Collaboration in Alzheimer's disease & beyond: the present and future of IMI initiatives in neurodegeneration

15.03.2017 • IMI Programme Office • Brussels, Belgium


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Contents

Welcome

Thank you for joining us in Brussels for this event on *Collaboration in Alzheimer's disease & beyond: the present and future of IMI initiatives in neurodegeneration.*

This event brings together the complete portfolio of IMI neurodegeneration projects, as well as other key stakeholder organisations and initiatives, both within and beyond Europe, with the goal of discussing existing collaboration and exploring future opportunities to work together to advance Alzheimer's research.

We look forward to welcoming all these strategic partners on neurodegeneration around the table, in order to showcase the IMI portfolio of projects in this area, to discuss the state of the art and lay the foundations for future collaboration in the fight against Alzheimer's disease and other neurodegenerative diseases.

Pierre Meulien
Executive Director

Elisabetta Vaudano
Principal Scientific Manager

Innovative Medicines Initiative (IMI)

Innovative Medicines Initiative (IMI)

Agenda

10:00 – 10:05 **Welcome**

Pierre Meulien, IMI Executive Director

10:05 – 11:30 **Presentations of IMI neurodegeneration projects: part I**

- **AETIONOMY: Organising mechanistic knowledge about neurodegenerative diseases for the improvement of drug development and therapy**
  Martin Hofmann-Apitius
  EMIF-AD: European Medical Information Framework (Alzheimer's disease)
  Simon Lovestone & Pieter Jelle Visser
- **EPAD: European prevention of Alzheimer's dementia consortium**
  Serge Van der Geyten and Craig Ritchie
- **AMYPAD: Amyloid imaging to prevent Alzheimer's disease**
  Gill Farrar and Frederik Barkhof
- **ROADMAP: Real world outcomes across the AD spectrum for better care: multi-modal data access platform**
  Catherine Reed
- **MOPEAD: Models of patient engagement for Alzheimer's disease**
  Merce' Boada and Laura Campo

11:30 – 11:45 *Coffee break*

11:45 – 13:00 **Presentations of IMI neurodegeneration projects: part II**

- **PRISM: Psychiatric Ratings using Intermediate Stratified Markers: providing quantitative biological measures to facilitate the discovery and development of new treatments for social and cognitive deficits in AD, SZ, and MD**
  Bernd Sommer
- **ADAPTED: Alzheimer's disease apolipoprotein pathology for treatment elucidation and development**

Agustin Ruiz & Margot Bakker

- **PHAGO: Inflammation and AD: modulating microglia function - focussing on TREM2 and CD33**
  Harold Neumann & Andreas Ebneth
- **IMPRIND: Inhibiting Misfolded protein propagation in neurodegenerative diseases**
  George Tofaris & Kenneth Tistrup
- **Data quality in preclinical research and development** (upcoming project)
  Thomas Steckler

13:00 – 14:00	*Networking lunch*
14:00 – 14:45	**Lecture on Alzheimer's disease** Craig Ritchie, Professor of the Psychiatry of Ageing, University of Edinburgh Jean Georges, Executive Director, Alzheimer Europe
15:00 – 15:30	**Introduction to the IMI public-private partnership including IP rules** Elisabetta Vaudano, Principal Scientific Manager, IMI Magali Poinot, Advisor to the Executive Director, IMI
15:30 – 16:00	**Presentation of the priorities for 2017 onwards, strategy and future directions by the IMI Strategic Governing Group (SGG) Neurodegeneration** Luc Truyen, Co-Chair SGG Neurodegeneration, Janssen
16:00 – 18:00	**Input, feedback and discussion with stakeholders and partners** Chaired by Elisabetta Vaudano, IMI
18:00	*End of meeting*
18:30 – 21:00	*Networking cocktail & dinner at The Hotel, Boulevard de Waterloo 38, Brussels*
21:00	*End of event*


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IMI neurodegeneration projects

IMI Strategic Governing Group Neurodegeneration (SGG ND)

The Strategic Governing Groups (SGGs) ensure the coordination of IMI's work in certain strategic areas and work to make the development of new topics more transparent and effective. As such, the SGGs are made of representatives of companies active or interested in the area covered by the scope of the SGG as well as representatives from the European Commission, the IMI Programme Office and the IMI Scientific Committee. The SGGs were created on the basis of Article 7.3.p of the legislation establishing the IMI 2 programme. This allows the Governing Board to set up advisory groups where appropriate.

The **mission of the SGG Neurodegeneration** is to address the highly unmet medical need for effective disease-modifying as well as symptomatic interventions in neurodegenerative disorders in general and Alzheimer's disease in particular. The comprehensive strategy comprises a detailed investigation of the course of the disease with the aim of identifying and validating new drug targets and molecules acting on them as well as biomarkers for patient stratification and treatment effect assessment. The SGG will also drive the development of new trial methodologies with novel endpoints as well as supporting infrastructure requirements to speed up clinical development, including the early generation of more patient and payer-relevant data so that access to effective therapies is facilitated. Ultimately, preventative, curative and/or symptomatic therapies should emerge from research programs supported by the SGG.

SGG ND Chairs and managers attending the event

Michael Hutton
Co-Chair SGG ND,
Distinguished Research Fellow and CSO Neurodegeneration
Eli Lilly

Mariana Constantinovici
Operations Manager External Funding R&D Global Government Grant Office,
Janssen

Luc Truyen
Co-Chair SGG ND
Global Head Development and External Affairs, Neuroscience
Janssen

Kim Cryns
Director R&D Global Government Grant Office,
Janssen

Georg C. Terstappen
Head, Platform Technologies & Science China and PTS Neurosciences TAU Portfolio Lead
GSK, R&D Centre Shanghai

ADAPTED
Alzheimer's Disease Apolipoprotein Pathology for Treatment Elucidation and Development

The ADAPTED project aims to increase the understanding of the function of the APOE gene by addressing the following goals: 1. Clarification of the role of APOE as a risk factor, 2. Target identification, 3. Generation and validation of high value APOE-related model systems and 4. Patient stratification. ADAPTED aims to systematically explore the APOE allele impact in relevant human neurological and peripheral cell systems, iPSC-derived cell systems and patient materials, using a range of omics and directed tools to identify changes that can be mechanistically connected to existing hypotheses.

