

ERA-NET NEURON

STRATEGIC RESEARCH AGENDA



PART II

**FEEDBACK FROM
PROFESSIONAL AND PATIENT
ORGANISATIONS**

ERA-NET NEURON Strategic Research Agenda

Part II

Feedback from professional and patient organisations

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INTRODUCTION

The ERA-NET NEURON is a network of research funding organisations and ministries across Europe, Israel, and Canada. Identifying current as well as upcoming and emerging hot topics in disease-related neuroscience is imperative for the success of NEURON. This is why developing a Strategic Research Agenda (SRA) to identify and tackle opportunities and challenges in disease-related neuroscience was a priority of NEURON. The SRA was authored by the international NEURON Scientific Advisory Board (SAB) and a group of additional scientists. Within the fields of neurological, psychiatric, sensory organ and peripheral nervous system disorders three main areas were addressed:

- (i) understanding disease mechanisms,
- (ii) understanding disease progression, and
- (iii) interventions.

The SRA was posted on the NEURON web site on January 19, 2015 (<http://www.neuron-eranet.eu/en/390.php>). This SRA provides a framework for future investment and addresses how European research efforts across Europe and beyond can be harnessed most effectively to improve prevention, diagnosis, and treatment of diseases affecting the brain and nervous system.

Promoting a dialogue between researchers and patient organisations represents another priority of NEURON. This is not only important for disseminating information about brain research but it also endeavours to include the needs of patients and their families in order to help shape research to address the major hurdles of brain pathologies. To that end, NEURON launched a survey to seek feedback from professional societies and patient organisations about the SRA.

METHODOLOGY

A questionnaire containing multiple choice and open end questions (see Annex 1) was sent out to 52 professional societies and 184 patient organisations originating from all NEURON partner countries.

Twelve professional societies and 13 patient organisations filled in the questionnaire, representing response rates of 23% and 7%, respectively (Figure 1). While direct feedback from several countries was missing, the response from the Federation of European Neuroscience Societies (FENS) represents 31 European countries and Israel, who are FENS members. Similarly, a European patient organisation provided a general view independent of country specifics.

The three main chapters of the SRA were covered by the questionnaire: disease mechanisms, disease progression, and interventions.

For each question concerning the level of satisfaction five choices were offered: totally satisfied, satisfied, moderately satisfied, unsatisfied, not satisfied. The first two choices were considered as a global positive satisfaction index while the last three were considered as a global negative satisfaction index. Additionally, comments were invited for each question.

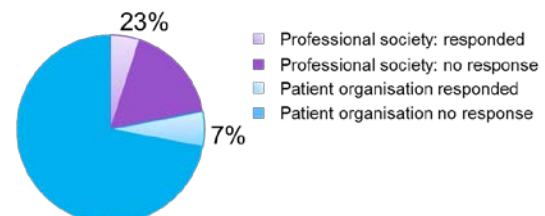


Figure 1: Response rates of contacted organisations. Twelve out of 52 professional societies (23%) and 13 out of 184 patient organisations (7%) responded to the survey.

SCIENTIFIC PRIORITIES

Concerning the **disease mechanism** priorities, 88% of the respondents expressed a generally positive opinion (Figure 2). The comments pointed out that understanding the basic neurobiological mechanisms in disease is critical to develop new treatments. However, studying pathological mechanisms was not considered a good way to support fundamental discoveries about normal brain functions. Overall, more emphasis on basic science unrelated to disease mechanisms was a common response to many questions. Also, one organisation emphasized that systems approaches must be prioritized above all in order to successfully address the identified areas. Furthermore, the translational dimension might be more clearly expressed to better integrate basic science and clinically oriented research. Additionally, it was specifically noted that the role of gender in brain biology has been neglected in the SRA.

The clinically oriented research priority on **disease progression** gained a slightly higher positive rate reaching 92% of generally positive opinion (Figure 3). The needs on the patients' side are extremely high as illustrated by the following sentence: "The important thing is the rapidity of the start of the diagnostic demarche [=process]". Furthermore, one specific comment indicated that comorbidity should be taken into account in disease oriented research.

