

# Accelerating research: the role of government, academia, and industry

**Moderator: Dr Hussein Manji**  
Janssen/J&J

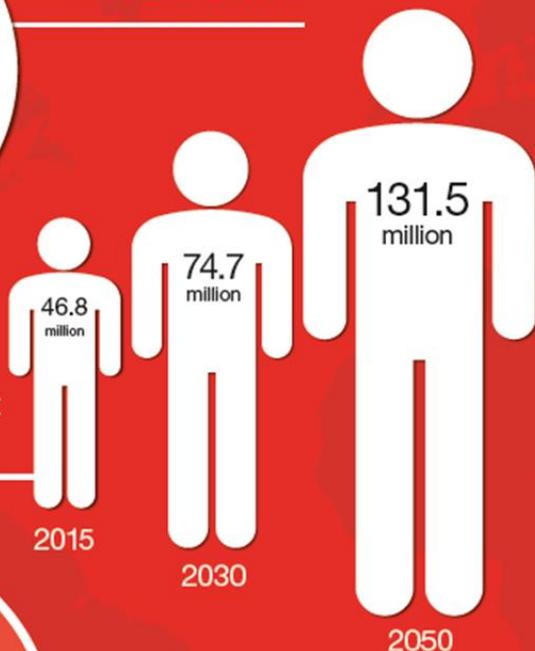
**#DefeatingDementia**

# Alzheimer's Disease is a Global Shared Societal Responsibility



Around the world, there will be 9.9 million new cases of dementia in 2015, **one every 3 seconds**

46.8 million people worldwide are living with dementia in 2015. This number will almost double every 20 years.



Much of the increase will take place in low and middle income countries (LMICs): in 2015, 58% of all people with dementia live in LMICs, rising to 63% in 2030 and 68% in 2050.

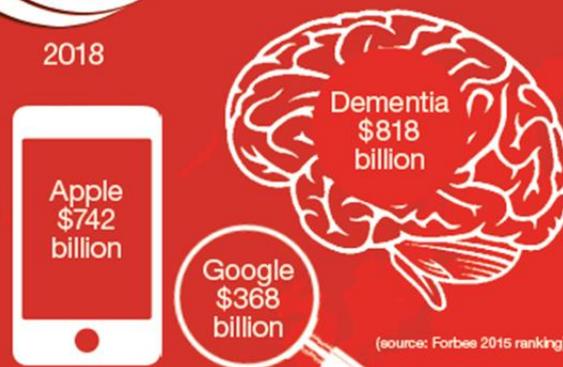


The total estimated worldwide cost of dementia in 2015 is US\$ 818 billion. By 2018, dementia will become a trillion dollar disease, rising to **US\$ 2 trillion by 2030**

If global dementia care were a country, it would be the

**18th largest economy**

in the world exceeding the market values of companies such as Apple and Google



This map shows the estimated number of people living with dementia in each world region in 2015.

We must now involve more countries and regions in the global action on dementia.



**Alzheimer's Disease International**

*The global voice on dementia*

# Our Commitment to Alzheimer's Disease

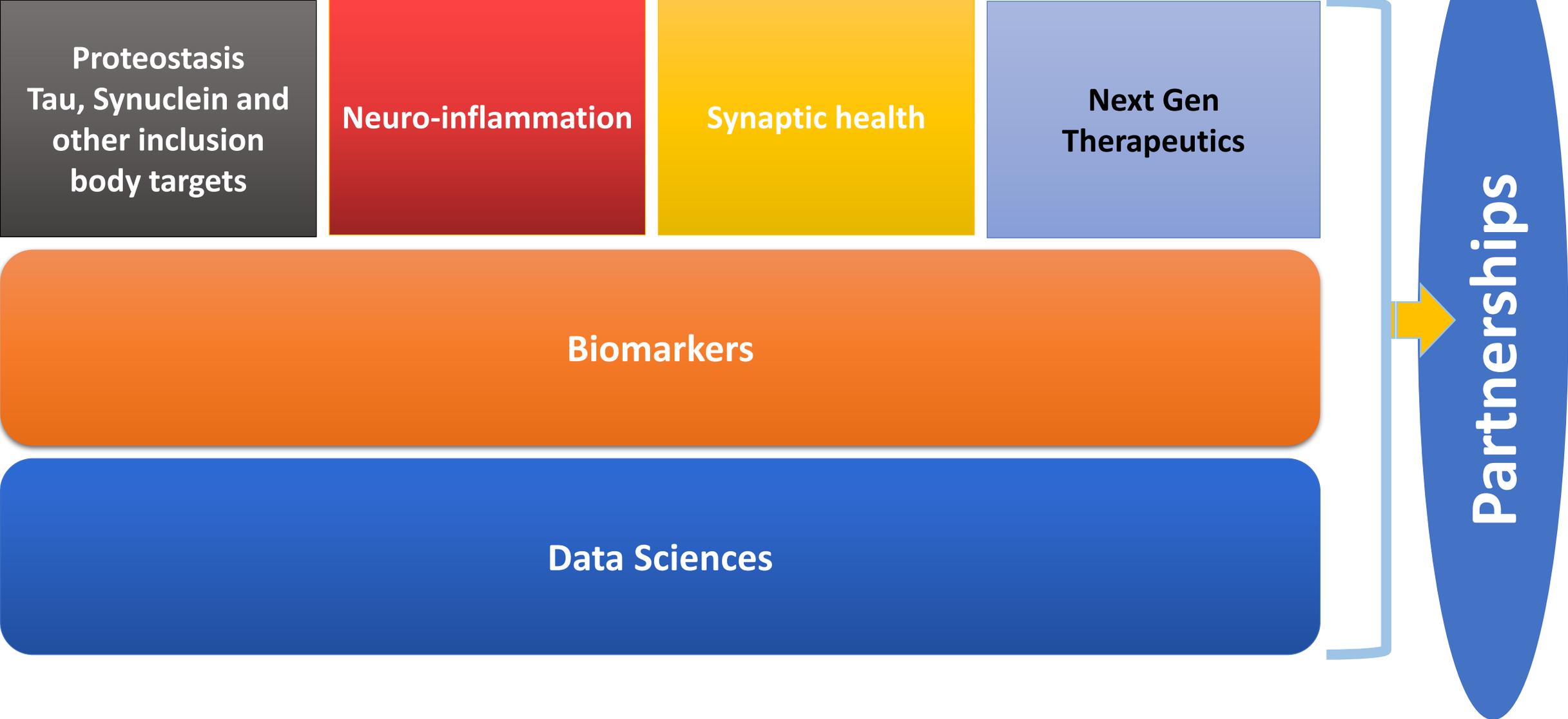
**Not only must the Industry stay optimistic about AD research, the entire society has to be optimistic to help patients & face the societal challenges of AD**

- **“Failed” clinical trials are leading to progress/insights**
  - **Setbacks offer insights that reshape scientific understanding and future research**
  - **Pre-trial characterization of potential patient cohort to facilitate trials (e.g., EPAD) and reduce failures**
  - **Regulators are progressive, willing to be bold with combination treatments and innovative trial designs**
- **Biomarker discovery (diagnosis/treatment efficacy) and novel targets (eg neuroimmunology) still have to be fully explored**
  - **Potential has not yet been fully realized**
  - **Novel targets continue to be identified and subject to validation and drug development campaigns**

