b) Linking to treatment responses

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points
a) Improve access to, and sharing of, infrastructure and resources
Agree
b) Improve access to patient groups, samples and data
Strongly Agree
c) Improve data and sample collection
Strongly Agree
d) Develop a register of persons with cognitive impairment
Strongly Agree
e) Develop centres of excellence
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Improve access to patient groups, samples and data
Second Priority
e) Develop centres of excellence
Third Priority
d) Develop a register of persons with cognitive impairment

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 forsee difficulties, how can we overcome these?

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

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
b) Ensure greater engagement with regulators
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community
d) Encourage industry to adopt a pre-competitive approach to research
e) Rethink patent lifetime and conduct public-private clinical trials
f) Review and update legislation on treatment
g) Review and update legislation on privacy and data disclosure

Question 2: Please rank the suggestions in order of priority
First Priority
Second Priority
Third Priority

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points
a) Translational research needs to be promoted
b) Encourage open-access sharing of data and materials
c) Joint academic-industry funding models
d) Simplify funding application systems
e) Maintain capacity for 'bottom-up' innovative funding

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 that may help us achieve this goal

Concluding questions

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?

UID
1973
Category of respondee
Commercial researcher - Pharmaceutical
Country
Hungary
Replying on behalf of organisation/company?
On behalf of my company

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Strongly Agree
b) Improve education and training of healthcare professionals
Strongly Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Agree
d) Increase training for translational and clinician-scientists
Strongly Agree
e) Increase numbers of post-doctoral level researchers
Agree

Question 2: If you had to choose one priority from the points above what would it be?
a) Improve dialogue between researchers and wider population

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
b) Survey long-term care standards and provision across Europe
c) Research into the needs of carers
d) Research into care approaches including end of life decision-making
e) Rethink approaches to care
f) Understand and investigate influence of comorbidities
g) Conduct research into effects of nutrition and frailty
h) Determine cost-effectiveness of healthcare pathways

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 has both advantages and disadvantages, how do you suggest we change the balance to promote the advantages?

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Agree
b) Promote development of non-pharmacological interventions
Agree
c) Conduct multi-centre primary prevention studies
Strongly Agree
d) Ensure better patient selection/stratification
Strongly Agree
e) Rethink approach to therapeutics
Agree
f) Support for high-risk projects
Neutral

Question 2: Please rank the suggestions in order of priority
First Priority

c) Conduct multi-centre primary prevention studies

Second Priority

d) Ensure better patient selection/stratification

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?

Long-term monitoring of biomerkers affected by neurodegenerative diseases would be costly and difficult in clinical studies trying preventive measures. Such studies should start with healthy people and last for years. It seems feasible only in the case of specific population of subjects with strong genetic load. Therefore pre-selection of the subjects would be very important. I can imagine that results of long-term open studies involving a large patient population on the effect of physical or mental training, diet, or preventive use of pharmacons could be useful. Providing an appropriate control over such studies would be obligate.

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points

a) Understand relationship between neurodegenerative disease and ageing

Strongly Agree

b) Improve understanding of disease stages

Strongly Agree

c) Improve understanding of disease mechanisms

Strongly Agree

d) Develop an improved understanding of the genetic basis for NDD

Strongly Agree

e) Determine the importance of genetic and environmental risk factors

Strongly Agree

f) Focus research on rare hereditary forms of disease

Neutral

g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups

Neutral

h) Develop more representative animal and cell-based models of disease

Strongly Agree

Question 2: Please rank the suggestions in order of priority

First Priority

c) Improve understanding of disease mechanisms

Second Priority

a) Understand relationship between NDD and ageing

Third Priority

h) Develop more representative animal and cell-based models

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

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

Stopping an ongoing treatment - even if it is not ideal - may cause irreversible worthening of the state of the patient if the alternative therapy is ineffective. Some therapies could be perhaps tried on an add-on basis. Safety studies would be inevitable, of course. For most conditions, however, there are easily available animal models, thus we could not gain very much from leaving out animal tests. The real problem is that a preclinical proof of concept does not guarantee the clinical success. Therefore we should improve the predictivity of our animal models.