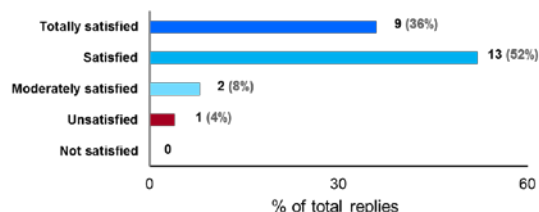


Figure 2: Distribution of responses reflecting satisfaction about the basic neuroscience priority. The responses "Totally satisfied" and "Satisfied" are counted as generally positive opinions. Thus, 88% of respondents expressed a positive opinion. The total number of responses and percentage of total replies are indicated adjacent to the bars, respectively.

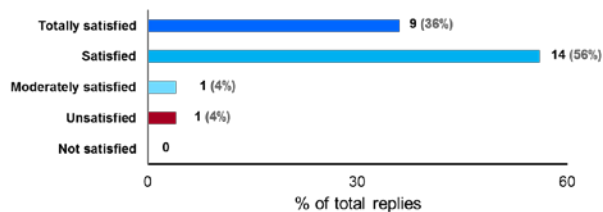


Figure 3: Distribution of responses reflecting satisfaction about the clinically oriented research priority. In sum, there was 92% of generally positive opinion. Conventions as in Figure 2.

For the third scientific priority concerning **clinical interventions**, 88% of the respondents expressed a positive opinion (Figure 4). For the patient organisations this third priority was the most important but they also acknowledged the importance of the other two priorities. Furthermore, some answers indicated that prevention and treatment are critical aspects that also require fundamental research. Finally, one respondent doubted that autism is a health problem and questioned the need of treatments.

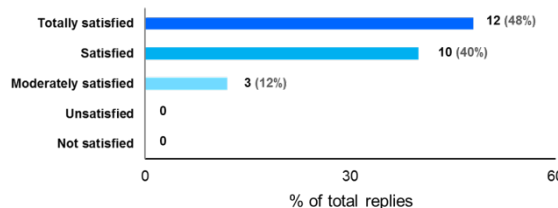


Figure 4: Distribution of responses reflecting satisfaction about the clinical intervention priority. In sum, there was 88% of positive opinion. Conventions as in Figure 2.

SPECIFIC PRIORITIES AND CHALLENGES

The questionnaire also requested opinions concerning other priorities. Importantly, research into common factors, genetic and other, between diseases (neurodegenerative or "functional") appeared as a topic to be promoted. Similarly, it appears that not enough emphasis has been placed onto social sciences and organisation of care. In line with this, social burden on families and carers represent a neglected issue. Some comments that the high prevalence of

Alzheimer disease and the fact that research about other less common neurodegenerative diseases should be supported also emerged. However, specific research focused on neurodegenerative disorders is outside the scope of NEURON, as this is covered by other funding instruments. Comments about the importance of specific disorders were also expressed by patient organisations whose members had a personal interest in those diseases.

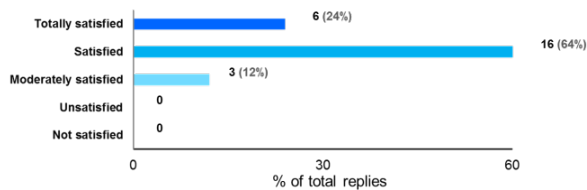


Figure 5: Distribution of responses reflecting satisfaction about the specific priorities and challenges on neurological diseases. In sum, there was 88% of positive opinion. Conventions as in Figure 2.

With regard to the subdivision between **neurology** and **psychiatry**, 88% of the responders were satisfied with the priorities in the neurological field (Figure 5), while only 76% of them were satisfied with the priorities in the psychiatric field (Figure 6). Some respondents were critical of the fact that the SRA does not give enough emphasis to the social context of mental health. In their opinion pure biological mechanistic and reductionist accounts of mental diseases are limited. Diverging opinions were also expressed about who should treat a given disease. Some respondents indicated that there should be more bridges between neurology and psychiatry. Others do not understand why psychiatrists are involved in the care of neurological disorders, and finally some even questioned the need to treat psychiatric disorders.