What ADAPTED is doing to advance neurodegeneration research

- Generation of iPSC-derived astrocyte and neuronal model systems from 4 isogenic sets derived from healthy control and AD patients. 2D and 3D as well as an organ-on-a-chip model will be studied
- Identification of CSF and blood-based metabolic signatures related to APOE genotypes
- Identification of APOE-dependent monocyte and macrophage biology
- Analysis of existing omics datasets stratified by APOE genotype

Project members attending the event

Margot Bakker
Project Director, Neuroscience Discovery
Abbvie

Agustin Ruiz
Chief Scientific Officer
Fundacio ACE

Project start date 01/10/2016 • end date 30/09/2019

AETIONOMY
Organising mechanistic knowledge about neurodegenerative diseases for the improvement of drug development and therapy

The AETIONOMY project has generated the largest inventory of disease mechanisms for neurodegenerative diseases (AD and PD) worldwide. An AETIONOMY disease mechanism typically integrates mechanistic information across scales, connecting biological pathways, genetic variation information, epigenetics, cellular physiology, clinical readouts like cognition and imaging features in one coherent representation. AETIONOMY mechanisms can be directly used for the functional interpretation of complex clinical study data (e.g. ADNI or PPMI), we are currently working on algorithms that allow us to stratify patients based on AETIONOMY disease mechanisms. The AETIONOMY inventory of mechanisms is freely available to everybody through the "mechanism-enrichment" server NeuroMMSigDB.

AETIONOMY's core objectives of neurodegeneration research:

- Identifying disease mechanisms in a systematic fashion
- Making disease mechanism information available in computable form for "Big Data" (complex data analysis) approaches
- Identifying subgroups of patients characterized by particular disease mechanisms
- Creating a "Virtual Dementia Cohort" that overcomes some of the limitations of real-world patient data collections
- Identification of "drugable mechanisms" rather than "targets"

Project members attending the event

Martin Hofmann-Apitius
Head of the Department of Bioinformatics
Fraunhofer Institute for Algorithms and Scientific Computing (SCAI)

Project start date 01/01/2014 • end date 31/12/2018

AMYPAD
Amyloid imaging to prevent Alzheimer's disease

AMYPAD aims to improve the understanding of the value in imaging β-amyloid deposition using positron emission tomography (PET). Since β-amyloid accumulation represents an early and necessary step in the path towards Alzheimer's Disease (AD), the *in vivo* assessment of β-amyloid plaques by PET can improve early diagnosis and potentially provide an opportunity for secondary prevention. Understanding the role of β-amyloid imaging enables the achievement of three goals: 1) improve early diagnosis and patient management, 2) enhance patient selection for clinical trials by better understanding AD's natural history, and 3) accurately monitor β-amyloid changes and quantify the impact of novel therapies.

What AMYPAD is doing to advance neurodegeneration research

Leveraging clinical and neuropsychological neurodegenerative markers gathered within EPAD, AMYPAD will quantitatively analyse up to 6000 β-amyloid PET scans from a large population cohort in the early stages of AD. This rich dataset will be used to develop accurate and complex disease models, as well as to optimize quantitative analyses of β-amyloid PET images in order to increase chances of detecting therapy-induced changes in clinical trials aimed at preventing neurodegeneration.

Project members attending the event

Frederik Barkhof
Professor of Neuroradiology.
VU University Medical Centre Amsterdam

Gill Farrar
Scientific Director, Medical Affairs. Life Sciences
GE Healthcare

Project start date 01/10/2016 • end date 30/09/2021

Data quality in preclinical research and development

There is substantial evidence that the robustness, rigor and validity of preclinical research is limited and problematic. We will define the variables in study design and data analysis that influence outcome in preclinical neuroscience (focus on Alzheimer's disease) and safety studies (focus on CNS safety). Further, we will define the components that will make up a fit-for purpose Quality Management System (QMS) for non-regulated R&D and validate the feasibility of the QMS in prospective studies, including in animal models of Alzheimer's disease. We will also deliver an online educational platform providing education and training in the principles and application of quality and rigour.

What the project will do to advance neurodegeneration research

- Provide evidence/data to enable the development of quality criteria for future preclinical studies in the field of Alzheimer's disease.
- Develop recommendations for more robust Alzheimer's Disease animal models.
- Develop an educational course on scientific quality principles to train (junior) researchers in the field of Alzheimer's disease.

Project member attending the event

Thomas Steckler
Associate Director
Janssen

EMIF-AD
European Medical Information Framework (Alzheimer's disease)

The European Medical Information Framework was founded to facilitate the visibility and use of hitherto hard-to-access datasets including both those generated through research and also through routine care. Platforms have been established that enable identification of data, pooling and harmonization of data and analysis of integrated datasets. Overall, EMIF platforms contain data from over 50k study participants and routine clinical data from 50m Europeans. These platforms are being used in EMIF-Alzheimer's Disease to pool cohort data and to interrogate real-world data in order to determine risk factors, to understand pathophysiological progression, to source samples for novel biomarker discovery and to facilitate clinical trials.

What EMIF-AD is doing to advance neurodegeneration research

Infrastructures include 1) EMIF-AD Catalogue, an online tool with cohort meta-data; 2) a suite of tools enabling data-integration from multiple cohorts as well as remote, federated access to real-world datasets and 3) a tranSMART data platform for storage and analysis of subject level data. Examples of research using these tools includes very large scale studies on epidemiology of pre-dementia, a multimodal biomarker study and on-going interrogation of huge real-world datasets.