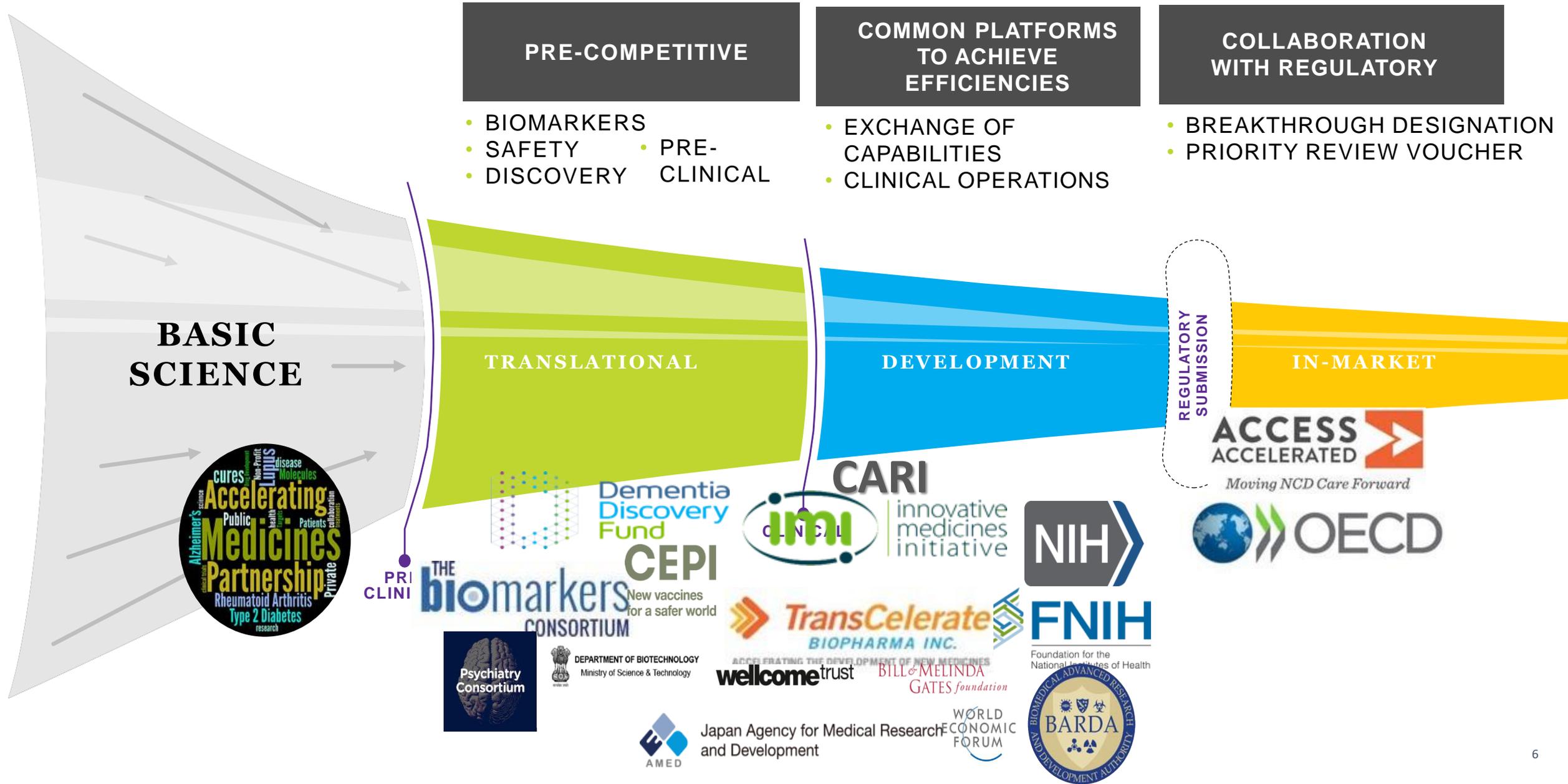
# Our Commitment to Alzheimer's Disease

- **Novel technologies, such as gene therapy or oligonucleotide-based modalities, have started to enter clinics for CNS indications**
  - Preliminary results are encouraging (e.g., GENERATION-HD1 (Huntington's disease, Roche/Genentech))
  - First FDA approvals for SMA – Spinraza (ASO, Biogen/Ionis) and Zolgensma (AAV, Novartis)
- **Data Sciences to explore real-world evidence and a link (e.g., genetics, omics, lifestyle, etc.) is in an exponential phase of development**
  - Examples include Machine Learning and Artificial Intelligence for drug re-purposing and pathway / target identification
- **A change in the “culture” of science in AD**
  - Increase in collaborative spirit and data-sharing across Pharma, Universities, and Hospitals
  - Funding increases steadily to support R&D, including PPPs

# Janssen's Commitment and Approach



# R&D Partnerships across the continuum



# Dementia Discovery Fund: DDF



## Background

The World Dementia Council was formed following a 2013 summit where G8 countries committed to identifying a **cure or a disease-modifying therapy** for dementia by 2025 and to **increase funding** for dementia research.

The DDF was founded in 2016 through a **funding vehicle workstream** of the World Dementia Council.

## Objectives

Dementia Discovery Fund is an innovative specialist venture capital fund that **brings together charity, government, industry and significant public/private investors** to create **new sources of investment** to support novel translational science

- Investing in novel science to:
  - Create meaningful new treatment for Alzheimer's Disease and other forms of dementia
  - Generate return for investors
- Focused on new therapeutic approaches that lead to the discovery and development of disease modifying therapies



Bill Gates



UNITEDHEALTH GROUP

woodford

# Healthy Brains Financing Initiative

**THE PROBLEM:**  
Brain health disorders are the world's biggest un-met medical need

- One in four people will be affected by mental or neurological disorders
- #1 cause of adult disability
- An estimated 450 million people worldwide live with a mental illness
- Global burden is \$3 trillion annually
- An estimated 12 billion working days are lost due to mental illness every year

Addressing this challenge will require a coordinated global response with investment from and involvement of a variety of stakeholders

- 33% of countries allocate less than 1% of their total health budgets to mental health
- 40% of countries have less than one hospital bed reserved for mental disorders per 10,000 people
- Financing alternatives being explored include social impact bond or fund structures

## THE RESPONSE: HBFI

\$10 billion umbrella set of financing mechanisms to fuel an unprecedented increase in brain science breakthroughs, from basic research to implementation, that will change the lives of those living with mental and neurological disorders and bend the unsustainable \$3 trillion global cost curve borne today by low, middle, and high income countries.

- Led by One Mind and the National Academy of Medicine.
- Developed in cooperation with the World Bank Group, Society for Neuroscience, World Health Organization, and other leading national and international organizations.

# Funding the Most Needed Science

Basic Science	Translational Science	Implementation Science
Investing in long-term research with the aim of generating breakthrough ideas that lead to treatments and, potentially, cures.	Accelerating promising therapies currently in development and repurposing existing therapies.	Applying proven interventions at scale to yield early wins, especially in low resource settings.

- Will develop a 10-year plan for the research necessary to develop new understandings of disease mechanisms, effective management, and means of prevention of mental, neurological, and substance use disorders.
- All funding decisions will be made with input from those who are living with disorders.

# Building the HBFI

- In preparation for a launch in early 2021, One Mind and NAM formed four working groups to develop key strategies and implantation plans.
  - **Global leaders across science, finance, philanthropy, and government are donating their time to this effort.**
- **One Mind is seeking start-up funding** to provide support for pre-launch activities in order to ensure a strong foundation for the HBFI.