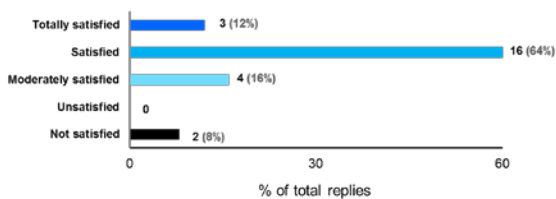


Figure 6: Distribution of responses reflecting satisfaction about the specific priorities and challenges on psychiatric disorders. In sum, there was 76% of positive opinion. Conventions as in Figure 2.

One aspect of the SRA covered research on the **sensory organs**. Eighty per cent of the respondents were satisfied with the priorities (Figure 7). One of them made a specific comment questioning the inclusion of **peripheral nervous system** diseases in a different category to neurological diseases. More emphasis on autonomic disorders was also requested by others. Notably, one major contributor to the questionnaire indicated that visual disorders alone represent one of the most important sources of economic medical costs. Hence, this contributor views research on visual disorders as being of the utmost importance and urges an emphasis on genetic, epigenetic, and environmental risk factors and to develop a personalized medicine approach to these disorders.

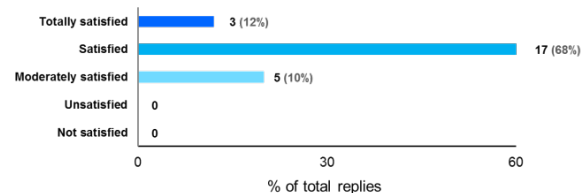


Figure 7: Distribution of responses reflecting satisfaction about the specific priorities and challenges on sensory organ diseases and peripheral nervous system disorders. In sum, there was 76% of positive opinion. Conventions as in Figure 2.

ENABLING ACTIVITIES

The enabling activities addressed in the SRA resulted in a 76% positive opinion (Figure 8). General comments about new instruments showed that improvement of this part of the SRA should be considered. A stronger linkage between basic scientists and clinicians, and support of network development were also encouraged. Further investment in early-career scientists and mobility should be supported. To this end, short term research visit grants and other mobility grants for exchange and training of researchers at all stages would be of great benefit for this program. Exchange with non-profit organisations in this field was considered beneficial and should be reinforced. In line with this, collaborative initia-

tives should be encouraged at the European level and worldwide. For instance, data sharing needs the harmonization of imaging data and clinical data to obtain a consistent data base that will enable faster and better patient stratification.

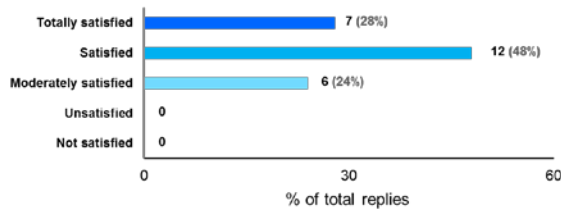


Figure 8: Distribution of responses reflecting satisfaction about enabling activities. In sum, there was 76% of global positive opinion. Conventions as in Figure 2.

OUTREACH ACTIVITIES

In terms of outreach activities, some patient organisations expressed the opinion that progress and the results of research should be relayed to patients and their families. This will potentially provide comfort for the families and the patients' state, even if it does not directly result in better health outcomes due to the research progress itself. This view is also shared by the

NEURON consortium and will pave the way for a symposium bringing together NEURON partners, professionals and patient organisations.

CONCLUSION AND ACKNOWLEDGEMENT

In summary, all answers to all questions pooled together, resulted in an 84% generally positive opinion about the NEURON SRA. We would like to thank participants for taking the time to answer the questionnaire and for their comments which will help the NEURON consortium to further shape the SRA and future activities. To conclude, the comment of one patient organisation appears to us of utmost importance and is in full agreement with the NEURON policy. This organisation applauds the fact that the ERA-NET NEURON recognizes that funded research has to be of the highest scientific excellence and urges NEURON to ensure that the analysis of associated policies must also be based on scientific evidence, rather than political considerations. It is with this spirit that the new NEURON SRA has been written and future policy will be developed.