Project members attending the event

Bart Vannieuwenhuyse
Senior Director Health Information Sciences, Janssen

Simon Lovestone
Professor of Translational Neuroscience, University of Oxford

Pieter Jelle Visser
Associate professor, clinical epidemiologist, Maastricht University, and VU university Medical Center Amsterdam

Johannes Streffer
Director Experimental Medicine, Clinical Scientist, Janssen

Project start date 01/01/2013 • end date 31/12/2017

EPAD
European prevention of Alzheimer's dementia consortium

EPAD aims to facilitate and accelerate the development of new treatments for the secondary prevention of dementia in people with Alzheimer's disease by setting up an adaptive platform study for clinical proof-of-concept and building the clinical trial infrastructure to support it.

To this end EPAD will establish a European-wide register of approximately 24,000 people at risk of developing Alzheimer's dementia drawing from existing population-based and clinical cohorts. From this group, 6000 research participants will be asked to join a pan-European EPAD Cohort for consistent, longitudinal follow-up, of which approximately 1500 will be invited to the EPAD Proof of Concept Trial.

EPAD's main objectives are:

- to define criteria for identifying AD pathology early in the course of disease in people who have no or minimal symptoms
- to improve the understanding of the early stages of Alzheimer's disease and how it leads to dementia
- to investigate new treatments that aim to prevent or delay the onset of clinical symptoms in people at risk of developing dementia utilizing novel, adaptive approaches that accelerate learning

Project start date 01/01/2015 • end date 31/12/2019

Project members attending the event

Serge Van der Geyten
Director, Neuroscience External Affairs, Janssen

Craig Ritchie
Professor of the Psychiatry of Ageing, University of Edinburgh, Centre for Clinical Brain Sciences

José Luis Molinuevo
Scientific Director
BarcelonaBeta Brain Research Centre - Pasqual Maragall Foundation

Lennert Steukers
Strategic Collaboration Manager, R&D PMO Therapeutic Areas, Janssen

IMPRiND
*I*nhibiting *M*isfolded protein *P*ropagation *i*n *N*eurodegenerative *D*iseases

The principal objective of IMPRiND is to map and target critical processes in the propagation and proteostatic response against misfolded α-synuclein and tau to help arrest the progression of PD and AD. The aims are to: (i) Identify disease-relevant misfolded assemblies and imprint their biological properties. (ii) Develop assays to monitor propagation and clearance that are suitable for screening. (iii) Perform genetic screens based on disease-relevant gene/protein networks and assess druggability of identified targets. (iv) Deliver robust validation using complex cellular systems with greater functional resemblance to the brain. (v) Improve existing animal models to standardize pathological to accelerate the assessment of therapeutic interventions.

What IMPRiND will do to advance neurodegeneration research

To advance neurodegeneration research the consortium is mobilising diverse expertise that builds on established track record. It gathers the best European experts in AD and PD pathobiology and drug discovery. It provides an organisational framework that strengthens collaboration between academia and industry to deliver new potential therapies in neurodegenerative diseases. IMPRiND embraces the open access policy and strives for complete dissemination of all resulting data, databases and publications.

Project members attending the event

George Tofaris
Associate Professor and Consultant Neurologist
University of Oxford

Kenneth Thirstrup
Principal Scientist
H. Lundbeck A/S

MOPEAD
Models of patient engagement for Alzheimer's disease

MOPEAD responds to the IMI2, Call 5 topic 5 "Evolving models of patient engagement and access for earlier identification of Alzheimer's disease (AD)". MOPEAD will test and evaluate four Patient Engagement Models to help identify patients at risk of AD in a five-country, multi-centre setting. It builds upon an already successful model, the Open House Initiative pioneered by Fundació ACE in Barcelona. In addition to Spain, four countries have been selected to implement these models based upon relevant expertise and the consortium brings together partners for their potential to become long-term assets to European efforts to tackle AD challenges.

What MOPEAD is doing to advance neurodegeneration research

MOPEAD is generating the structure to help memory clinics and health systems to implement different Patient Engagement models that will lead to Early Detection of AD. Meanwhile MOPEAD can be the trigger for new stratified approaches to AD and will provide tools to raise social awareness on the importance of Mild Cognitive Impairment. Ultimately, MOPEAD will respond to the urgency of finding interventions to halt AD by stimulating a faster recruitment of patients into clinical trials.

Project members attending the event

Mercè Boada
Founder and medical director
Fundació ACE

Laura Campo
Advisor, Global Advocacy,
Eli Lilly

Project start date 01/10/2016 • end date 30/11/2019

PHAGO
Inflammation and AD: modulating microglia function - focussing on TREM2 and CD33

The ambition is to improve patient outcomes through a better understanding of the biology of TREM2 and CD33 and their biological networks and pathways, and pave the way for future development of therapies aimed at phagocyte dysfunction in AD. We will generate knowledge on the role of TREM2 and CD33 in neuroinflammation and will develop validated assays and identify tools and/or tool antibodies targeted to TREM2/CD33 and related target functions. Those tools will be transferred rapidly and might become suitable for pharmaceutical and industrial research use. Scientific knowledge will be disseminated to relevant stakeholders via the creation of a knowledge database.

What PHAGO is doing to advance neurodegeneration research

We will generate data on modulating microglia/macrophage activation through TREM2, CD33 and related signalling pathways, and determine the effects of such modulation on microglia/macrophage function. Thus, European research and pharmaceutical industries will benefit from understanding fundamental AD disease processes and from identification of druggable points in the TREM2/CD33 signalling pathway to regulate the phagocytes. On a long-term perspective we also hope to achieve benefits for patients with AD or related neurodegenerative diseases.