Diagnosis, disease definitions and outcome measures

Question 1: Please indicate to what extent you endorse the following points

a) Redefine and standardise disease definitions and diagnosis

Strongly Agree

b) Redefine and harmonise clinical endpoints and outcomes

Agree

c) Develop new biomarkers

Strongly Agree

d) Consider regulatory approaches

Neutral

Question 2: Which of the following do you think is most important in terms of biomarkers?
c) Providing an indicator of, and sensitivity to, disease progression

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points
a) Improve access to, and sharing of, infrastructure and resources
Agree
b) Improve access to patient groups, samples and data
Strongly Agree
c) Improve data and sample collection
Agree
d) Develop a register of persons with cognitive impairment
Disagree
e) Develop centres of excellence
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
e) Develop centres of excellence
Second Priority
b) Improve access to patient groups, samples and data
Third Priority

Question 3: What can be done to facilitate increased sharing of data?
To create more publicly accessible data bases.

Question 4: What are your views on making data open access? If you forsee difficulties, how can we overcome these?
Intellectual property issues considerably hinder making data open access. The regulations around IP rights needs to be changed.

Question 5: Relating to point (e) do you see any risk(s) in developing centres of excellence?
No

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
b) Ensure greater engagement with regulators
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community
d) Encourage industry to adopt a pre-competitive approach to research
e) Rethink patent lifetime and conduct public-private clinical trials
f) Review and update legislation on treatment
g) Review and update legislation on privacy and data disclosure

Question 2: Please rank the suggestions in order of priority
First Priority
Second Priority
Third Priority

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points
a) Translational research needs to be promoted
b) Encourage open-access sharing of data and materials
c) Joint academic-industry funding models
d) Simplify funding application systems
e) Maintain capacity for 'bottom-up' innovative funding

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 that may help us achieve this goal

Concluding questions

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?

UID
2013
Category of respondee
Commercial researcher - Imaging
Country
Belgium
Replying on behalf of organisation/company?
On behalf of my company

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Agree
b) Improve education and training of healthcare professionals
Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Neutral
d) Increase training for translational and clinician-scientists
Agree
e) Increase numbers of post-doctoral level researchers
Agree

Question 2: If you had to choose one priority from the points above what would it be?
d) Increase training for translational and clinician-scientists

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?
Multidisciplinary collaboration (eg medical / image processing), both in research and clinical practice

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
b) Survey long-term care standards and provision across Europe
c) Research into the needs of carers
d) Research into care approaches including end of life decision-making
Strongly Agree
e) Rethink approaches to care
Strongly Agree
f) Understand and investigate influence of comorbidities
g) Conduct research into effects of nutrition and frailty
Strongly Agree
h) Determine cost-effectiveness of healthcare pathways
Strongly Agree

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 has both advantages and disadvantages, how do you suggest we change the balance to promote the advantages?

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Neutral
b) Promote development of non-pharmacological interventions
Strongly Agree
c) Conduct multi-centre primary prevention studies
d) Ensure better patient selection/stratification
Strongly Agree
e) Rethink approach to therapeutics
Strongly Agree
f) Support for high-risk projects

Agree

Question 2: Please rank the suggestions in order of priority
First Priority
d) Ensure better patient selection/stratification
Second Priority
b) Promote development of non-pharmacological interventions
Third Priority
c) Conduct multi-centre primary prevention studies

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?

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
Agree
b) Improve understanding of disease stages
Agree
c) Improve understanding of disease mechanisms
Agree
d) Develop an improved understanding of the genetic basis for NDD
Agree
e) Determine the importance of genetic and environmental risk factors
Agree
f) Focus research on rare hereditary forms of disease
Agree
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Strongly Agree
h) Develop more representative animal and cell-based models of disease
Disagree

Question 2: Please rank the suggestions in order of priority
First Priority
g) Establish pan-European population-based studies
Second Priority
a) Understand relationship between NDD and ageing
Third Priority
b) Improve understanding of disease stages

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

Question 1: Please indicate to what extent you endorse the following points
a) Redefine and standardise disease definitions and diagnosis
Agree
b) Redefine and harmonise clinical endpoints and outcomes
Strongly Agree
c) Develop new biomarkers
Strongly Agree
d) Consider regulatory approaches
Strongly Agree

Question 2: Which of the following do you think is most important in terms of biomarkers?
c) Providing an indicator of, and sensitivity to, disease progression