Use of Proceeds	Pay-for Metrics	Finance Structure	Governance
Defining the research priorities to be funded (basic, translational, and implementation science).	Identifying metrics and measurement methodologies to demonstrate ROI of HBFI activities.	Determining the finance mechanism(s) that are the best fit for the variety of research to be funded and funders to be involved.	Establishing overall governance structure of initiative, including finance instruments and grantmaking activities.

# Accelerating research: the role of government, academia, and industry

**Dr Pierre Meulien**

Director, IMI

**#DefeatingDementia**

# Accelerating Innovation in Dementia through Public Private Partnerships:

*The Innovative Medicines Initiative (IMI)*

Pierre Meulien • IMI Executive Director

World Dementia Council- Tokyo- Japan- October 18th 2019

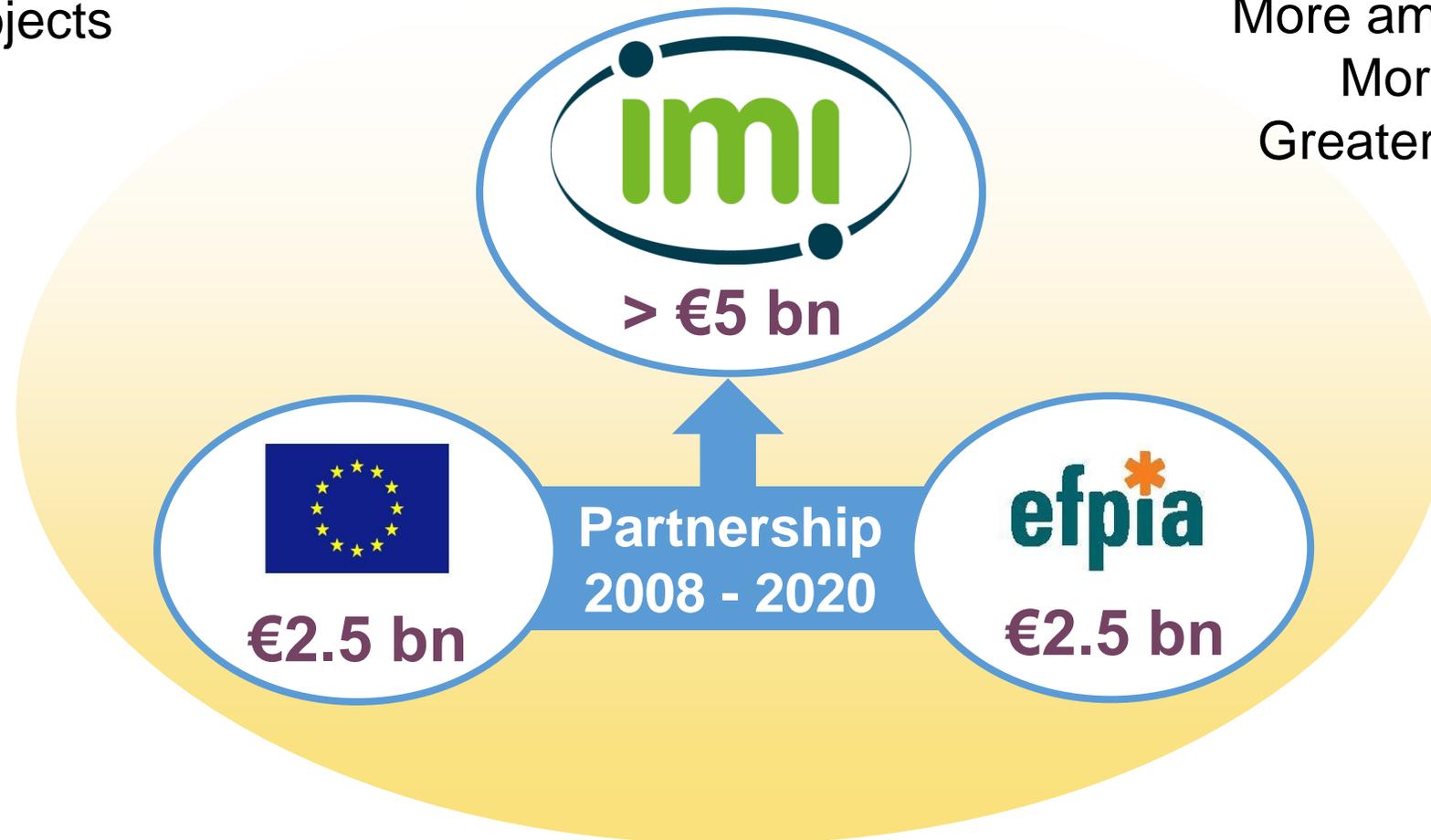
# IMI – Europe's partnership for health

## IMI1: 2008-2013

€2 bn budget  
59 projects

## IMI2: 2014-2020

€3.3 bn budget  
More ambitious  
More open  
Greater scope

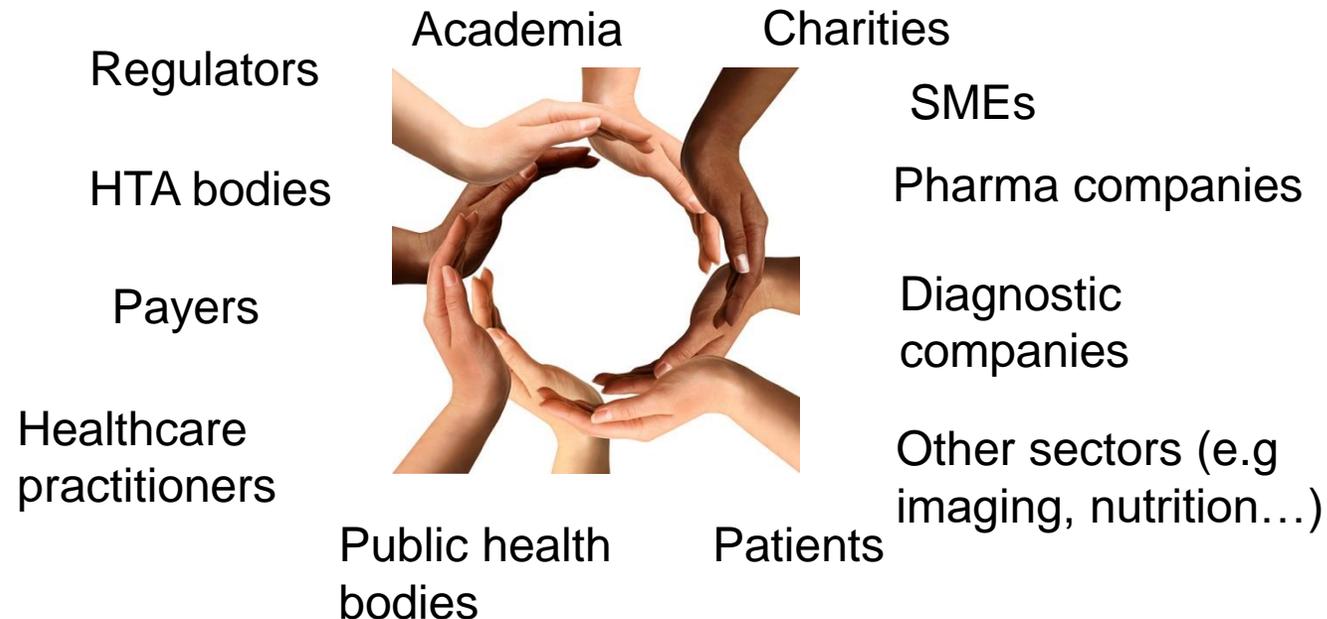




# IMI – Ecosystem for innovative collaborations

IMI is a **neutral platform** where **all involved** in drug development can engage in **open collaboration** on **shared challenges in areas of unmet medical needs**.