Project members attending the event

Harald Neumann
Professor, Group Leader
Universitätsklinikum Bonn

Andreas Ebneth
Scientific Director
Janssen

Project start date 01/11/2016 • end date 31/10/2021

PRISM
Psychiatric Ratings using Intermediate Stratified Markers: providing quantitative biological measures to facilitate the discovery and development of new treatments for social and cognitive deficits in AD, SZ, and MD

Neuropsychiatric conditions are diagnosed solely based on behavioural symptoms observed, as there are few objective biomarkers compared to conditions, such as diabetes. Although many different neuropsychiatric diseases share symptoms, there is limited knowledge about the underlying biological causes of a specific disease. The PRISM project aims to demonstrate that quantitative biological parameters of shared symptom domains across neuropsychiatric disorders including schizophrenia (SZ), Alzheimer's disease (AD), and major depressive disorder (MD) can be used to create biologically meaningful clusters blind to the starting diagnosis. These will provide new assessment tools across disorders, and predictive, preclinical animal systems for subsequent neurobiological and pharmacological testing.

What PRISM is doing to advance neurodegeneration research

- Proof-of-concept analyses to cluster and differentiate SZ and AD patients on the basis of quantitative biological parameters
- Explore dimensional relationships between pathology (e.g. cognitive deficits) and social withdrawal
- Develop a deeper understanding of the quantitative biology of social withdrawal using clinical data from patients and by establishing a network of pre-clinical research sites able to perform high-quality back-translation studies
- Develop the regulatory path for social withdrawal across disorders

Project member attending the event

Bernd Sommer
Global Head CNS Diseases Research
Boehringer Ingelheim

Project start date 01/04/2016 • end date 31/03/2019

ROADMAP
Real world outcomes across the AD spectrum for better care: multi-modal data access platform

ROADMAP's goal is to lay the foundation of a EU-wide data sharing space for Real World Evidence (RWE) outcomes for better care in AD. By 2018, the ROADMAP public private consortium with its 24 EU partners, coordinated by the University of Oxford and Novartis, aims to deliver guiding principles and recommendations on integrating available RWE for healthcare decision making within AD. Through the involvement of different stakeholders, ROADMAP will identify and prioritise AD outcomes of individual and disease relevance and health-economic importance. To accomplish this, data from 6 EU Member States involving 75 national databases and clinical registries will be used.

What ROADMAP is doing to advance neurodegeneration research

ROADMAP will explore how different real world data sources can model disease progression in AD, allowing future treatments to be more closely linked to patient outcomes. Further, it will model long-term economic impacts of different disease trajectories and treatment pathways. Analysis results and stakeholder engagement will be used to identify data and knowledge gaps. This information will be used to lay the foundation for a long-term European-based world-leading RWE environment.

Project members attending the event

Catherine Reed
Principal Research Scientist, Global Patient Outcomes/Real World Evidence (GPORWE), Eli Lilly

Laura Campo
Advisor, Global Advocacy, Eli Lilly

Martin Pan
Associate Director, EU+Canada Medical, Alzheimer's Disease, Biogen

Robert Hyde
Senior Director, EU+Canada Medical, Clinical Research and Real World Evidence, Biogen

Bjoern Sperling
Senior Medical Director, Clinical Development
Biogen

Project start date 01/11/2016 • end date 31/10/2018


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Key stakeholders in neurodegeneration research

Alzheimer's Association

The Alzheimer's Association is the leading voluntary health organisation in Alzheimer's care, support and research.

Our mission is to eliminate Alzheimer's disease through the advancement of research, to provide and enhance care and support for all affected, and to reduce the risk of dementia through the promotion of brain health.

Our vision is a world without Alzheimer's disease. For more information, visit www.alz.org.

What the Alzheimer's Association is doing to advance neurodegeneration research

- World's largest, most impactful non-profit funder of Alzheimer's and dementia science, spanning full spectrum of field's research needs.
- Convene Alzheimer's Association International Conference (AAIC), the world's leading conference for dementia science.
- Lead numerous scientific efforts to advance our understanding of disease, address gaps in our knowledge, and develop necessary resources for the scientific community.
- Lead public policy to increase research funding in US and around world.

Organisation representative attending the event

Heather Snyder
Senior Director, Medical & Scientific Relations
Alzheimer's Association

Alzheimer Europe

Alzheimer Europe is a non-governmental organisation founded in 1989 which counts 39 Alzheimer's associations from 34 countries as its members. The organisation is a lobbying organisation promoting dementia awareness, care and research within the EU institutions. The organisation considers its mission as "changing perceptions, practice and policy in order to improve the quality of life of people with dementia and their carers".

Alzheimer Europe also actively contributes to the following IMI projects: AETIONOMY, AMYPAD, EMIF, EPAD, MOPEAD, PharmaCog and ROADMAP.

What Alzheimer Europe is doing to advance neurodegeneration research

Alzheimer Europe actively supports EU projects by representing the views of people with dementia and their carers, by contributing to the discussions of the ethical issues raised by the project and by leading or supporting dissemination ctivities about project activities towards the wider dementia community.

In addition, Alzheimer Europe lobbies for the development of national and European dementia strategies with increased funding for research and greater collaboration at EU level.

Organisation representative attending the event

Jean GEORGES
Executive Director
Alzheimer Europe

Centre for the Development of Industrial Technology (CDTI), Spain

CDTI is a public business entity under Spain's Ministry of Economy, Industry and Competitiveness. Among its competencies, the management of national and international R&D programmes is to be highlighted. Besides this, CDTI carries out financing of R&D projects developed by Spanish companies and performs the evaluation of calls for national R&D programmes.

Its European Union R&D Programmes Division carries out the management of the different thematic areas under the pillars of Leadership Industrial Technologies and Societal Challenges of H2020, performing as NCP, and having a National Delegate in all the management committees.

What CDTI is doing to advance neurodegeneration research

CDTI finances R&D projects in all fields, mainly in a bottom-up approach. Considering neurodegeneration research, CDTI has supported industrial projects (with or without academic subcontractors) for the period of 2010-2016 with around 57 M€.