ANNEX 1**QUESTIONNAIRE ERA-NET NEURON, SRA 2015:
COMMENTS OR SUGGESTIONS****Question 1: "Understanding disease mechanisms (cell-based & animal models, comorbidities, and resilience)"?**

- Understanding the basic biological mechanisms in disease is critical
- Why always see diseases and not positive?
- Fundamental science is not supported. Understanding the healthy brain is essential for further development. Focusing only on pathology is not a good way to support fundamental discoveries.
- Translational dimension might be more clearly expressed here
- There are so many rare and orphan diseases that few of them benefit from a depth research
- The list of topics covers well this priority area
- Understanding physiological aspects of disease is fine, but we need to have a final and complete understanding of brain physiology as well
- Role of gender

Question 2: "Understanding Disease Progression (Pathology, Diagnosis, Biomarkers, Stratification)"?

- The important is the rapidity of the start of the diagnostic demarche [= process]
- Fundamental science is not supported. Understanding the healthy brain is essential for further development. Focusing only on pathology is not a good way to support fundamental discoveries.
- Comorbidity might be taken into account here
- The list of topics covers well this priority area
- The major outcome of identifying biomarkers should be explicitly reported in the Table. It requires intensive basic research that should be underlined in the text.

Question 3: "Interventions (Prevention, Treatment, Care / Management)"?

- For our patient association this third priority is the most important but we know the other ones are important
- There is overlap with aims 1 and 2, so it could be made clearer
- Treatment? Why treatment? Autism is not an health problem
- Of huge importance!! I would consider this as highest priority
- The general priority covers the most essential topics in this area
- Prevention and treatment is a critical aspect that requires fundamental research.

Question 4: "Do you think that other priorities should be included within the frame of ERANET NEURON on translational research?"

- Don't forget, Alzheimer is not the only neurodegenerative disease needing translational research. Spinocerebellar and Friedreich ataxias are important
- Fundamental science. We are very far from understanding the normal brain, in short of this it is futile to concentrate ONLY on pathology.
- Research into common factors, genetic and other, between diseases (neurodegenerative or "functional") would deserve to be promoted
- Measure of the social burden on families and caregivers
- For true translational approaches it will be important to organize networks between fundamental and clinical researchers
- How to know if some programs can benefit to others in different areas

- The management of symptoms in multiple sclerosis (e.g. fatigue, cognitive impairments...)
- Preventive approaches must be a major priority
- As a patient organisation, we think that the progress and the results of the research should be relayed to the patients and their family. Even if it doesn't make research move forward it would comfort the families and potentially improve the patient's state.

Question 5: “Specific Priorities and Challenges on Neurological Diseases?”

- Don't forget intellectual deficiencies
- The Strategic Research Agenda should explicitly include rare and pediatric neurological diseases

Question 6: “Specific priorities and challenges on psychiatric disorders?”

- Psychiatric disorders are not part of our neurodegenerative disease
- I am not a psychiatrist, but this does not give enough emphasis to the social context of mental health. A pure biological mechanistic and reductionist account is limited
- Psychiatry? Why, neurological states must be treated by psychiatry? Why must they be treated?
- the Strategic Research Agenda should explicitly include rare and pediatric psychiatric disorders
- Necessity to share experience between experts in neurology and psychiatry

Question 7: “Specific priorities and challenges on sensory organ diseases and peripheral nervous system disorders?”

- I do not think that is particularly logical to put peripheral nervous system disease in a different category to 'neurological diseases'. It will confuse some people
- Autonomic nervous system underrepresented
- Again, rare and pediatric conditions should be mentioned under this specific priority and challenges

Question 8: “Enabling activities of ERA-NET NEURON?”