All partners needed to find transformative solutions to **reduce late stage attrition, speed patient access** and **improve health outcomes** and find solutions for a sustainable healthcare system



# IMI 2 budget (2014 – 2020)

## EU funding goes to:

Universities

SMEs

Mid-sized companies

Patient groups

etc...



€1.638 bn



€1.425 bn

Other  
€213 m

IMI 2 total budget  
€3.276 billion

## EFPIA companies

receive no funding

contribute to projects 'in kind'

Associated Partners e.g. charities, non-EFPIA companies

# EFPIA Partners in Research



# IMI2 Associated Partners as of March 2019

Accelerate Diagnostics  
Autism Speaks  
Autistica  
BD Switzerland Sarl  
Bill and Melinda Gates Foundation (BMGF)  
Bio-rad Laboratories  
Cepheid Europe  
CHDI Foundation  
Children's Tumour Foundation  
Diamond Light Source  
International Diabetes Foundation  
Invicro  
JDRF  
KTH Royal Institute of Technology

Leona M. and Harry B. Helmsley  
Charitable Trust  
McGill University  
Medicines for Malaria Venture  
Obesity Action Coalition  
Ontario Institute of Cancer Research  
Parkinson's UK  
Simon Foundation Autism Research  
Initiative (SFARI)  
Software AG  
Springworks Therapeutics  
T1D Exchange (formerly Unitio)  
TB Alliance  
University of Dundee  
Wellcome Trust

Total Number of Associated Partners:

27

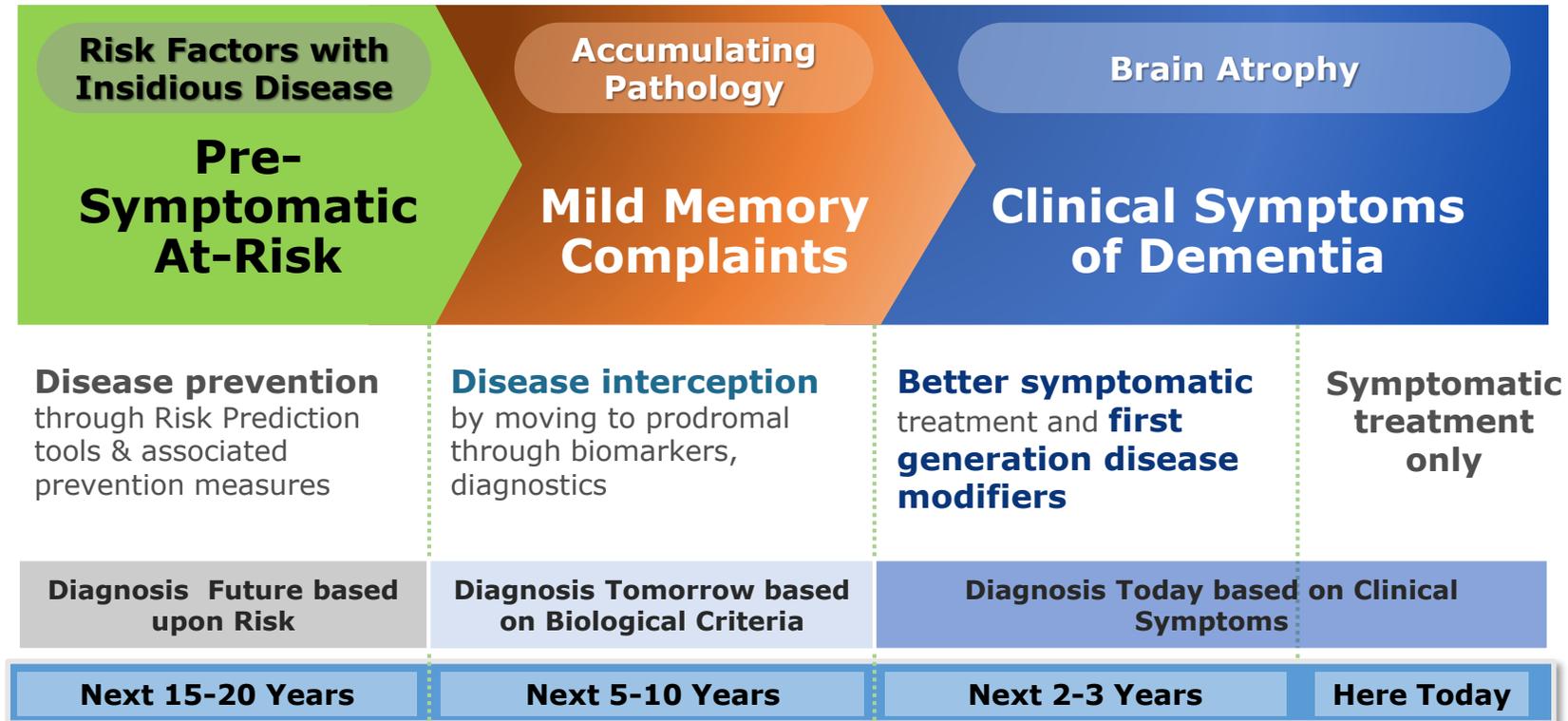
Total EUR committed:

**EUR 162,232,214**

## Some areas of achievement and potential future activities

- Technology platforms like ELF, EHR4CR, EMIF-EHDN
- Gaps in the ecosystem
  - Paediatric CTN
  - A project on the use of medicines in pregnant women and during the neonatal period
- Market failures like AMR
- Complex but highly relevant public health burdens like dementia, pain, autism or diabetes
- Areas undergoing technology convergence
  - Big data and remote biosensor technologies
  - Microbiome
  - Advanced therapies
  - Disease interception (changing the course of disease progression in chronic diseases)

# Neurodegeneration: Diagnosis and Therapeutics Moving Earlier



*Neurodegeneration Therapeutics is moving earlier in disease process: the concept of disease interception*

# IMI neurodegenerative diseases portfolio

## Challenge 1: What are the underlying causes of dementia?

**AETIONOMY:** Identifying subgroups of dementia, based on underlying genetic / molecular causes, to allow tailored treatments

## Challenge 2: Who is at greatest risk of developing dementia?

**EMIF:** Linking & analysing data from many studies to identify markers of risk

**Patients involved**

## Challenge 3: How can we improve clinical trial design?

**EPAD:** Setting up innovative clinical trials that allow multiple treatments to be tested at same time

## Challenge 4: Can brain scans aid research & treatment?

**AMYPAD:** Studying how brain scans of 'amyloid plaques' could aid diagnosis, treatment & research

# IMI action on neurodegeneration

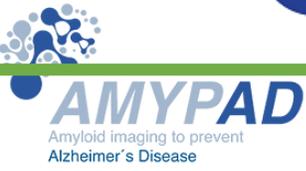
Remote Assessment of Disease and Relapse Programme (RADAR)