Moreover, CDTI supports the Spanish participation in International Programmes: H2020, JPND,Eureka, Iberoeka and other bilateral programmes, in which neurodegeneration research support is included for different types of entities.

Organisation representative attending the event

Marta Gómez Quintanilla

Chair of the IMI States Representatives Group

Spanish Health & IMI representative – H2020

Spanish delegate in JPND

Spanish Centre for the Development of Industrial Technology

Critical Path Institute (C-PATH)
Coalition Against Major Diseases (CAMD)

CAMD is one of twelve consortia of the Critical Path Institute, a non-profit organisation delivering on the mission outlined by the U.S. Food and Drug Administration's Critical Path Initiative. CAMD aims to create Drug Development Tools (DDTs) and advance regulatory science that increases the efficiency of the development process, and accelerates the delivery of innovative treatments for cognitive decline and dementia. While the primary focus is Alzheimer disease (AD), a disease with tremendous unmet medical need, the consortium is also engaged in understanding the common elements of dementia across other neurodegenerative diseases (e.g., Parkinson disease, Multiple Sclerosis, Huntington disease, etc.).

CAMD advances:

- Regulatory science readiness for biomarkers and DDTs including imaging (hippocampal vMRI), CSF fluid biomarkers (amyloid & tau analytes), Biometric Monitoring Devices that assess physical activity/frailty, sleep, and cognition
- CDISC standard development that supports database modelling and simulation of disease progression, and creation of clinical trial simulation tools for pre-dementia AD
- The creation of concise Informed Consent Forms (ICFs) that enable future data sharing

Organisation representatives attending the event

Inish M. O'Doherty
Director, Transplant Therapeutics Consortium (TTC)
Director, Type 1 Diabetes (T1D) Consortium
Critical Path Institute

Stephen P Arnerić
Executive Director, Coalition Against Major Diseases (CAMD**)**
Critical Path Institute

Graham Higson
Executive Director, European Office
Critical Path Institute

Dementia Discovery Fund

The DDF is a highly innovative new fund backed by six of the world's leading pharmaceutical and biotech companies, the UK Government and Alzheimer's Research UK to discover and develop new disease-modifying drugs for dementia. The time is ripe to find new treatments that improve the lives of dementia sufferers and slow or stop dementia's progression. A recent explosion in, for example, genetic data means we now have a better understanding of the biology of the different diseases causing dementia. The DDF will invest directly in early projects and companies with innovative ideas for novel dementia drug discovery.

What the Dementia Discovery Fund is doing to advance neurodegeneration research

The DDF is focused exclusively on identifying, funding and supporting new approaches to treating dementia. To date, we have invested in eight companies or projects at stages ranging from target or platform exploration to preclinical development. These investments represent a variety of novel, non-amyloid approaches to dementia and, if successful, would create precedent for further public and private funding of new approaches.

Organisation representative attending the event

Tetsuyuki Maruyama
Chief Scientific Officer
Dementia Discovery Fund Venture Fund

European Brain Council

The European Brain Council (EBC) is a non-profit organisation gathering patient associations, major brain-related societies as well as industries. Established in March 2002, its mission is to promote brain research in order to improve the quality of life of those living with brain disorders in Europe. 165 million Europeans are living with a brain disorder, causing a global cost (direct and indirect) exceeding 800 billion euros for the National Health budgets.

What the European Brain Council is doing to advance neurodegeneration research

EBC Value of Treatment for Brain Disorders Research Project (2015-2017) aims to assess the treatment gap and the cost of non- (or inadequate) treatment, and promote a holistic healthcare approach. Value of Treatment case studies address the obstacles to effective implementation of early diagnosis and treatment, and illustrate large variations across health systems. At the final conference on 22 June 2017, case studies will be showcased demonstrating health gains and socio-economic impacts of best practice health care interventions. A policy white paper will be released.

Organisation representatives attending the event

Vinciane Quoidbach
Public Health& Policy, Research Project Manager
European Brain Council

European Commission
Directorate General for Communications Networks, Content and Technology (DG CONNECT)

Inside the European Commission, Directorate-General for Communications Networks, Content and Technology, the Unit on Future and Emerging Technologies (FET) Flagships manages a portfolio of pilot research initiatives, namely the Graphene Flagship and the Human Brain Project (HBP). They are funded in the context of the EU Framework Programmes for Research and Innovation, currently Horizon 2020. FET Flagships aim at delivering major breakthroughs in science and technology, as a novel approach for Europe to address grand scientific challenges and boost innovation. The Unit also prepares the ground for future Flagships.

What DG CONNECT is doing to advance neurodegeneration research

- The Human Brain Project addresses the challenge of understanding the human brain and build a cutting-edge ICT-based research infrastructure for neuroscience, computing and brain-related medicine.
- Federating and analysing patients' data will allow researchers to identify the biological signatures of disease, enable early diagnosis and personalised medicine, and then simulate disease in brain models, to probe the causes of neurological and psychiatric disease, and to screen putative treatments.

Organisation representative attending the event

François Junique
Programme Officer - FET Flagships
Directorate General for
Communications Networks, Content
and Technology
(DG CONNECT)
European Commission

European Commission
Directorate General for Health and Food Safety (DG SANTE)
Unit B5 - Medicines: policy, authorisation and monitoring

Inside the European Commission, Directorate-General for Health and Food Safety (DG SANTE), the Unit B5 on Medicines: policy, authorisation and monitoring manages marketing authorisation procedures for medicinal products authorised at the EU level, works with the European Medicines Agency (EMA) in order to ensure that medicinal products placed on the EU market conform to the EU standards on safety, quality and efficacy, acts as entry point for all aspects concerning the EMA, manages and implements EU regulatory framework on medicinal products for human use and promotes international cooperation in the area of medicinal products and regulatory dialogue.

What DG SANTE is doing to advance neurodegeneration research

- Management of initiatives to facilitate the authorisation of innovative products for unmet medical needs.
- Holds a dialogue with Member States and other stakeholders within the expert group on Safe and Timely Access to Medicines for Patients (STAMP).