- Don't forget rare neurologic diseases
- For true translational approaches it will be important to organize networks between fundamental and clinical researchers
- Crossed initiatives are mandatory and should be pushed at European level or more
- To invest on early-career scientists is a good thing. However, if each country has no possibility to propose an academic career to young researchers afterwards, it will be not benefic at all. Interaction with European Initiatives is a good thing. We should not forget to exchange also with non-profit organisations in this field. Our association is part of a European network of non-profit organisations which fund basic and translational research. These interactions would be beneficial for everyone.
- The description of these activities remains a little vague
- Short term research visits grants and other mobility grants for exchange and training of all stages researchers would be of great benefit for this program
- Research priorities are great but the general public (and patients associations) have limited visibility on what ERANET does or can help achieve
- Data sharing needs the harmonization of imaging data to obtain a consistent data base that will enable faster and better patients stratification.

OTHER COMMENTS OR SUGGESTIONS

The European... Society applauds the ERA-NET NEURON in its efforts to stress the importance of research in the sensory organs and the eye in particular. We therefore consider the Strategic Research Agenda as a pledge to address major challenges crucial in advancing

knowledge in the sensory system research. This may also contribute further in improving the long-term coordination and implementation of research programs and relevant policies in Europe...

The ...Society applauds that ERA-NET NEURON recognizes the fact that the funded research has to be of highest scientific excellence and we urge the ERA-NET NEURON to ensure that analysis of associated policies must also be based on scientific evidence, rather than political considerations.

...

The ...Society supports fully the commitment to recognize and encourage multidisciplinary and believes that the scientific priorities as described in page 8 onwards are very good particularly the ones referring to novel technologies, sources and thinking and the use of 'smart' and 'big' data. Exploring risk factors (genetic, epigenetic, and environmental) for nervous system disorders (NSD) are of paramount importance as they are protective factors, shared risk factors.

We agree with the ERA-NET NEURON criteria for an effective research agenda but we would like to emphasize that systems thinking approach must be prioritized above all in order for the EU to address successfully the identified areas. The ... Society believes that fostering systems approaches should be placed as a principal goal and stressed more throughout the Strategic Research Agenda. It is this thinking that will allow for integration of knowledge at all levels and decision making based on scientific evidence rather than political considerations. In particular we support the following research priorities as a number of efforts on these fronts have shown promise in our field:

(I) to uncover genetic, epigenetic, and environmental risk factors for NSD.

(II) to pave the way for approaches to develop personalized medicine. This should involve both an improved account of the specific genetic, epigenetic, and environmental 'make-up' of individuals as well as a detailed characterization of the responses to exposures to specific pharmacological agents.

Similarly the ...Society agrees on the fact that the sensory system is amenable to gene- and cell- based therapeutic approaches as some recent evidence shows on the genetic approaches ...

The ...Society supports the mission of ERA-NET NEURON regarding the translation of novel discoveries from basic research into effective therapies...

The ...Society welcomes the presentation of the retina as the 'window into the brain'. Considering the eye as a whole not only stresses the paramount importance of the sensory organ in the costly management of other diseases but also maintains the systems approach as stated elsewhere in the document.

Recent research demonstrates the importance of such a 'window in the brain' in clinical practice in assessing disease progression and disease management as for example the retinal nerve fibre layer defects in Alzheimer's disease. Tools traditionally applied to ocular pathologies such as optical coherence tomography, hold a potential in monitoring the efficacy of treatment in various neurologic conditions such as Alzheimer's, Parkinson's and multiple sclerosis. Furthermore fundus photography documenting abnormalities of disease processes affecting the eye, such as glaucoma, is also indicated to follow the progress of disease such optic nerve atrophy from multiple sclerosis or other central nervous system anomalies.

The ...Society would also like to stress that developments in teleophthalmology have increased the importance of the eye in multi-disease screening as demonstrated by the exam-

ple of diabetic retinopathy and fundus photography. Diabetic retinopathy is not strictly speaking a brain disease, it is however a manifestation of a systemic condition.