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points

a) Improve access to, and sharing of, infrastructure and resources
Agree
b) Improve access to patient groups, samples and data
Strongly Agree
c) Improve data and sample collection
Strongly Agree
d) Develop a register of persons with cognitive impairment
Disagree
e) Develop centres of excellence
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Improve access to patient groups, samples and data
Second Priority
a) Improve access to, and sharing of, infrastructure and resources
Third Priority
e) Develop centres of excellence

Question 3: What can be done to facilitate increased sharing of data?
cfr. ADNI initiative, open databases of clinical trials (to start maybe only placebo part), clarify and change legislation

Question 4: What are your views on making data open access? If you forsee difficulties, how can we overcome these?
promote the idea of data sharing in both industry and academia: eg by promoting/forcing researchers to open their data simultaneous with the publication.

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

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
Strongly Agree
b) Ensure greater engagement with regulators
Strongly Agree
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community
Strongly Agree
d) Encourage industry to adopt a pre-competitive approach to research
Strongly Agree
e) Rethink patent lifetime and conduct public-private clinical trials
Disagree
f) Review and update legislation on treatment
Agree
g) Review and update legislation on privacy and data disclosure
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Need for evidence-based policy
Second Priority
d) Encourage industry to adopt pre-competitive approach
Third Priority
b) Ensure greater engagement with regulators

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points
a) Translational research needs to be promoted
Strongly Agree
b) Encourage open-access sharing of data and materials
Strongly Agree
c) Joint academic-industry funding models
Strongly Agree
d) Simplify funding application systems
Strongly Agree
e) Maintain capacity for 'bottom-up' innovative funding

Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Encourage open-access sharing of data and materials
Second Priority
c) Joint academic-industry funding models
Third Priority
a) Translational research needs to be promoted

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

Concluding questions

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?

UID
2041
Category of respondee
Commercial researcher - Pharmaceutical
Country
Denmark
Replying on behalf of organisation/company?
On behalf of my company

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Strongly Agree
b) Improve education and training of healthcare professionals
Strongly Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Strongly Agree
d) Increase training for translational and clinician-scientists
Strongly Agree
e) Increase numbers of post-doctoral level researchers
Strongly Agree

Question 2: If you had to choose one priority from the points above what would it be?
c) Increase overall numbers of researchers

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
Agree
b) Survey long-term care standards and provision across Europe
Agree
c) Research into the needs of carers
Neutral
d) Research into care approaches including end of life decision-making
Neutral
e) Rethink approaches to care
Agree
f) Understand and investigate influence of comorbidities
Strongly Agree
g) Conduct research into effects of nutrition and frailty
Neutral
h) Determine cost-effectiveness of healthcare pathways
Neutral

Question 2: Please rank the suggestions in order of priority
First Priority
e) Rethink approaches to care
Second Priority
f) Understand and investigate influence of comorbidities on NDD
Third Priority

Question 3: How would you define "care"?
I agree with Heidegger..

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

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

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Strongly Agree
b) Promote development of non-pharmacological interventions
Disagree

c) Conduct multi-centre primary prevention studies
Neutral
d) Ensure better patient selection/stratification
Strongly Agree
e) Rethink approach to therapeutics
f) Support for high-risk projects
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Increase involvement of individuals in research
Second Priority
d) Ensure better patient selection/stratification
Third Priority

Question 3: How can we encourage more people to take part in research and/or register to donate brain material?
Harmonise donor programs across countries and clinical centers in Europe.

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?
Nutrition and Alzheimers disease Exercise and Alzheimers disease Poly pills and Alzheimers disease

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
b) Improve understanding of disease stages
Agree
c) Improve understanding of disease mechanisms
Strongly Agree
d) Develop an improved understanding of the genetic basis for NDD
Neutral
e) Determine the importance of genetic and environmental risk factors
Neutral
f) Focus research on rare hereditary forms of disease
Strongly Agree
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Strongly Agree
h) Develop more representative animal and cell-based models of disease
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
c) Improve understanding of disease mechanisms
Second Priority
h) Develop more representative animal and cell-based models
Third Priority
f) Focus research on rare hereditary forms of disease

Question 3: In relation to disease mechanisms which of the following do you think it is most important to investigate?
c) Mechanisms of neuronal death and dysfunction

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
1) Good mechanistic rationale and in-vitro proof-of-mechanism. 2) Safety and Tox package in place. 3) Clear endpoint defined in clinical population + mechanistic endpoint (biomarker) for efficacy.

