JPND Patient Stakeholder Consultation Meeting: Brussels,

26 May 2011

Key Outputs Report

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I Executive summary

In order to obtain perspectives from patient and carer organisations, JPND held a face-to-face consultation meeting on May 26th, 2011 in Brussels which aimed to identify patient-centred priorities for JPND research. The consultation brought together representatives from pan-European patient and carer organisations including those groups with specific interests in the neurodegenerative diseases that fall within the focus of the JPND research agenda.

The key research aims to emerge from the meeting were:

1. Improving understanding of patient and carer participation in clinical research
2. Addressing gaps in the education and training of clinical researchers, healthcare professionals and patients / carers
3. Improving patient-relevant outcome measures
4. Developing guidelines for appropriate use of rating scales and measurements used for research and clinical practice, including better quantifiable measures for cognitive decline
5. Improving understanding of the impact of the carer role on the quality of care given to ND patients
6. Investigating the behavioural underpinnings of ND-associated stigma
7. Improving understanding of ND-specific end-of-life effects on carers and families
8. Increasing health economic analysis of care including socio-economic analysis.

II Background

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

The Patient Stakeholder consultation meeting took place at a pivotal juncture in the formulation of the JPND Strategic Research Agenda (SRA), an initiative which will provide a roadmap for future activity and investment in EU-wide neurodegeneration research over the coming decade. The SRA is being developed through the outputs from a series of academic expert workshops which are deriving the objective scientific consensus priorities to be addressed by the JPND Management Board. The JPND strategy will take a holistic perspective, incorporating the views of a wide range of stakeholders. Stakeholder groups will contribute to the fourth and final academic workshop and participate in a broad, web-based consultation over the summer. The finalised SRA will be delivered in the latter part of 2011 by the JPND Management Board in the light of policy considerations and the views of all stakeholders. A complete list of JPND stakeholders is available in Annex 3.

III Aim and scope of the meeting

The meeting represented the first stage in the initiative to engage patient stakeholders in outlining the research opportunities and key priorities for JPND research. The aim of the meeting was to provide:

- An opportunity for patient stakeholders to contribute toward the framing of the SRA.
- A view of the research opportunities and barriers to progress in the field, and discussion on patient-relevant priorities for action in the near and longer-term.

This draft overview has been collated by a writing group made up of the chairs, rapporteurs and note takers of the two discussion groups from the meeting, as identified in the workshop agenda provided in Annex 1.

Meeting Format

The agenda for the meeting is provided at Annex 1 and the list of attending participants is given at Annex 2. It began with plenary presentations from key patient organisations about the needs and drivers in their decision making and was followed by a series of presentations from the JPND representatives about the ongoing efforts to construct an SRA for ND research in Europe. Patient input was required on three interim reports from the academic expert workshops conducted under the JPND SRA development initiative, and the three reports were available to all delegates in advance of the meeting. Thereafter, in breakout groups, the meeting delegates discussed, in light of the plenary discussions, and the interim reports, their views on patient-centred needs and opportunities for JPND research.

IV Summary of plenary presentations

The added value of patient partnership in ND Research

***Amanda Worpole**, Executive Director, European Federation of Neurological Associations*

Science is improved by patient involvement. It is practical to include patients in the research process and patient participation will inform and improve clinical practice. Patient's knowledge on diseases is obtained through personal experience, and through different generations. Although patients and researchers may view things differently (e.g. risk assessment), patient knowledge is complementary to clinical and scientific knowledge and should not be wasted.

Patients must be seen as full partners in the research process and in the generation of future research agendas. Patient participation brings added value to the relevance of research questions, the significance and prioritisation of research and in protocol design. However, the patient role must be specifically clarified to avoid tokenism. There are acknowledged concerns on all sides about the ability to contribute, technical knowledge, exposure to difficult facts and fear of looking foolish.

Increasing patient participation in the selection and design of patient-related outcome measures can have a real impact on the quality of research. (e.g. Outcome Measures in Rheumatoid Arthritis Clinical Trials (OMERACT) multi-stakeholder forum for rheumatology research). The European Federation of Neurological Associations (EFNA) has also been involved in the training of patients so they can represent themselves in processes that evaluate the quality and relevance to patient health of research findings such as the UK Health Technology Assessment (HTA).

Healthcare research in today's demographic context - older people's perspective on EU action

***Anne-Sophie Parent**, Secretary General, AGE Europe*

AGE Europe represents the main target group of the European Innovation Platform on Active and Healthy Aging and brings older generation perspectives to the patient working party of the European Medicines Agency. AGE Europe have developed an international charter for end-of-life decisions and recommend the inclusion of the UN Convention on the rights of people with disabilities in JPND outputs. 2012 will be the European Year for Active Ageing and Solidarity between Generations.

Whereas the holistic view of research contained in the JPND interim reports is very welcome, this broader approach was less apparent in the SAB workshop recommendations. Also, many patient organisations do not have the required expertise to address some of the technical issues around basic and clinical research for the JPND research agenda.

