Topic: Support and coordination action for the projects of the Neurodegeneration area of the Innovative Medicines Initiative

All information regarding future IMI Call topics is indicative and subject to change. Final information about future IMI Calls will be communicated after approval by the IMI Governing Board.

Topic details

Action type	Coordination and Support Action (CSA)
Submission & evaluation process	2 Stages

Specific challenges to be addressed

Dementia affects over 47 million people globally. As populations age, this figure is projected to increase to 75.6 million by 2030, and more than triple by 2050. The disease places a huge and growing burden on health and social care systems as well as on the families and carers of those affected. Yet despite decades of research and large investments, there is still neither treatment nor cure for the disease and success in clinical trials remains elusive.

There are significant medical, scientific, ethical, regulatory, and operational issues around the question of what can be done to support biomedical research and health innovation for the delivery of the required diagnostics and disease-modifying treatments for Alzheimer's disease (AD) and other dementias.

After the G8 meeting in London December 2013[1], a significant increase was observed in the number of initiatives focused on advancing the field of dementia research. These initiatives while geographically diverse are mostly either of public-private nature with aim to optimise pre-competitive collaboration and knowledge generation or large collaborative public efforts which deliver innovative results that would benefit from further translation to practice (see figure 1). The Innovative Medicines Initiative (IMI) projects "European Medical Information Framework" (**EMIF -** http://www.emif.eu/), "Organising mechanistic knowledge about

[1] https://www.gov.uk/government/publications/g8-dementia-summit-global-action-against-dementia/g8-dementia-summit-global-action-against-dementia-11-december-2013

neurodegenerative diseases for the improvement of drug development and therapy" (**AETIONOMY -** https://www.aetionomy.eu/en/vision.html) and "European prevention of Alzheimer's dementia consortium" (**EPAD -** http://ep-ad.org/) stated their willingness for collaboration in March 2015, creating the IMI Alzheimer's Research platform[2]. After that, and in just the first three years of its activities, IMI2 JU implemented eight new projects in the area of neurodegeneration and more are in the pipeline. These initiatives have been launched either via the Strategic Governing Group Neurodegeneration (**SGG ND -** https://www.imi.europa.eu/content/strategic-governing-groups#neurodegeneration), or as part of platforms such as the Remote assessment of disease and relapse (**RADAR -** https://www.radar-cns.org/ and http://ec.europa.eu/research/participants/portal/desktop/en/opportunities/h2020/topics/imi2-2017-12-01.html) and Big Data for Better Outcomes (**BD4BO -** http://bd4bo.eu/) ones.

These diverse initiatives now cover the Research and Development (R&D) value chain from bench to bedside (see figure 1). Although several of these initiatives have started leveraging on one another, an operational coordination of the activities promoted by all these actors is missing, and despite the growing number of initiatives, there are no agreed metrics to show their value in advancing research and remove bottlenecks toward the delivery of innovative treatments to patients.

There is a constant need for strengthening the information flow and enhancing the exchange of experience on on-going and future European and international research and innovation activities concerning neurodegeneration, at IMI level and beyond, as well as for maintaining continuous dialogue between all stakeholder groups and initiatives to allow an evaluation on how the investment is impacting the area.

Effective and efficient collaboration and coordination among the IMI portfolio of projects in the area of Neurodegeneration and related national, European and global initiatives is a key success factor for the important public-private investment to achieve its full impact, as also highlighted in a recent meeting hosted in Brussels by the IMI2 JU[3].

It also is also evident that projects share several areas of common interest (e.g. modelling and simulation, imaging) and have developed best practices that would be very useful for other ongoing and upcoming initiatives, but due to the silo-like structure of the individual initiatives, the opportunity for real and effective cross fertilisation is limited and based on the "good will" of enthusiastic individuals. Indeed this has been the case with the IMI Alzheimer's Research Platform, which links three projects which have some specific complementaries, however now the portfolio of IMI projects has grown significantly and it is much more diverse in scope and focus, making the need for a more structural and tailor-made support structure mandatory.

Projects would also benefit from support (including access to learnings from other projects) towards the submission of results for regulatory and/or health-technology assessment (HTA) to ensure that important results can timely impact regulatory practice and the health care system. Often the data to support a regulatory/HTA submission are only fully available in the very final phase of the projects or even after their official end which may hamper their submission and subsequent follow up.