**RADAR AD\***  
\*proposal evaluation stage



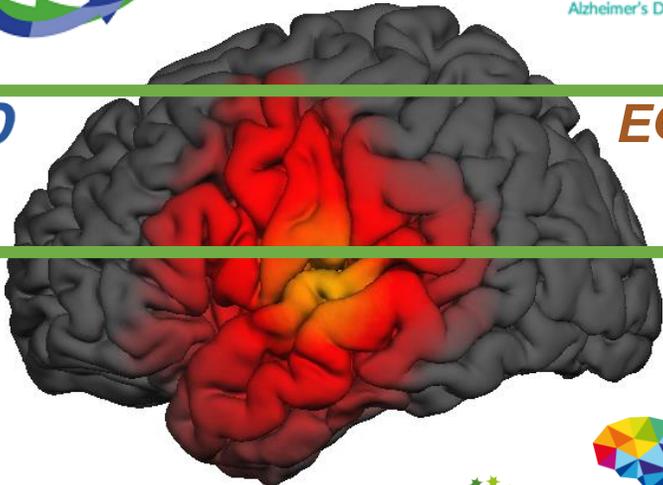
Big Data for Better Outcomes Programme (BD4BO)



**EQIPD**



**IMPRiND**



# Precompetitive Vs Competitive R&D

- Changes over time
- Tendency is a shift downstream
- Realisation by private sector that for some targets there is a need to share risk
- Risk can come in many forms
  - Market failure
  - Scientific complexity

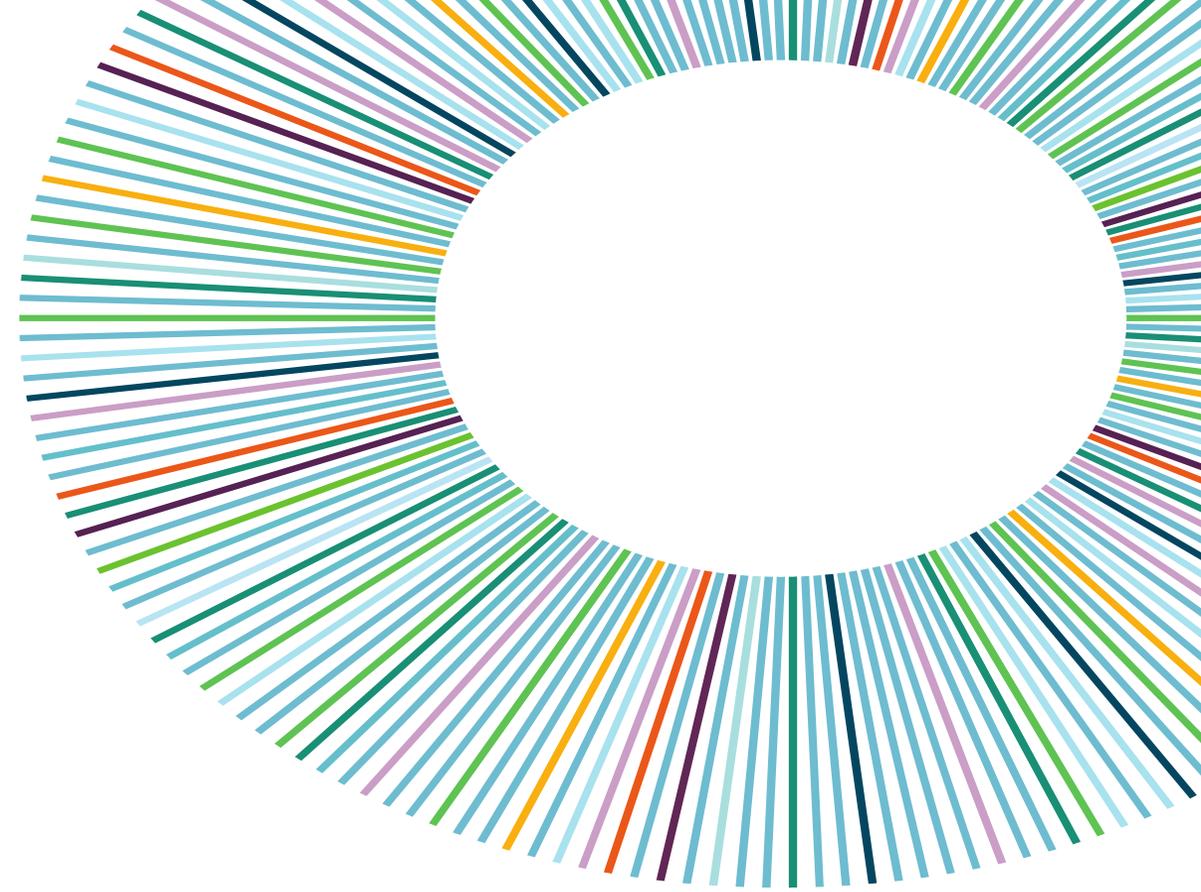
Visit our new website  
[www.imi.europa.eu](http://www.imi.europa.eu)

Sign up our newsletter  
[bit.ly/IMInewsletter](http://bit.ly/IMInewsletter)

Follow us on Twitter  
[@IMI\\_JU](https://twitter.com/IMI_JU)

Join our LinkedIn group  
[bit.ly/LinkedInIMI](http://bit.ly/LinkedInIMI)

Email us  
[infodesk@imi.europa.eu](mailto:infodesk@imi.europa.eu)



Thank you!

# Accelerating research: the role of government, academia, and industry

**Dr Isao Teshirogi**  
CEO, Shionogi

**#DefeatingDementia**

World Dementia Council Meeting  
October 18, 2019



# Shionogi R&D vision in CNS disease area

Isao Teshirogi, Ph.D.  
President & CEO  
Shionogi & Co., Ltd.