Diagnosis, disease definitions and outcome measures

Question 1: Please indicate to what extent you endorse the following points
a) Redefine and standardise disease definitions and diagnosis
Strongly Agree
b) Redefine and harmonise clinical endpoints and outcomes
Strongly Agree
c) Develop new biomarkers
Strongly Agree
d) Consider regulatory approaches
Strongly Disagree

Question 2: Which of the following do you think is most important in terms of biomarkers?
b) Linking to treatment responses

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points
a) Improve access to, and sharing of, infrastructure and resources
Strongly Agree
b) Improve access to patient groups, samples and data
Strongly Agree
c) Improve data and sample collection
Strongly Agree
d) Develop a register of persons with cognitive impairment
Strongly Agree
e) Develop centres of excellence
Neutral

Question 2: Please rank the suggestions in order of priority
First Priority
a) Improve access to, and sharing of, infrastructure and resources
Second Priority
c) Improve data and sample collection
Third Priority
b) Improve access to patient groups, samples and data

Question 3: What can be done to facilitate increased sharing of data?
Open access license to cookie-jar resources such as transgenic animals, cell lines, primary imortalised cells from well defined patient populations etc.

Question 4: What are your views on making data open access? If you forsee difficulties, how can we overcome these?
Make a clear open access license text. One could discuss whether a central repository of resources should be maintained, but this can easily become a costly exercise in ''stamp collection''. So contributer of specific resource should be willing to give out material at a specific cost.

Question 5: Relating to point (e) do you see any risk(s) in developing centres of excellence?
Yes - difficult to change direction of research - centers of excellence often become inflexible 'old boys networks'. Research within neurodegeneration should be driven by the need for novel therapies. There is ofcourse a need for basic research. Not sure this is a JPND issue.

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
Agree
b) Ensure greater engagement with regulators
Strongly Agree
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community
Neutral
d) Encourage industry to adopt a pre-competitive approach to research
Agree
e) Rethink patent lifetime and conduct public-private clinical trials
Neutral
f) Review and update legislation on treatment
Neutral
g) Review and update legislation on privacy and data disclosure
Neutral

Question 2: Please rank the suggestions in order of priority
First Priority
b) Ensure greater engagement with regulators
Second Priority
c) Facilitate research in areas outside university and hospital sectors
Third Priority

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points
a) Translational research needs to be promoted
Strongly Agree
b) Encourage open-access sharing of data and materials
Strongly Agree
c) Joint academic-industry funding models
Strongly Agree
d) Simplify funding application systems
Strongly Agree
e) Maintain capacity for 'bottom-up' innovative funding
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Translational research needs to be promoted
Second Priority
c) Joint academic-industry funding models
Third Priority
e) Maintain capacity for 'bottom-up' innovative funding

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

Concluding questions

Question 1: Are there strategies you think we have overlooked, if so please suggest below (up to three suggestions)
JPND should encurage funding of novel innovative ideas rather than funding more of ''the same'' stuff.

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?

UID
1736
Title
Dr
First Name
Arjen
Surname
Slooter
Category of respondee
Commercial researcher
Company
University Medical Centre Utrecht
Country
Netherlands
Replying on behalf of organisation/company?
As a private individual

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Neutral
b) Improve education and training of healthcare professionals
Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Agree
d) Increase training for translational and clinician-scientists
Agree
e) Increase numbers of post-doctoral level researchers
Agree

Question 2: If you had to choose one priority from the points above what would it be?
c) Increase overall numbers of researchers

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?
Delirium, in particular delirium in critically ill patients

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
Agree
b) Survey long-term care standards and provision across Europe
Agree
c) Research into the needs of carers
Agree
d) Research into care approaches including end of life decision-making
Agree
e) Rethink approaches to care
Agree
f) Understand and investigate influence of comorbidities
Strongly Agree
g) Conduct research into effects of nutrition and frailty
Strongly Agree
h) Determine cost-effectiveness of healthcare pathways
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
g) Conduct research into effects of nutrition and frailty
Second Priority
f) Understand and investigate influence of comorbidities on NDD
Third Priority
h) Determine cost-effectiveness of healthcare pathways

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 has both advantages and disadvantages, how do you suggest we change the balance to promote the advantages?