Proposals that the JPND initiative might consider include improving older people's participation in clinical trials, improving knowledge on the impact of poly-pharmacy and investigating the transition between institutional, community and home-based care.

V Summary of the recommendations arising from group discussions

Patient participation in clinical research

- Investigate the factors that influence research participation by ND patients and carers - for example, why families do not consent to brain donation post-mortem.
- Increase appropriate patient/carer input into the pragmatic translation of research findings into real-world settings.
- Increase the involvement of patients/families/carers at scientific meetings.
- Develop, make available and follow good exemplars for collaboration between scientists and patients.
- Build more critical mass for research by increasing collaboration and sharing of data between patient groups
- Engage minority communities in research by building on existing registries.
- Include lay and executive summaries for all funded research studies.
- Increase patient involvement in the peer review process.

Diagnosis of ND

- Increase research into harmonisation and validation of rating scales and measurements used for research and clinical practice.
- Develop better quantifiable measures for cognitive decline.
- Identify best practice in making clinical measures acceptable to patients and pragmatic for multi-centre use.
- As part of the health services research agenda, investigate patient pathways to diagnosis and care.

Patient-related outcome measures

- Increase patient participation in the selection and design of patient-related outcome measures.
- Investigate the variations in perceptions and understandings of risk between researchers and patients / carers.

Education and Training

- Raise awareness among General Practitioners of appropriate use of specific diagnosis tools and early referral to specialists when necessary.
- Agree harmonized diagnostic tools across different clinical domains and centres that take account of cultural and societal differences across Europe.

- Educate health professionals to ensure the precise application of validated rating scales across different clinical specialisms, to achieve more reliable diagnosis and monitoring of disease progression and outcomes.

- Educate and train patients/carers through provision of accessible, accurate, peer reviewed information on research outcomes.

- Increase understanding among researchers of clinical care issues and patient needs in research (e.g. tractability, carer involvement).

Carer role in ND

- Increase understanding of the complex role of carers and the effects on quality of care given to ND patients when a carer is under emotional and physical stress - for example when a patient is discharged to home, carers may be asked to take over clinical roles such as performing complex nursing procedures (e.g. care of a PEG – percutaneous endoscopic gastrostomy) yet are not provided with sufficient support, while needing to continue their more general care role.

- Investigate to what extent a potential 'carer effect' could modify the outcomes of pharmacological interventions on patients.

- Conduct studies into the impact of ND disease on the quality of life of informal carers (for example, effects on social inclusion, health, economic contribution)

Bereavement/after-death issues

- Document the unique ND-related bereavement effects (both economic and social) on carers and families.

Behavioural underpinnings of stigma

- Increase understanding of the drivers of stigmatization of ND patients. Lessons can be learned from the de-stigmatization of cancer patients over the last thirty years.

Health economic data

- Promote the collection and availability of strong health economic data for ND research.

- Investigate the effects of socioeconomic backgrounds on quality of care for ND patients.

Engaging with industry

- It makes sense to seize the opportunity arising through current changes in the approach of the biopharmaceutical industry, coupled to increased levels of dialogue with the academic and charity sector, for example by seeking to pool resources with industry to help "de-risk" their investment strategy – e.g. through sharing animal models and untested compounds between academia and industry.

- Increase availability of research results on interventions that do not progress to market, and investigate the alternative use of such interventions.

VI Priorities with impact on patients' lives

In the near term (< 3 years)

- Strongly agree on need for better outcome measures for the evaluation of disease progression and treatments.
- Health economic analysis of care including socio-economic analysis.
- Promote existing EU cohorts (for example the Euro-HD network) for new studies; Consider harmonized approaches for both expansion of existing registries and new prospective studies.
- Expand patient registries by increasing uptake among the wider community (e.g. minority groups, older people) to ensure benefits for the widest groups of patients.
- Care-based research: need for capture and validation of evidence (e.g. on assistive-living technologies).
- Opportunity to engage with industry - "de-risking".
- Enhance communication between patients/carers and researchers to ensure improved understanding of the needs of each sector and the objectives and outputs of research.

In the longer term (> 5 years)

- Obtaining better measures that track the course of ND disease for early diagnosis.
- Robust longitudinal, prospective, lifestyle studies / identification and tracking of risk factors.

VII Additional Comments

- Patients strongly support the sharing of resources across the research community. For example toxicology reports from animal models that may have been utilised for studies in the pharma sector that are now discontinued.
- There is a strong need for an ongoing two-way dialogue between national patient organisations and the JPND initiative to assist processes of communication and translation (e.g. acronyms, language barriers, etc).
- A dilemma exists if patients and patient organisations are asked to choose between supporting research directed at early interventions vs. long-term longitudinal studies to identify risk and for preventive approaches, although it was considered there would be support for the latter approach if there was confidence in the impact this would achieve.
- JPND must take sufficient account of the significant differences in European infrastructural capacity for research so that all countries can benefit in terms of implementation of the SRA.