We do note that neurodegenerative disease (such as Parkinson's) is not excluded from NEURON and it may be covered by specific/targeted funding streams for neurodegeneration. We would like, however, to bring forward the fact that there is research evidence on the neurological causes for at least some of the visual function deficit at Parkinson's patients (for example difficulty with eye movements can disturb visual function).

Furthermore evidence is emerging regarding disease cross-mechanisms in stroke, dementia and sensory organs particularly on the importance of mitochondrial function in glaucoma as well as Parkinson's and Alzheimer's. Such cross-mechanisms between diseases will inevitably have an impact on determining new therapeutic goals and interventions. For example the reported link between visual field defects and cortical degeneration inevitably points to the need to include in the therapeutic goals the prevention of cortical degeneration associated with the eye disease and which may limit the efficacy of rehabilitation programs.

For all these reasons the ... Society believes that the importance of eye research must be strengthened within the framework proposed by the SRA and that more emphasis should be given to sensory system in general to ensure clarity at political level regarding the target research areas.

...

ANNEX 2

QUESTIONNAIRE ERA-NET NEURON, SRA 2015

QUESTIONNAIRE ERA-NET

The ERA-NET NEURON is a network of research funding organizations and ministries across Europe, Israel, and Canada. It is dedicated to disease-related neurosciences. The Strategic Research Agenda has been composed to identify the current but also the upcoming and emerging hot topics within the fields of neurological, psychiatric, sensory organ and peripheral nervous system disorders.

Strategic research agenda of ERA-NET NEURON III

Question 1

* Are you satisfied with the first scientific priority "Topic 1: understanding disease mechanisms (cell-based & animal models, comorbidities, and resilience)"?

Êtes-vous satisfait-e avec la première priorité scientifique : "Comprendre les mécanismes des maladies (modèles animaux et cellulaires, comorbidité, et résilience) ?

Comments or suggestions

Commentaires / suggestions

Question 2

* Are you satisfied with the second scientific priority "Understanding Disease Progression (Pathology, Diagnosis, Biomarkers, Stratification)"?

Etes-vous satisfait-e de la deuxième priorité "Comprendre la progression de la maladie (pathologie, diagnostique, biomarqueurs, stratification) ?

Comments or suggestions

Commentaires / suggestions

Question 3

* Are you satisfied with the third scientific priority "Interventions (Prevention, Treatment, Care / Management)"?

Etes-vous satisfait-e de la troisième priorité scientifique "Intervention (prévention, traitement, attention / management) ?

Comments or suggestions

Commentaires / suggestions

Question 4

Do you think that other priorities should be included within the frame of ERANET NEURON on translational research?

* Yes Oui

No Non

If yes, please list

Si oui, lesquelles ?

Question 5

* Are you satisfied with the Specific Priorities and Challenges on Neurological Diseases?

Etes-vous satisfait-e des priorités et des challenges spécifiques sur les maladies neurologiques ?

Comments or suggestions

Commentaires / suggestions

Question 6

* Are you satisfied with the specific priorities and challenges on psychiatric disorders?

Etes-vous satisfait-e des priorités et challenges spécifiques sur les troubles psychiatriques ?

Comments or suggestions

Commentaires / suggestions

Question 7

* Are you satisfied with the specific priorities and challenges on sensory organ diseases and peripheral nervous system disorders?

Etes-vous satisfait-e des priorités et challenges spécifiques sur les maladies des organes sensoriels et les troubles du système nerveux périphérique ?

Comments or suggestions

Commentaires / suggestions

Question 8

* Are you satisfied with the enabling activities of ERANET NEURON?

Etes-vous satisfait-e des activités rendues possibles grâce à l'ERANET NEURON ?

Comments or suggestions

Commentaires / suggestions

Personal information

Country

Pays

Organization

Organisation

Size of the representation (members)

Taille

Name

Nom

* Email

Do you want to receive all new information about ERANET NEURON?

Yes *Oui*

No *Non*

* Champs obligatoires

Envoi

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