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Agree
b) Promote development of non-pharmacological interventions
Agree
c) Conduct multi-centre primary prevention studies
Agree
d) Ensure better patient selection/stratification
Agree
e) Rethink approach to therapeutics
Agree
f) Support for high-risk projects
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Increase involvement of individuals in research
Second Priority
c) Conduct multi-centre primary prevention studies
Third Priority
e) Rethink approach to therapeutics

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?

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
Agree
b) Improve understanding of disease stages
Agree
c) Improve understanding of disease mechanisms
Agree
d) Develop an improved understanding of the genetic basis for NDD
Agree
e) Determine the importance of genetic and environmental risk factors
Agree
f) Focus research on rare hereditary forms of disease
Disagree
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Agree
h) Develop more representative animal and cell-based models of disease
Agree

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?
c) Mechanisms of neuronal death and dysfunction

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

Question 1: Please indicate to what extent you endorse the following points
a) Redefine and standardise disease definitions and diagnosis

Agree

b) Redefine and harmonise clinical endpoints and outcomes

Agree

c) Develop new biomarkers

Agree

d) Consider regulatory approaches

Agree

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

b) Linking to treatment responses

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points

a) Improve access to, and sharing of, infrastructure and resources

Strongly Agree

b) Improve access to patient groups, samples and data

Agree

c) Improve data and sample collection

Agree

d) Develop a register of persons with cognitive impairment

Agree

e) Develop centres of excellence

Agree

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 forsee difficulties, how can we overcome these?

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

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points

a) Need for evidence-based policy

Agree

b) Ensure greater engagement with regulators

c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community

Agree

d) Encourage industry to adopt a pre-competitive approach to research

Neutral

e) Rethink patent lifetime and conduct public-private clinical trials

Agree

f) Review and update legislation on treatment

Agree

g) Review and update legislation on privacy and data disclosure

Agree

Question 2: Please rank the suggestions in order of priority

First Priority

Second Priority

Third Priority

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points

a) Translational research needs to be promoted

Agree

b) Encourage open-access sharing of data and materials

Agree

c) Joint academic-industry funding models

Agree

d) Simplify funding application systems

Strongly Agree

e) Maintain capacity for 'bottom-up' innovative funding

Strongly Agree

Question 2: Please rank the suggestions in order of priority

First Priority

e) Maintain capacity for 'bottom-up' innovative funding

Second Priority

d) Simplify funding application systems

Third Priority

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

Concluding questions

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?

UID
1416
Category of respondee
Commercial researcher - Pharmaceutical
Country
United Kingdom
Replying on behalf of organisation/company?
As a private individual

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Agree
b) Improve education and training of healthcare professionals
Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Strongly Agree
d) Increase training for translational and clinician-scientists
Strongly Agree
e) Increase numbers of post-doctoral level researchers
Agree

Question 2: If you had to choose one priority from the points above what would it be?
b) Improve education and training of healthcare professionals

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?
Pharmacogenetics

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
b) Survey long-term care standards and provision across Europe
Agree
c) Research into the needs of carers
Agree
d) Research into care approaches including end of life decision-making
Neutral
e) Rethink approaches to care
Agree
f) Understand and investigate influence of comorbidities
Agree
g) Conduct research into effects of nutrition and frailty
Agree
h) Determine cost-effectiveness of healthcare pathways
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
b) Survey long-term care standards and provision across Europe
Second Priority
e) Rethink approaches to care
Third Priority
h) Determine cost-effectiveness of healthcare pathways

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 has both advantages and disadvantages, how do you suggest we change the balance to promote the advantages?

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Agree
b) Promote development of non-pharmacological interventions
Strongly Agree

c) Conduct multi-centre primary prevention studies
Neutral
d) Ensure better patient selection/stratification
Strongly Agree
e) Rethink approach to therapeutics
Agree
f) Support for high-risk projects
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Increase involvement of individuals in research
Second Priority
d) Ensure better patient selection/stratification
Third Priority
b) Promote development of non-pharmacological interventions

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?