- JPND needs to have appropriate awareness of the global context of the research priorities identified to date e.g. relevant work carried out in the USA.
- JPND should look to increase understanding of the prevalence of ND diseases, harnessing existing observations from many diverse populations.
- Data on the JPND mapping exercise should be archived and updated and information on completed studies should be made publicly available in a form accessible to the lay reader.
- It is highly important that the impact of disease on carers and families is not overlooked.
- JPND should look to increase understanding of the information needs of patients and carers upon diagnosis of ND. i.e. what information is required, when it should be given, by whom and in what format.

VIII Annex

Annex 1 Meeting Agenda

10:50am: Welcome and Introduction

Martin Rossor, UCL, Vice-Chair JPND Scientific Advisory Board

Philippe Amouyel, INSERM, Chair JPND

11:00am: The added value of patient partnership in ND Research

Amanda Worpole, European Federation of Neurological Associations

11:15am: Healthcare research in today's demographic context – older people's perspective on EU action

Anne-Sophie Parent, AGE Europe

11:30am: JPND - SRA Development

Catherine Moody, Medical Research Council, UK

11:45am: JPND – Mapping of ND Research in the EU

Caitriona Creely, Health Research Board, Ireland

12:00pm: Outputs from Expert Workshops

Martin Rossor, University College London, UK

12:20pm: Questions and Discussion

1:45pm: Breakout sessions on Expert Workshop outputs

A. Three Workshop Reports + Other Priorities (Chair: M. Rossor)
B. Three Workshop Reports + Other Priorities (Chair: R: Buckle)

3:30pm: Break + Rapporteur Compilation

4:00pm: Chair's Report + Discussion

4:45pm: Next Steps and Closing Remarks

Derick Mitchell and Martin Rossor

Annex 2 List of Participants

Patient Organisation Representatives

Name	Position	Affiliation
Kieran Breen	Director of R&D	Parkinson's UK and the European Parkinson's Disease Association
Audrey Craven	President	European Headache Alliance
Beatrice De Schepper	President	European Huntington Association
Annette Dumas	EU Public Affairs Officer	Alzheimer Europe
Frank Goodwin	Secretary	Eurocarers
Asunción Martínez	President	International Huntington Association
Sue Millman	General Secretary	Euro-Ataxia
Anne-Sophie Parent	Secretary General	AGE Platform Europe
Evy Reviers	Director	International Alliance of ALS/MND associations
Gillian Turner	National Coordinator	CJD Support Network, UK and member of the CJD International Support Alliance
Amanda Worpole	Executive Director	European Federation of Neurological Associations

JPND Staff and Observers

Name	Position	Affiliation
Philippe Amouyel	JPND Chair	INSERM, France
Rob Buckle	JPND Management Board	UK Medical Research Council
Caitriona Creely	JPND Programme Manager	Health Research Board, Ireland
Derick Mitchell	JPND Communications Executive	Health Research Board, Ireland
Catherine Moody	JPND Programme Manager	UK Medical Research Council
Jorge Pinto Antunes	Policy Officer, DG-SANCO	European Commission
Manuel Romaris	Policy Officer, DG-Research	European Commission
Martin Rossor	Vice-Chair, JPND Scientific Advisory Board	University College London
Micol Zappa	JPND Programme Manager	MIUR, Italy

Annex 3 JPND Stakeholder Categories

Stakeholder group	Subgroup		
1. Key influencers	**For example:** Politicians – MEPs and National Ministers and Politicians Policy Makers – European Commission, National Agencies Leaders of pan-European initiatives Public champions or Celebrities		
2. Scientists – public researchers	Researchers in hospital / university environment		
3. Scientists – private researchers (industry)	Biotechnology Pharmaceutical Diagnostics	Assisted Living Medical Devices ICT	Health Care Providers
4. Research funding organisations	State funding agencies	Philanthropic organisations	Private funders
5. Health policy makers	Government health department / ministry Government research department / ministry Regional departments Policy specialists		
6. Healthcare professionals	General Practitioners Geriatricians Nurses Physiotherapists Hospital Administrators		
7. Patient interest groups	European Umbrella Organisations National Organisations Research Charities ND-Specific Patient Groups Patient Advocacy Groups Carer Groups		
8. Media	Health-related media General Media		
9. Public	Public Champions and Celebrities		

Annex 4 Glossary

CJD	Creutzfeldt-Jakob Disease
DG-Research	Directorate General for Research and Innovation, European Commission
DG-SANCO	Directorate General for Health and Consumers, European Commission
HD	Huntington's Disease
HRB	Health Research Board, Ireland
HTA	Health Technology Assessment
INSERM	National Institute for Health Research, France
JPND	Joint Programme in Neurodegenerative Diseases, especially Alzheimer's
MIUR	Italian Ministry of Education, Universities, and Research
MRC	Medical Research Council, UK
ND	Neurodegenerative Disease
OMERACT	Outcome Measures in Rheumatoid Arthritis Clinical Trials
PEG	Percutaneous Endoscopic Gastrostomy
SAB	Scientific Advisory Board
SRA	Strategic Research Agenda
UCL	University College London