Organisation representative attending the event

Dagmar Stara
Principal administrator
Unit B5 – Medicines: policy, authorisation and monitoring,
Directorate General for Health and Food Safety (DG SANTE)
European Commission

European Commission
Directorate General for Research & Innovation (DG RTD)
Neuroscience Sector

Within the Unit "Non-communicable diseases and the challenge of healthy ageing" of DG Research & Innovation, the Neuroscience sector is responsible for developing strategies and contributing to the implementation of the Innovation Union and the EU Framework Programmes for research in the area of neuroscience.

The sector is also in charge of some major EU research initiatives in the area of neuroscience such as the Joint Programming Initiative on Neurodegenerative Disorders (JPND), the International Consortium on Traumatic Brain Injuries (InTBIR) and the Network of national funding organisations on disease-related neuroscience (NEURON).

Organisation representatives attending the event

Stephan Hogan
Head of Sector for Neuroscience
Directorate General for Research & Innovation (DG RTD)
European Commission

Lara Passante
Responsible neurodegenerative diseases & brain imaging portfolios
Directorate General for Research & Innovation (DG RTD)
European Commission

Mark Goldammer
Programme and Project Officer
Directorate General for Research & Innovation (DG RTD)
European Commission

European Medicines Agency (EMA)

The mission of the European Medicines Agency (EMA) is to foster scientific excellence in the evaluation and supervision of medicines, for the benefit of public and animal health in the European Union (EU). EMA is a decentralised agency of the European Union (EU), located in London. It began operating in 1995. The Agency is responsible for the scientific evaluation, supervision and safety monitoring of medicines developed by pharmaceutical companies for use in the EU. EMA protects public and animal health in 28 EU Member States, as well as the countries of the European Economic Area, by ensuring that all medicines available on the EU market are safe, effective and of high quality. Main activities include:

- Provision of independent, science-based recommendations on the quality, safety and efficacy of medicines;
- Application of efficient and transparent evaluation procedures to help bring new medicines to the market by means of a single, EU-wide marketing authorisation granted by the European Commission;
- Implement measures for continuously supervising the quality, safety and efficacy of authorised medicines to ensure that their benefits outweigh their risks;
- Provide scientific advice and incentives to stimulate the development and improve the availability of innovative new medicines;
- Involves representatives of patients, healthcare professionals and other stakeholders in its work, to facilitate dialogue on issues of common interest.

EMA serves a market of over 500 million people living in the EU.

What EMA is doing to advance neurodegeneration research

- The EMA Committee for Medicinal Products for Human Use (CHMP) can issue Scientific Advice (SA) and a qualification opinion on the acceptability of a specific use of a method, such as the use of a novel methodology or an imaging method in the context of research and development. The method can apply to non-clinical or to clinical studies, such as the use of a novel biomarker.
- CHMP has given over 100 scientific advices, qualification advices/opinions and Health Technology Advise -Scientific advise – ((HTA/SA) over the last 10 years.

Organisation representatives attending the event

Manuel Haas

Head of CNS Office

Evaluation Division

EMA

Maria Isaac

Senior Scientific Officer

Product Development Scientific Support Department

EMA

European Research Council (ERC)

The European Research Council (ERC) supports investigator-driven research by awarding long-term grants to individual researchers worldwide to carry out research in an EU or associated country. It is a component of Horizon 2020. Three schemes support investigators at different stages of their career to run five-year-projects with individual budgets from EUR 1.5 to 3.5 million: Starting Grants provide young researchers conditions to become independent, Consolidator Grants support researchers to strengthen recently created teams, Advanced Grants support well-established researchers to develop innovative high-risk research. To date, the ERC has funded over 6,500 researchers.

What the European Research Council (ERC) is doing to advance neurodegeneration research

The ERC is particularly active in funding research in all domains of neuroscience and neurology, with over 700 projects supported for a granted budget over EUR 1 billion. Among these projects, a large share tackles neurodegeneration with novel or interdisciplinary approaches, from genetics and molecular studies to cognitive neuroscience and clinical application.

European Research Council's representatives attending the event

Nicolas Voilley
Coordinator for Neuroscience
European Research Council (ERC)

Cary Esselens
Scientific Officer for Neuroscience
European Research Council (ERC)

Janka Matrai
Scientific Officer for Neuroscience
European Research Council (ERC)

European Federation of Pharmaceutical Industries and Associations (EFPIA)

The European Federation of Pharmaceutical Industries and Associations (EFPIA) represents the pharmaceutical industry operating in Europe. Through its direct membership of 33 national associations and 40 leading pharmaceutical companies, EFPIA is the voice on the EU scene of 1,900 companies committed to researching, developing and bringing to patients new medicines that will improve health and the quality of life around the world. EFPIA is a founding member of IMI.

Organisation representatives attending the event

Magda Chlebus
Director Science Policy
EFPIA

Nicolas Creff
Senior Manager Research Partnerships
EFPIA

European Biopharmaceutical Enterprises (EBE)

European Biopharmaceutical Enterprises, EBE, is the European industry association representing biopharmaceutical companies of all sizes. A specialised group of EFPIA, the European Federation of Pharmaceutical Industries and Associations, EBE is Europe's expert voice for emerging bioscience & technology and the leading platform for health innovation ecosystems. Our members provide an active forum for stakeholder engagement where emerging knowledge and expertise meet to accelerate to therapeutic innovations by patients. EBE focuses on expertise in Innovation & funding models, Emerging biotech & science for regulatory advancement and advocacy, Personalised Medicine, Biomanufacturing, Advanced Therapies and Biotherapeutics, including biosimilars and microbiome-based therapies. Whilst not a singled out work topic within EBE, our members contribute to advancing research on neurodegeneration through their company R&D programs. EBE members participate in IMI projects. EBE enables its members to find innovative funding solutions to continue their work on neurodegeneration. Our working group on Personalised Medicine also supports biomarker research and development of meaningful biobanks.