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
Agree
b) Improve understanding of disease stages
Agree
c) Improve understanding of disease mechanisms
Strongly Agree
d) Develop an improved understanding of the genetic basis for NDD
Strongly Agree
e) Determine the importance of genetic and environmental risk factors
Strongly Agree
f) Focus research on rare hereditary forms of disease
Agree
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Agree
h) Develop more representative animal and cell-based models of disease
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
c) Improve understanding of disease mechanisms
Second Priority
e) Determine the importance of genetic and environmental risk factors
Third Priority
b) Improve understanding of disease stages

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

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

Question 1: Please indicate to what extent you endorse the following points
a) Redefine and standardise disease definitions and diagnosis
Agree
b) Redefine and harmonise clinical endpoints and outcomes
Strongly Agree
c) Develop new biomarkers
Strongly Agree
d) Consider regulatory approaches
Agree

Question 2: Which of the following do you think is most important in terms of biomarkers?
d) Facilitating back-translation to models of disease

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points
a) Improve access to, and sharing of, infrastructure and resources
Neutral
b) Improve access to patient groups, samples and data
Neutral
c) Improve data and sample collection
Agree
d) Develop a register of persons with cognitive impairment
Agree
e) Develop centres of excellence
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
e) Develop centres of excellence
Second Priority
d) Develop a register of persons with cognitive impairment
Third Priority
c) Improve data and sample collection

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 forsee difficulties, how can we overcome these?

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

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
Agree
b) Ensure greater engagement with regulators
Agree
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community
Agree
d) Encourage industry to adopt a pre-competitive approach to research
Neutral
e) Rethink patent lifetime and conduct public-private clinical trials
Neutral
f) Review and update legislation on treatment
Agree
g) Review and update legislation on privacy and data disclosure
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Need for evidence-based policy
Second Priority
b) Ensure greater engagement with regulators
Third Priority
g) Review and update legislation on privacy and data disclosure

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points
a) Translational research needs to be promoted
Agree
b) Encourage open-access sharing of data and materials
Neutral
c) Joint academic-industry funding models
Agree

d) Simplify funding application systems
Agree
e) Maintain capacity for 'bottom-up' innovative funding
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
a) Translational research needs to be promoted
Second Priority
c) Joint academic-industry funding models
Third Priority
d) Simplify funding application systems

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

Concluding questions

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?

UID
1466
Category of respondee
Commercial researcher - Pharmaceutical
Country
United Kingdom
Replying on behalf of organisation/company?
As a private individual

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Agree
b) Improve education and training of healthcare professionals
Agree
c) Increase numbers of neurodegenerative disease (NDD) researchers
Strongly Agree
d) Increase training for translational and clinician-scientists
Agree
e) Increase numbers of post-doctoral level researchers
Agree

Question 2: If you had to choose one priority from the points above what would it be?
c) Increase overall numbers of researchers

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?
Need to deeply understand the molecular processes involved in ND, and then targets will emerge. Need to understand risk associated with targets. Probably will need to intervene during the prodromal/proximity stage of disease but this would require huge collaboration between pharma, academia, not-for-profits. A new model may be required as the risk may be too great, too challenging for pharma alone.

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
Agree
b) Survey long-term care standards and provision across Europe
Agree
c) Research into the needs of carers
Strongly Agree
d) Research into care approaches including end of life decision-making
Agree
e) Rethink approaches to care
Agree
f) Understand and investigate influence of comorbidities
Strongly Agree
g) Conduct research into effects of nutrition and frailty
Agree
h) Determine cost-effectiveness of healthcare pathways
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
f) Understand and investigate influence of comorbidities on NDD
Second Priority
h) Determine cost-effectiveness of healthcare pathways
Third Priority
g) Conduct research into effects of nutrition and frailty

Question 3: How would you define "care"?
Understanding patient reality, and mitigation of effects of suffering.

Question 4: Have you any (additional) suggestions as to how care systems should be revised?
I have not really thought about it. I need to more deeply understand current systems

Question 5: Provision of care at home has both advantages and disadvantages, how do you suggest we change the balance to promote the advantages?
See answer to Q4

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Neutral
b) Promote development of non-pharmacological interventions
Agree
c) Conduct multi-centre primary prevention studies
Agree
d) Ensure better patient selection/stratification
Agree
e) Rethink approach to therapeutics
Agree
f) Support for high-risk projects
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
d) Ensure better patient selection/stratification
Second Priority
e) Rethink approach to therapeutics
Third Priority
c) Conduct multi-centre primary prevention studies

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?