A significant challenge in collaboration is the burden required to develop agreements and good practices for sharing and re-use of data, biological tools (e.g. cell lines) and other materials, activities that are normally either not or only minimally resourced under individual initiatives and can be labour intensive and require expertise (e.g. legal, ethical) not always readily available for each project.

All projects face the challenge of sustainability of their results, and the lack of a source of advice and support in finding/choosing relevant solutions beyond the project lifetime. There is therefore a clear need for support to ensure that collaboration and coordination become intentional and structural to the portfolio of projects in the IMI strategic area of neurodegeneration, by providing the necessary resources and framework.

[2] http://www.imi.europa.eu/content/press-release-imi-ad-platform
[3] http://www.imi.europa.eu/events/2017/09/21/collaboration-alzheimer%E2%80%99s-disease-beyond

Last but not least there would be a very high value in having a framework to facilitate collaboration and coordination of the many initiatives focussed on neurodegeneration, in and beyond IMI, and to develop some metrics to show their value in advancing research and remove bottlenecks toward the delivery of innovative treatments to patients.

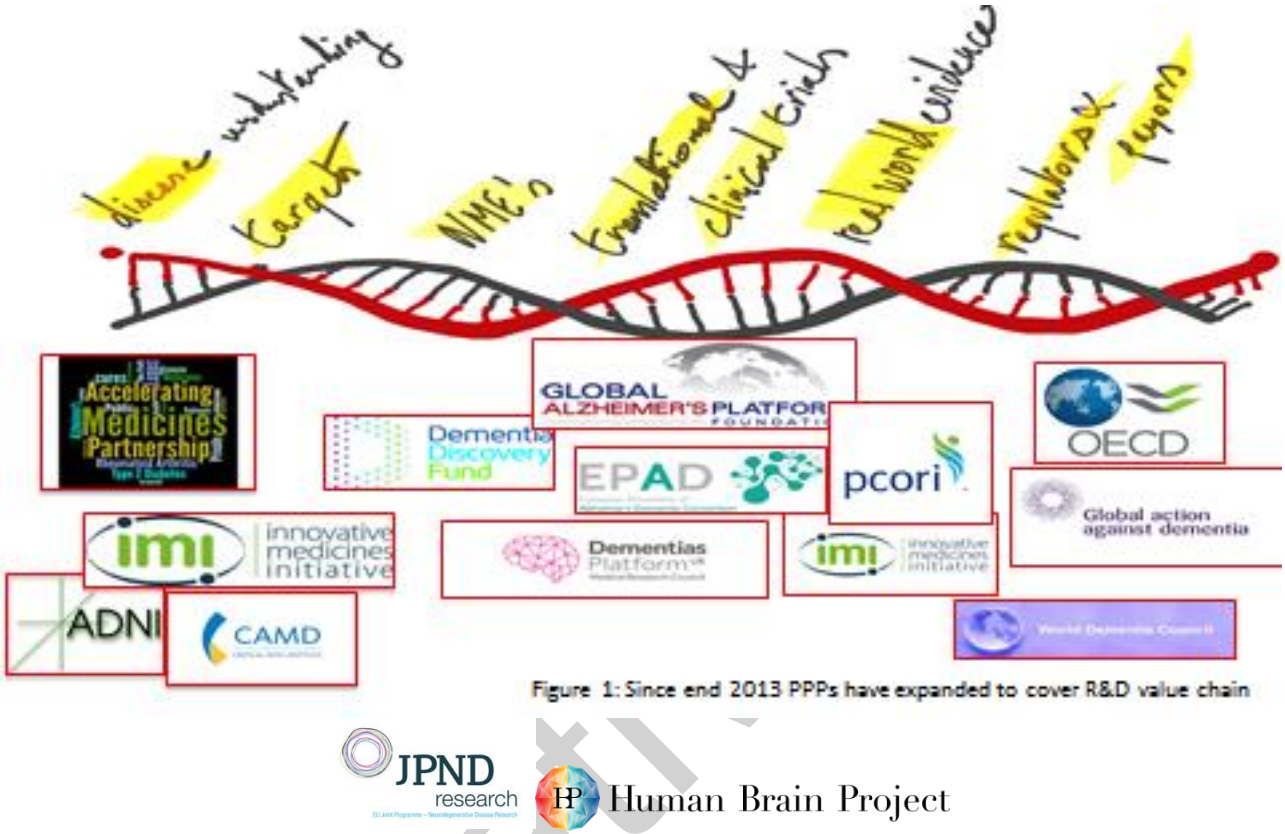

Figure 1: Since end 2013 PPPs have expanded to cover R&D value chain

Scope

The overall scope of the coordination and support action is to provide the necessary overall framework and resources to achieve effective and efficient coordination and collaboration among the ongoing and future projects in the IMI strategic area of neurodegeneration. This will include:

- Develop a framework to coordinate and support the operational alignment of the IMI Neurodegeneration research portfolio, including a process to ensure that projects make appropriate use and can access existing infrastructures;
- Provide expert advice and other support to facilitate sharing and access of data, biological tools and other materials among projects;
- Provide expert advice and support in preparing for regulatory and/or HTAs interactions (e.g. legal support, access to relevant expertise, funding to pay submission fees) to ensure that appropriate regulatory input is provided when most valuable and also beyond the timeframe of a project;
- Establish and manage workshops designed to share common approaches/best practice across IMI projects and beyond;

- Develop a framework to coordinate and efficiently support the operational alignment of the IMI led actions with other relevant partnerships and initiatives at national, European and global level (e.g. DPUK[4], DZNE[5], JPND[6], CAMD[7], NIH/AMP[8], WHO[9], GAP[10], World Dementia Council[11]);
- Create a platform to enable the mapping of partnerships and collaborative efforts that have supported over the past years research in Alzheimer's disease to capture their contributions and identify the remaining gaps and develop metrics and benchmarks to measure value, including socio-economic impact;
- Develop outreach and engagement actions with other international/national/regional initiatives including patient organizations to promote and increase the value of trans-national and international research collaborations;
- Communicate and disseminate on joint activities and initiatives in the field of neurodegenerative diseases;
- Seek alignement and coordination on issues of common interest such as Ethical, Legal and Social Implications of clinical neurodegenerative disease (especially Alzheimer's disease) research, where several learnings are already available but disperse.

Expected key deliverables

- An operational platform to coordinate and support the activities of the IMI neurodegeneration projects, including new relevant IMI2 actions and international collaborations. Assure that cross project dependencies/synergies are operationally supported enabling actual delivery on them. Such platform should be developed including consideration for self-sustainability beyond the action funding;
- A resource to enable timely and effective interaction with regulatory authorities and HTAs;
- A series of guidelines and good practices for the access and sharing of data, biological tools and other materials among projects, as well as a resource to facilitate the process;
- An advisory board and an up-to-date and dynamic catalogue of potential solutions for sustainability of project results;
- A series of workshops and relevant proceedings to share common approaches/best practice across IMI projects;
- Metrics and benchmark to measure success;
- A public depository (to be self-sustainable after the end of the action) for protocols, deliverables, white papers etc produced by the action and by the IMI projects to insure their optimal dissemination to the wider scientific community;
- A relevant programme of outreach activities;
- Joint white papers to provide an aligned and educated perspective of all key stakeholders on key issues in the area of neurodegenation research, including regulatory and HTA perspectives;
- A map of the partnerships and collaborative efforts that have supported over the past years research in Alzheimer's disease to capture their contributions and identify the remaining gaps.

[4] DPUK – D ementias Platform UK: https://www.dementiasplatform.uk/
[5] DZNE – Forschungszentrum für neurodegenerative Erkrankungen: http://www.dzne.de/home.html
[6] JPND – Neurodegenerative Disease Research: http://www.neurodegenerationresearch.eu/
[7] CAMD – Coalition Against Major Diseases: https://c-path.org/programs/camd/
[8] NIH/AMP – National Institutes of Health / Accelerating Medicines Partnership: https://www.nih.gov/research-training/accelerating-medicines-partnership-amp
[9] WHO – World Health Organization: http://www.who.int/en/
[10] GAP – Global Alzheimer's Platform: http://globalalzplatform.org/
[11] World Dementia Council: https://worlddementiacouncil.org/

Expected impact

The expected impact would be:

- An enhanced impact of the individual projects by creating structural synergy and collaboration;
- An enhanced visibility of the significant public and private investment of IMI in the area of Neurodegeneration;
- To ensure the results of IMI projects are developed optimally for the benefit of patients and the health systems including strategies for sustainability and uptake;
- An optimization of the impact of IMI projects' activities in neurodegeneration toward the achievement of the IMI2 Council Regulation objectives and in particular those aiming to:
    - Increase the success rate in clinical trials of priority medicines identified by the World Health Organisation;
    - where possible, reduce the time to reach clinical proof of concept in medicine development, such as for cancer, immunological, respiratory, neurological and neurodegenerative diseases;
    - develop new therapies for diseases for which there is a high unmet need, such as Alzheimer's disease;
    - develop diagnostic and treatment biomarkers for diseases clearly linked to clinical relevance and approved by regulators.
- An analysis of the scientific results and achievements delivered by the various partnerships in neurodegeneration so far, and an understanding of their translation into more efficient and faster development of new medical products in this area and of critical factors for a successful translation;
- An overview and a framework to inform future collaborative research globally and facilitate the translation to innovative treatments for patients.