Organisation representative attending the event

Barbara Freischem
Executive Director
European Biopharmaceutical Enterprises, EBE

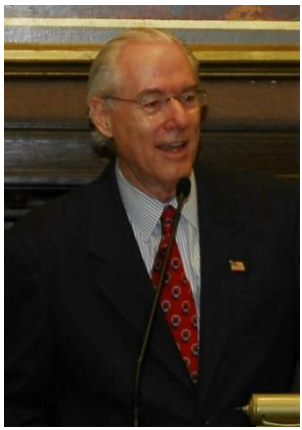
Global CEO Initiative on Alzheimer's Disease (CEOi), a network of UsAgainstAlzheimer's

CEOi is a patient-powered coalition, organised under UsAgainstAlzheimer's, that works with private sector, governments and international organizations to stop Alzheimer's by 2025. CEOi is committed to five priorities, including: 1) accelerating the clinical trial process; 2) increasing investment in Alzheimer's disease research and care; 3) improving care delivery; 4) engaging regulators and payers; and 5) driving innovation using big data. A significant milestone for the organization, through our commitment to these priorities, CEOi created The Global Alzheimer's Platform (GAP), a standing global, trial-ready platform to drive quality, efficiency and innovation in clinical trials.

What CEOi is doing to advance neurodegeneration research

- Created The Global Alzheimer's Platform (GAP) to create a standing clinical trial infrastructure that speeds drug development.
- Developed a predictive algorithm to understand who and when a person is likely to develop mild cognitive impairment and Alzheimer's disease years before the symptoms emerge.
- Engaging patients, payers and regulators to drive forward patient-focused drug development.

Organisation representative attending the event



George Vradenburg
Convener, The Global CEO Initiative on Alzheimer's Disease;
Co-Founder and Chairman, UsAgainstAlzheimer's;
Chair, Board of Directors, Global Alzheimer's Platform Foundation

EU Joint Programme for Neurodegenerative Disease Research (JPND)

JPND is the largest global research initiative aimed at tackling the challenge of neurodegenerative diseases. Although initially a European programme, JPND has since gone global, with 30 countries now participating. JPND aims to increase coordinated investment between countries in research aimed at finding causes, developing cures and identifying appropriate ways to care for those living with neurodegenerative diseases, with a specific focus on Alzheimer's disease and other dementias, Huntington's disease, motor neurone diseases (such as ALS), Parkinson's disease and related disorders, prion disease, Spinal muscular atrophy and Spinocerebellar ataxia.

What JPND is doing to advance neurodegeneration research

Since it was established in 2009, JPND has been increasing the effectiveness and impact of neurodegenerative disease research around the world by facilitating the coordination of current and future approaches, aligning national research programmes, collaborating where appropriate and by sharing tools, techniques and other resources more efficiently among participating countries.

Organisation representative attending the event

Adriana Maggi
Vice-chair
Joint Programming for
Neurodegenerative Diseases (JPND)

Human Brain Project – Medical Informatics Platform

The Medical Informatics Platform (MIP), one of the six Human Brain Project platforms, was released begin 2016 aiming to advance our understanding of brain's function and its disorders. It allows for interactive access to clinically relevant information about the healthy and diseased brain. MIPs collaborative framework provides the opportunity to federate, analyse and interpret data from hospitals and research centres in Europe. Currently, we integrate data from 5 University hospitals and a major research initiative in Traumatic Brain Injury. Among the achieved milestones we consider not only the distributed analyses of eHRs, brain and omics data, but also the discovery of new dementia subtypes representing unique biological signatures of diseases.

What the Medical Informatics Platform is doing to advance neurodegeneration research

Utilising state-of-the-art technology, neuroimaging and optimised analytic techniques, the MIP helps researchers make huge strides toward a clearer understanding of brain diseases.

One of our important goals is to characterise complete disease pathways, from the molecular level up to observable disorders of cognition and behaviour. The aim is to identify the biological disease signatures (unique combinations of biological and clinical signals) associated with specific pathways. Identifying disease signatures will make it possible to derive causal models of diseases and treatments, preparing the way for new techniques of personalised medicine.

Organisation representative attending the event

Ferath Kherif
Leader HBP – Medical Informatics Platform
Lausanne University Hospital (Centre Hospitalier Universitaire Vaudoise)

IRCCS-Mario Negri Institute for Pharmacological Research

Non-profit organisation and an internationally recognised centre of excellence in the field of biomedical research since 1963. The Institute features six departments on various biomedical disciplines including neuroscience, oncology, public health, cardiovascular research, environmental health sciences**,** and molecular biochemistry. The activities aim to integrate different expertise and background knowledge to elucidate the etiopathogenesis of human diseases and develop effective therapeutic strategies. Training programmes in the Institute include Master's School, post-doc programme and a PhD school in collaboration with the Open University (UK). The Neuroscience department studies CNS diseases: epilepsy, Alzheimer and prion's diseases, amyotrophic lateral sclerosis, stroke and drug abuse.

What our Institute is doing to advance neurodegeneration research

- Models of the misfolding protein aggregation in Alzheimer's, Parkinson's and prion diseases, fronto-temporal dementia.
- Models of familial ALS with different disease progression to identify prognostic markers and therapeutic targets.
- Biobank of human samples finalized to the genetic studies and the identification of new biological markers.
- Acute brain injury models of neurotrauma, haemorrhagic lesions, stroke, and seizures. Parallel exploration of mechanisms in experimental and clinical settings to refine models and develop neurorestorative treatments.
- Clinical epidemiology, clinical course, clinical trials and observational studies.