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
Agree
b) Improve understanding of disease stages
Agree
c) Improve understanding of disease mechanisms
Strongly Agree
d) Develop an improved understanding of the genetic basis for NDD
Agree
e) Determine the importance of genetic and environmental risk factors
Agree
f) Focus research on rare hereditary forms of disease
Strongly Agree
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Agree
h) Develop more representative animal and cell-based models of disease
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
c) Improve understanding of disease mechanisms
Second Priority
f) Focus research on rare hereditary forms of disease
Third Priority
b) Improve understanding of disease stages

Question 3: In relation to disease mechanisms which of the following do you think it is most important to investigate?
c) Mechanisms of neuronal death and dysfunction

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
As long as the human studies could be conducted safely, why not? It may be impossible to model human pathology in a model, so, if that is the case, why waste time? As long as target engagement can be demonstrated, then patient benefit can be determined.

Diagnosis, disease definitions and outcome measures

Question 1: Please indicate to what extent you endorse the following points
a) Redefine and standardise disease definitions and diagnosis
Agree
b) Redefine and harmonise clinical endpoints and outcomes
Agree
c) Develop new biomarkers
Strongly Agree
d) Consider regulatory approaches
Agree

Question 2: Which of the following do you think is most important in terms of biomarkers?
c) Providing an indicator of, and sensitivity to, disease progression

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points
a) Improve access to, and sharing of, infrastructure and resources
Agree
b) Improve access to patient groups, samples and data
Agree
c) Improve data and sample collection
Agree
d) Develop a register of persons with cognitive impairment
Agree
e) Develop centres of excellence
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
e) Develop centres of excellence
Second Priority
a) Improve access to, and sharing of, infrastructure and resources
Third Priority
c) Improve data and sample collection

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

Question 4: What are your views on making data open access? If you forsee difficulties, how can we overcome these?
View it as pre-competitive. IP may be an issue but participants may want to consider the former.

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

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
b) Ensure greater engagement with regulators
Agree
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community
Agree
d) Encourage industry to adopt a pre-competitive approach to research
Strongly Agree
e) Rethink patent lifetime and conduct public-private clinical trials
Agree
f) Review and update legislation on treatment
Agree
g) Review and update legislation on privacy and data disclosure
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
d) Encourage industry to adopt pre-competitive approach
Second Priority
e) Rethink patent lifetime and conduct public-private clinical trials
Third Priority
b) Ensure greater engagement with regulators

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points
a) Translational research needs to be promoted
Agree
b) Encourage open-access sharing of data and materials
Agree
c) Joint academic-industry funding models
Agree
d) Simplify funding application systems
Agree
e) Maintain capacity for 'bottom-up' innovative funding
Agree

Question 2: Please rank the suggestions in order of priority
First Priority
c) Joint academic-industry funding models
Second Priority
d) Simplify funding application systems
Third Priority
e) Maintain capacity for 'bottom-up' innovative funding

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

Concluding questions

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?

UID
1914
Category of respondee
Commercial researcher - Pharmaceutical
Country
France
Replying on behalf of organisation/company?
As a private individual

Education, training and collaboration

Question 1: Please indicate to what extent you endorse the following points
a) Improve dialogue between researchers and the wider population
Neutral
b) Improve education and training of healthcare professionals
Neutral
c) Increase numbers of neurodegenerative disease (NDD) researchers
Neutral
d) Increase training for translational and clinician-scientists
Agree
e) Increase numbers of post-doctoral level researchers
Disagree

Question 2: If you had to choose one priority from the points above what would it be?
d) Increase training for translational and clinician-scientists

Question 3: Are there any other specialist areas which you think need promoting or should be given greater emphasis?

Health and social care issues

Question 1: Please indicate to what extent you endorse the following points
a) Define the term "care"
Neutral
b) Survey long-term care standards and provision across Europe
Agree
c) Research into the needs of carers
Neutral
d) Research into care approaches including end of life decision-making
Agree
e) Rethink approaches to care
Disagree
f) Understand and investigate influence of comorbidities
Agree
g) Conduct research into effects of nutrition and frailty
Agree
h) Determine cost-effectiveness of healthcare pathways
Neutral

Question 2: Please rank the suggestions in order of priority
First Priority
g) Conduct research into effects of nutrition and frailty
Second Priority
f) Understand and investigate influence of comorbidities on NDD
Third Priority
d) Research end of life decision making

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 has both advantages and disadvantages, how do you suggest we change the balance to promote the advantages?