Potential synergies with other Consortia

Applicants should take into consideration, while preparing their short proposal, relevant national, European (both research projects as well as research infrastructure initiatives), and non-European initiatives. Synergies and complementarities should be considered in order to incorporate past achievements, available data and lessons learnt where possible, thus avoiding unnecessary overlap and duplication of efforts and funding.

The action will have to build strong links with the portfolio of IMI projects in the area of neurodegeneration[12] to ensure that the activities are in good synergy with those potentially already ongoing within individual initiatives.

It is also expected to leverage and build on efforts and lessons learnt from other initiatives and organizations at National, European and Global level (e.g. DPUK, DZNE, NIH/AMP, JPND, CAMD, WHO, GAP, World Dementia Council, among others).

Finally the action should build synergies and complementarities with other relevant coordination activities in IMI and H2020.

[12] https://www.imi.europa.eu/content/ongoing-projects

Industry Consortium

The industry consortium will contribute the following expertise and assets:

- contribution to project and meeting management;
- measurement and analytical tools;
- regulatory affairs;
- data privacy law and related legal aspects;
- medical affairs and health care communication;
- contribution to website management;
- data/knowledge management, repository of knowledge;
- experts time in different relevant scientific areas.

The industry consortium will also provide their expertise in the conduct and follow up of management tasks to secure this overall programme platform (including any IT system to help the work of the platform and the communication between partners) as well as provide the necessary resources for programme management, e.g. from defining strategic priorities to the organisation of meetings / workshops / teleconferences.

Indicative duration of the action

The indicative duration of the action is 36 months.

Applicant consortium

The applicant consortium will be selected on the basis of the submitted short proposal.

The applicant consortium is expected to address all the objectives and make key contributions to the defined deliverables in synergy with the industry consortium which will join the the selected applicant consortium in preparation of the full proposal for stage 2. This may require mobilising expertise in:

- project management and coordination;
- organisation and logistics of workshops and international meetings;
- knowledge and expertise in legal, ethics and data privacy aspects on the management of sensitive personal level data and on management of biological tools, including intellectual property (IP) considerations;
- data hosting and maintenance;
- regulatory science;
- health economics;
- medical/scientific writing;
- outreach and communication targeted for the different stakeholders and public at large;
- development of effective communication tools including websites and social media, platforms to create awareness of the programme and disseminate findings;
- expertise to create training and communication materials based on results of the programme.

Suggested architecture of the full proposal

The applicant consortium should submit a short proposal which includes their suggestions for creating a full proposal architecture, taking into consideration the industry participation including their contributions and expertise.

In the spirit of the partnership, and to reflect how IMI2 JU call topics are built on identified scientific priorities agreed together with EFPIA beneficiaries/large industrial beneficiaries, these beneficiaries intend to significantly contribute to the programme and project leadership as well as project financial management.

The final architecture of the full proposal will be defined by the participants in compliance with the IMI2 JU rules and with a view to the achievement of the project objectives. The allocation of a leading role within the consortium will be discussed in the course of the drafting of the full proposal to be submitted at stage 2. To facilitate the formation of the final consortium, until the roles are formally appointed through the consortium agreement, the proposed project leader from among EFPIA beneficiaries/large industrial beneficiaries shall facilitate an efficient negotiation of project content and required agreements. All beneficiaries are encouraged to discuss the project architecture and governance and the weighting of responsibilities and priorities therein.

The architecture outlined below for the full proposal is a suggestion. Different innovative project designs are welcome, if properly justified.

Work package 1 – Platform for coordination and collaboration (including sustainability)

- Set up and maintenance of a website including relevant web-pages tailor made for the different stakeholders, with special attention to patients;
- Implementation of the operational platform to coordinate and support the activities of the IMI neurodegeneration projects and international collaborations. Assure that cross project dependencies/synergies are operationally supported enabling actual delivery on them;
- Running of workshops and production of relevant proceedings to share common approaches/best practice across IMI projects;
- Establishment and maintenance of a public depository for protocols, deliverables, white papers etc produced by the action and by the IMI projects to insure their optimal dissemination to the wider scientific community;
- Establishment of an advisory board and set up and maintenance of up-to-date and dynamic catalogue of potential solutions for sustainability of project results.