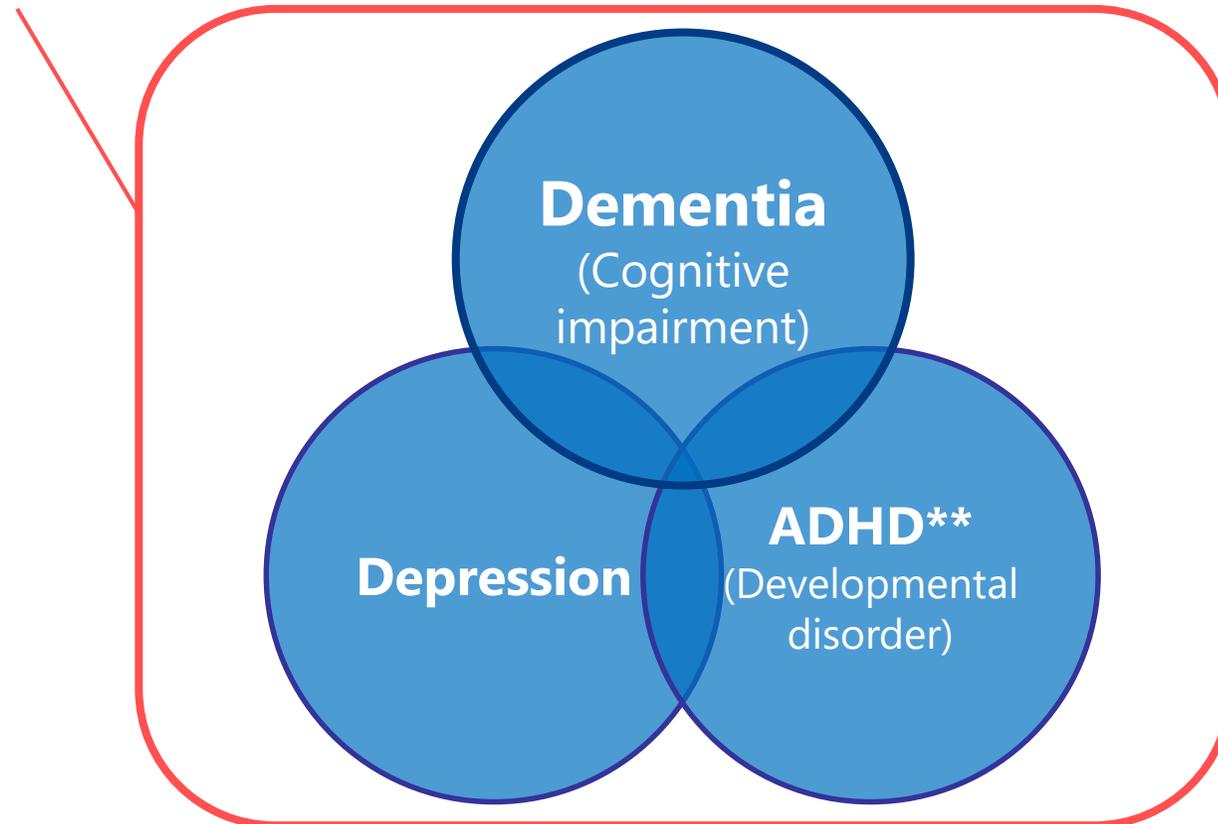


PRIVILEGED &  
CONFIDENTIAL

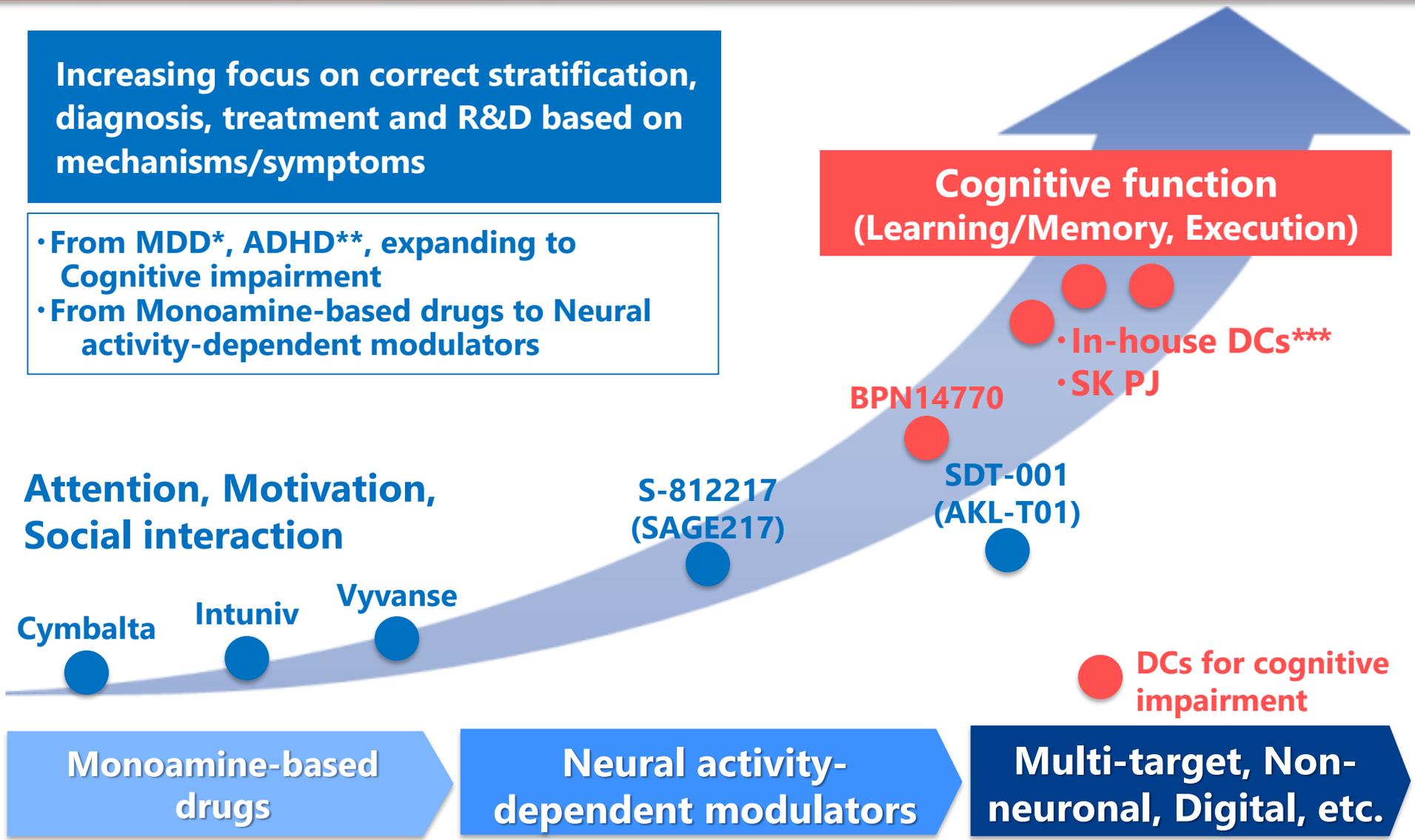
# Therapeutic Focus Areas in Shionogi



- Infectious diseases
- **CNS\*** diseases and pain



# Our Vision in CNS Disease Area



# Our Vision in CNS Therapeutic Area



As is

- Symptoms vary widely even in the same disease
- Effective therapies should be based on deep understanding of the mechanisms of brain function