Prevention/treatment strategies and trials

Question 1: Please indicate to what extent you endorse the following points
a) Increase involvement of individuals in research
Agree
b) Promote development of non-pharmacological interventions
Neutral

c) Conduct multi-centre primary prevention studies
Strongly Agree
d) Ensure better patient selection/stratification
Neutral
e) Rethink approach to therapeutics
Agree
f) Support for high-risk projects
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
f) Support for high-risk projects
Second Priority
c) Conduct multi-centre primary prevention studies
Third Priority
a) Increase involvement of individuals in research

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?

Disease cause, mechanisms and models

Question 1: Please indicate to what extent you endorse the following points
a) Understand relationship between neurodegenerative disease and ageing
Neutral
b) Improve understanding of disease stages
Neutral
c) Improve understanding of disease mechanisms
Agree
d) Develop an improved understanding of the genetic basis for NDD
Strongly Agree
e) Determine the importance of genetic and environmental risk factors
Strongly Agree
f) Focus research on rare hereditary forms of disease
Neutral
g) Establish pan-European population-based studies including year-on-year (longitudinal) studies in high risk groups
Agree
h) Develop more representative animal and cell-based models of disease
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
h) Develop more representative animal and cell-based models
Second Priority
c) Improve understanding of disease mechanisms
Third Priority
d) Develop an improved understanding of the genetic basis

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

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
yes, when cell-based models demonstrate convincing pharmacologic effects and safety profile otherwise of molecule is acceptable.

Diagnosis, disease definitions and outcome measures

Question 1: Please indicate to what extent you endorse the following points
a) Redefine and standardise disease definitions and diagnosis
Neutral
b) Redefine and harmonise clinical endpoints and outcomes
Strongly Agree
c) Develop new biomarkers
Strongly Agree
d) Consider regulatory approaches

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

Data, registries, repositories and centres

Question 1: Please indicate to what extent you endorse the following points
a) Improve access to, and sharing of, infrastructure and resources
Neutral
b) Improve access to patient groups, samples and data
Agree
c) Improve data and sample collection
Agree
d) Develop a register of persons with cognitive impairment
Strongly Agree
e) Develop centres of excellence
Agree

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 forsee difficulties, how can we overcome these?

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

Policy, regulation and legislation

Question 1: Please indicate to what extent you endorse the following points
a) Need for evidence-based policy
Disagree
b) Ensure greater engagement with regulators
Neutral
c) Facilitate research in areas outside the universities and hospitals in sectors such as care homes and within the wider community
Agree
d) Encourage industry to adopt a pre-competitive approach to research
Strongly Agree
e) Rethink patent lifetime and conduct public-private clinical trials
Strongly Agree
f) Review and update legislation on treatment
Strongly Agree
g) Review and update legislation on privacy and data disclosure
Neutral

Question 2: Please rank the suggestions in order of priority
First Priority
e) Rethink patent lifetime and conduct public-private clinical trials
Second Priority
d) Encourage industry to adopt pre-competitive approach
Third Priority

Question 3: Can you suggest any further policy or regulatory approaches that might encourage or promote the development of new treatments?

Funding and funding mechanisms

Question 1: Please indicate to what extent you endorse the following points
a) Translational research needs to be promoted
Agree
b) Encourage open-access sharing of data and materials
Strongly Agree
c) Joint academic-industry funding models
Agree
d) Simplify funding application systems
Agree

e) Maintain capacity for 'bottom-up' innovative funding
Strongly Agree

Question 2: Please rank the suggestions in order of priority
First Priority
e) Maintain capacity for 'bottom-up' innovative funding
Second Priority
Third Priority

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

Concluding questions

Question 1: Are there strategies you think we have overlooked, if so please suggest below (up to three suggestions)
Regulatory framework needs to be fundamentally re-worked to allow novel treatments to reach patients one safety has been established and early evidence of efficacy has been established; i.e. before definitive evidence of efficacy has been established. The duration of trials in many age-related NDDs is such that waiting for the final answer to fully establish the label will mean that most patients who have an established NDD will not benefit from any of the therapies that are in development today. Rather, if there is a reasonable possibility that patients would benefit and the risk has been defined, individual patients and their physicians should have the ability to use that treatment. Compassionate use and other early access mechanisms are inadequate because if companies are not allowed to truly sell the product (and recoup their costs to manufacture at commercial scale) they cannot make the drug available.

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?