Organisation representative attending the event

Annamaria Vezzani
Member of the IMI Scientific Committee
Strategic Governing Group Neurodegeneration
PhD, Head of Exp Neurology Laboratory,
Department of Neuroscience
IRCCS-Mario Negri Institute for
Pharmacological Research, Milano, Italy

Medical Research Council (MRC)

The Medical Research Council (MRC) is the main Government agency responsible for UK biomedical research. In 2015/16 the MRC spent £928m on world-class research across the biomedical spectrum, from fundamental lab-based science to clinical trials. The heart of the MRC's mission is to improve human health by supporting excellent science, and training the very best scientists. The MRC works closely with the NHS and the UK Health Departments to deliver its mission, and give a high priority to research that is likely to make a real difference to clinical practice and the health of the population. MRC funds research on neurodegenerative diseases primarily through the Neurosciences and Mental Health Board.

What the Medical Research Council is doing to advance neurodegeneration research

Neurodegenerative disease research has been a major part of the MRC's scientific strategy since 2008 and currently the Council is establishing a £250m UK Dementia Research Institute (DRI) together with Alzheimer's Society and Alzheimer's Research UK. MRC launched the Dementias Research Platform UK (DPUK) in 2014 and plays a leading role in the transnational initiatives Joint Programming in Neurodegenerative Disease (JPND) and the network for Centres of Excellence in Neurodegeneration (CoEN).

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Organisation representative attending the event

Simon Fisher
Programme Manager (JPND)
Medical Research Council

National Institute for health and Care Excellence (NICE)

NICE is responsible for producing evidence based guidance and advice, quality standards and performance metrics for health, public health and social care. NICE guidance is officially for England but there are agreements to provide certain NICE products and services to Wales, Scotland and Northern Ireland. NICE routinely commissions or undertakes secondary research to support guidance production and is also involved in primary research, such as observational research to support NICE Interventional Procedure and Medical Technology guidance.

What NICE is doing to advance neurodegeneration research

- NICE is a partner in the Innovative Medicine Initiative's ROADMAP project (http://www.roadmap-alzheimer.org/). It is a multi-national public-private partnership, which aims to provide the foundation for a Europe-wide real-world evidence platform on Alzheimer's disease.
- The project provides a unique opportunity to gather evidence from a range of sources, such as electronic health records, and discover what outcomes are important to patients and carers. Ultimately, the aim of setting up such a platform is to facilitate and accelerate bringing new and much-needed treatments to patients.

Organisation representatives attending the event

Pall Jonsson
Senior Scientific Adviser
NICE

Jacoline Bouvy
Scientific Adviser
NICE

Parkinson’s UK

Parkinson's UK is the largest charity funder of research into Parkinson's in the UK. Its aims are to improve the quality of life for people affected by Parkinson's and find a cure for the condition. It funds research aimed at finding better treatments and improving the understanding of Parkinson's and its causes, and raises funding through donations, legacies, community fundraising, events and corporate partnerships. The charity offers support and information to people affected by Parkinson's, their families and carers through a network of 350 local groups across England, Wales, Scotland and Northern Ireland.

What Parkinson UK is doing to advance neurodegeneration research

At Parkinson's UK, everything we do is driven by people with Parkinson's, so we are striving for new and better treatments in years, rather than decades. People with the condition and their families are setting the research agenda, making decisions about the projects we fund and working in partnership with researchers at every stage. Parkinson's UK is the largest member-led charitable funder of Parkinson's research in Europe. So far, we have invested more than £80million in ground-breaking research. We are focused on finding new and better treatments for Parkinson's, and one day a cure. We are investing in big ideas that will speed up the research process, and deliver treatments to the people who need them faster. We are also keen to fund research that improves quality of life in the shorter term

Organisation representative attending the event

Arthur Roach
Director of Research:
Parkinson's UK

World Dementia Council

Established after the G8 dementia summit in December 2013, the World Dementia Council unites leading experts from across the global dementia community including researchers, academics, non-governmental organisations, industry, regulators, public sector and people living with dementia with the shared ambition to find solutions to this devastating condition. With 24 members across six continents, the World Dementia Council has a unique role to play in changing the course of dementia and supporting millions of people living with this devastating disease.

What the World Dementia Council is doing to advance neurodegeneration research

We aim to achieve a world where society, governments, industry, researchers and health and care systems have worked together to transform the prospects for people affected by Alzheimer's and other forms of dementia so that the diseases no longer destroy lives in the way they do today. The WDC has five core priorities: 1) Finance: advancing levels of innovative and global public and private finance; 2) Integrated Development: Increasing the speed and reliability of delivering innovative medicines through efficient and effective integrated drug development; 3) Research, Open Science and Data: fostering a culture of open science and collaborative global research, including the use of bid data approaches; 4) Care: Ensuring the quality of life and delivery of quality care for people living with dementia and their carers; 5) Risk Reduction: Reducing the risk of dementia through lifestyle and other approaches.

Organisation representative attending the event

Raj Long
Vice Chair & Senior Advisor – Integrated Development
World Dementia Council
Bill & Melinda Gates Foundation

US Food & Drug Administration (FDA)

The Food and Drug Administration (FDA) is responsible for protecting the public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, and medical devices; and by ensuring the safety of our nation's food supply, cosmetics, and products that emit radiation.

FDA is responsible for advancing the public health by helping to speed innovations that make medical products more effective, safer, and more affordable and by helping the public get the accurate, science-based information they need to use medical products and foods to maintain and improve their health.

Since its enactment in 1938, FDA has seen an enormous increase in the magnitude and complexity of its regulatory responsibilities. New areas of science, evolving technologies, and globalization have profoundly altered the Agency's regulatory landscape. Today FDA's regulatory activities range from developing new models to assess the safety of gene therapy to building a new prevention-based food safety system for a globalised economy and creating a national electronic system that will track the safety of FDA-regulated medical products once they reach the market.

Organisation representative attending the event

Ameeta Parekh
Senior Advisor for Scientific Collaborations
Center for Drug Evaluation and Research Office of Translational Sciences
U.S. Food and Drug Administration

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