Issues

**Patient diagnosis /stratification with biomarkers**

**Understanding the mechanisms of brain function**

**Appropriate therapeutic option**

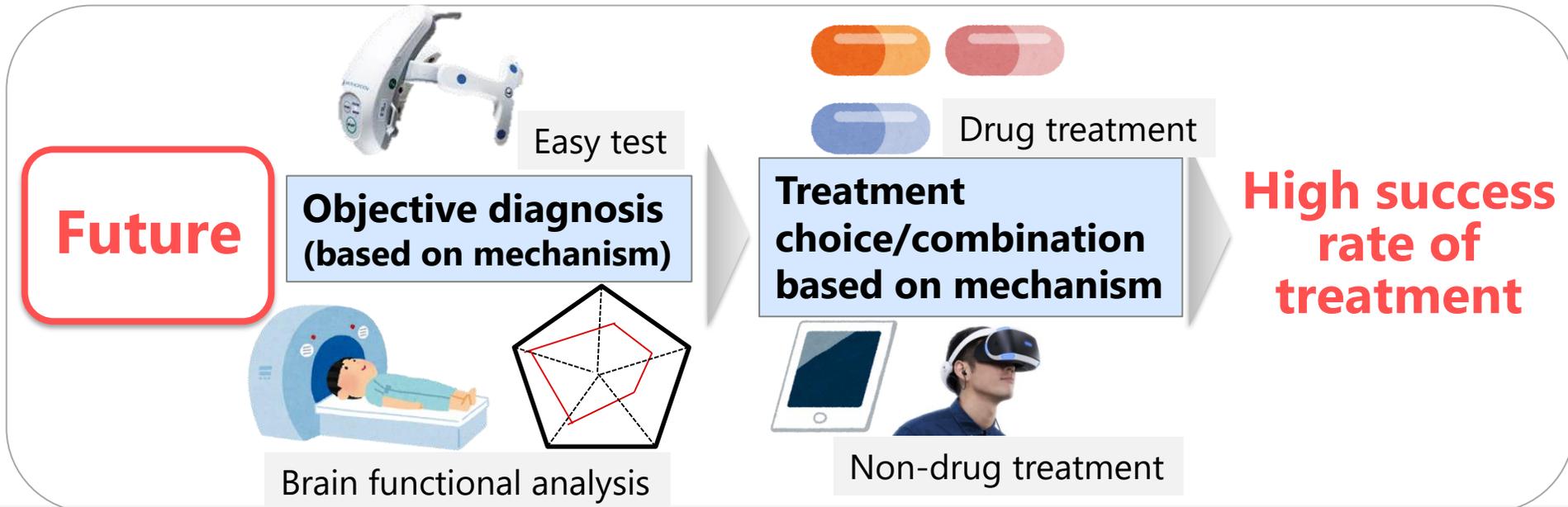
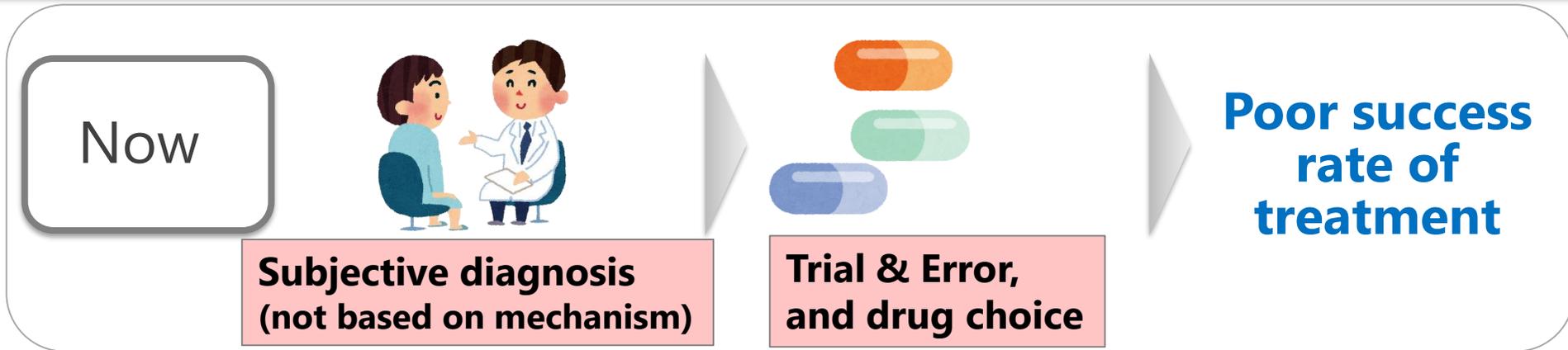
- Development of novel biomarkers based on brain function in CNS diseases

- Development of evaluation index which can be utilized in both human and animals (MTC\*)
- Development of nonclinical evaluation methods and discovery of novel drug targets (SK PJ\*\*)

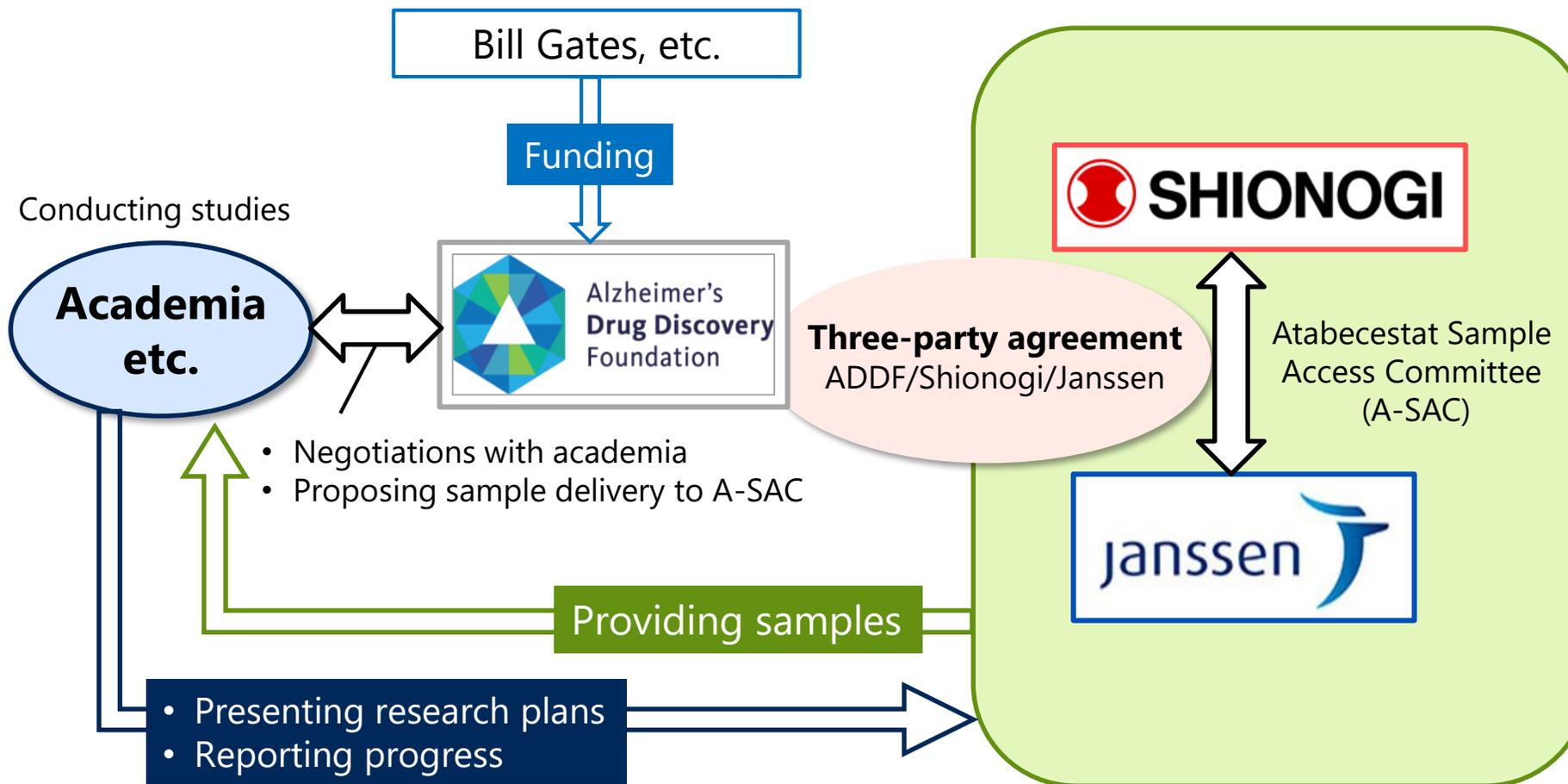
- Creation of novel drug candidates
- Creation of digital medicines
- Development of brain delivery platform by novel PDC\*\*\*

**Provide the appropriate therapy with precise diagnosis based on brain function**

# Future Diagnosis/treatment of CNS Diseases



# Scheme of Clinical Sample Access



# Accelerating research: the role of government, academia, and industry

**Dr Tadafumi Kato**

Deputy Director, Riken Institute

**#DefeatingDementia**

World Dementia Council

**Defeating dementia: the international collaboration challenge**

“Accelerating research: the role of government, academia and industry in developing funding and research models”