Industry consortium contribution:

- strong programme management skills;
- expertise in value-based healthcare, Real World Evidence (RWE);
- network with leaders of IMI projects (BD4BO and others);
- governmental affairs and public policy.

Expected applicant consortium contribution:

- strong programme management skills, including roadmapping tools;
- experience in coordinating projects of similar complexity/ scale/ sustainability;
- expertise in initiatives related to health outcomes and value-based healthcare.

Work package 2 – Support for regulatory and/or HTA interactions

- Establishment of a resource (including all relevant support and best practices) to enable timely and effective interaction with regulatory authorities and HTAs.

Industry consortium and applicant consortium contribution:

The types of resources will be similar from both sides of the consortium since we anticipate there will be need for a variety of specific roles, including information/knowledge management skills, project management, business analysis, healthcare systems expertise, expertise on outcome definition, measurement tools, etc. for standardization of methodologies across diseases, Health Econmics and Outcomes Research (HEOR) expertise, knowledge about health funding models and various coordinating activities.

Work package 3 – Communication, Dissemination and Outreach

- Alignment of dissemination and communication strategies across the projects;
- Joint white papers to provide an aligned and educated perspective of all key stakeholders on key issues in the area of neurodegeneration research, including regulatory and HTA perspectives;
- Creation of material for internal and external communication;
- Setting up of social media platforms and inventory of communication;
- Support publication and other dissemination of findings of IMI neurodegeneration projects, including through training activities;
- A relevant programme of outreach activities.

Work package 4 – Mapping and Impact analysis

- Analysis of the socio-economic impact of the IMI portfolio in neurodegeneration;
- Implementation and maintenance of a map of the partnerships and collaborative efforts that have supported over the past years research in Alzheimer's disease to capture their contributions and identify the remaining gaps.

Industry consortium contribution:

- communication (communication strategies, media, social media);
- website set up and management;
- science writers;
- events organisations;
- stakeholder engagement expertise at national and EU level with all relevant stakeholders, including but not limited to HTAs, regulators, payers, patients, medical societies, and providers;
- organisation of multi-stakeholder meetings, workshops or forums to foster stakeholder engagement.

Expected applicant consortium contribution:

- communication strategies and tools;
- health economics impact analysis;
- development/adaptation of tools, models and methods for montoring and measuring impact
- science writers;
- creating communication materials;

- creating training materials and delivering trainings;
- appropriate resource and expertise from HTAs, regulators, payers, providers, patient organizations, medical societies and other appropriate stakeholders;
- organisation of multi-stakeholder meetings, workshops or forums to foster stakeholder engagement, especially with additional Healthcare systems' stakeholders beyond the consortium.

Work package 5 – Standards and guidance for the use and re-use, access, and sharing of human samples, biological tools and data

- Implementation of a resource, including expert advice, sharing of learnings, writing of guidelines and other support, to facilitate sharing and access of data, biological tools and other materials among projects;
- Development of minimum standards (templates) for ICFs for clinical studies and other research studies;
- Development of guidance documents to facilitate the work with the generated ICF templates, including their terminology and application, and provision of guidance on related aspects of data privacy laws and regulations (e.g. concept of anonymization) for IMI/IMI2 projects and non-IMI related addressees;
- Development of standards, training and educational guidance on aspects of data privacy laws and regulations, data protection mechanisms and consequences of their application for IMI/IMI2 projects as well as non-IMI related addressees (e.g. patients).

Industry consortium contribution:

- legal expertise in connection with data privacy and related legal matters.

Expected applicant consortium contribution:

- knowledge and expertise in legal, ethics and data privacy aspects on the management of sensitive personal level data from several angles: 1) from an academic perspective as well as from the perspective of groups of academic research organizations, 2) from the perspective of healthcare SMEs, in particular biobanking SMEs or health-IT companies, 3) as well as from the perspective of national and international supervisory/regulatory authorities dealing with data protection in the healthcare context on a regular basis (ideally one participant from each interest groups); understanding of patient and physician concerns such as in patient organizations and medical associations; ethical considerations as relevant in ethics committees.