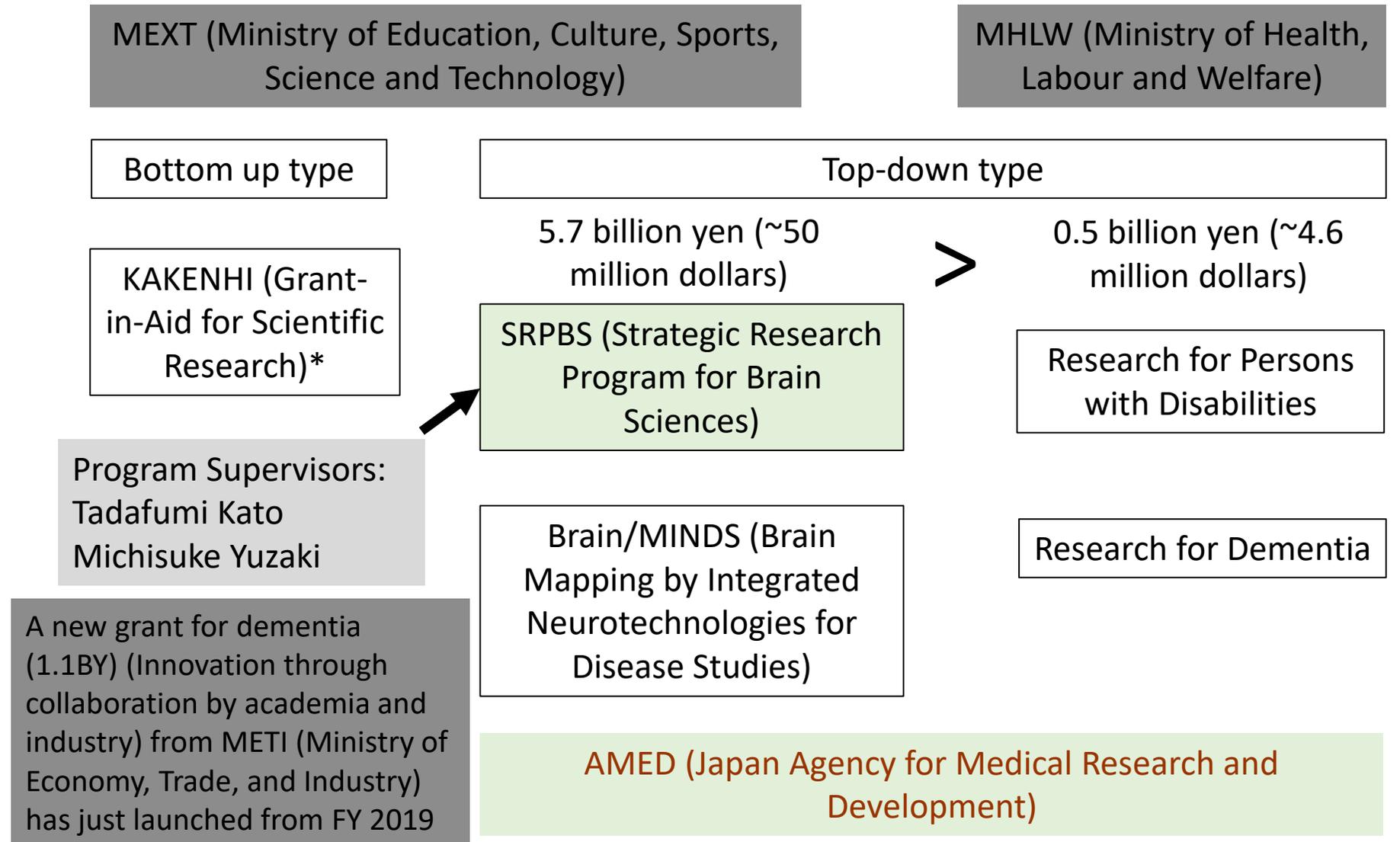
11:00 – 11:55, October 18/2019, Imperial Hotel, Tokyo

**Promotion of dementia research in Japan**

**Tadafumi Kato, MD., Ph.D.**

Lab. for Molecular Dynamics of Mental Disorders RIKEN  
Center for Brain Science

# Funding systems for brain sciences in Japan



## SRPBS (Strategic Research Program for Brain Sciences) (2011~)

- 2011~ “Brain Research to conquer neuropsychiatric disorders” (3Ds: Depression, Dementia, Developmental disorders)
- 2016 ~ “Concurring neuropsychiatric disorders by enhanced collaboration between clinical and basic research” (3Ds and Resource/Ethics)
- 1.69 billion yen/year (15.7 million dollars/year) (FY2018)

# Dementia Research Group of SRPBS

*Disease*

*Basic Research*

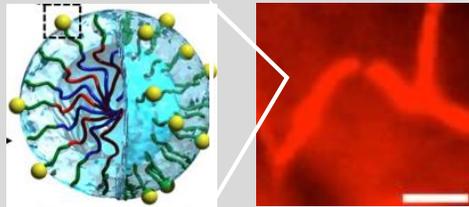
*Translational Research*

*Clinical Applications*

Alzheimer's disease

- Glucose metabolism and A $\beta$  dynamics (Iwatsubo)
- Microglia and neuro-inflammation (Yamanaka)
- Plasma A $\beta$  biomarker (Kakuta\*)
- Secretase inhibitor and A $\beta$  dynamics (Iwatsubo)

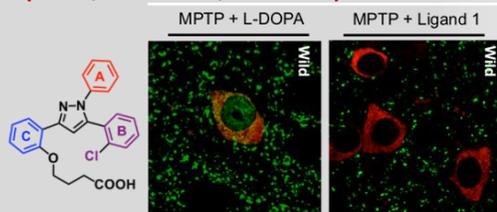
BBB-permeable nano-carrier



- BBB permeable A $\beta$  antibody (Yokota)
- Development of new treatment targeting ILEI/p3-Alc $\beta$ (Nishimura)

Dementia with Lewy Body Disease

Anti-synuclein accumulation effect of FABP3 ligands (PCT/JP2017/13742)



- Degradation mechanism of  $\alpha$ -synuclein (Kabuta\*)
- Novel degradation mechanism of Tau (Shimizu)
- Diagnosis and treatment by FABP3 ligands (Fukunaga)

FTD

- Molecular target therapy by antisense-oligonucleotide (Sobue)

Across dementia

- Mechanism of dissemination of protein aggregates (Nukina)
- Sleep and dementia (Hayashi)

# Representative PPPs (Public Private Partnerships) projects

Country	Name	Participants	Goal	Activity
US	ADNI	NIH, Industry, NPOs	PET, longitudinal analyses of CSF markers,	Data sharing
US	MATRICS	NIMH/industry/academia	Drug development for cognitive dysfunction	Test battery of cognitive function
US	BC (Biomarker consortium)	FDA, NIH, Industry, FNIH	Development of biomarkers (9 disease including AD)	Distribution of blood samples
EU	NewMeds	academia, industry	Depression, Schizophrenia	Distribute CNV model mice
US	CMC (CommonMind Consortium)	University/Industry (Takeda etc)	Studies of etiology and treatment of neuropsychiatric disorders	Sharing of RNAseq data
Japan	<b>CINP/JSNP PPP taskforce (2015~)</b>	<b>Industry (18), University (6), Institutes (4)</b>	<b>Biomarkers for drug development (PET etc), Biomarkers for stratification of patients, Building up database of RCTs</b>	
US	BRAIN Initiative Program	NIH Industry	Development of devices for neuro-	