



INNOVATES, ENABLES & PERFORMS



# **Programma Europeo Innovative Medicine Initiative 2: un'opportunità per l'industria della salute**

***Sfide e opportunità internazionali nella filiera della  
salute***

Paola Tarroni, Research Portfolio Manager



# Axxam Corporate Information

## Key facts

- Private Italian company founded in Nov 2001 as a spin-off from the Bayer Group
- Ownership: Founders and Managers (72%), Bayer Group (23%) and Zambon Group (5%)
- Locations: Bresso/Milano (headquarters/biology labs), Napoli (chemistry labs), and Boston/USA (commercial office at CIC-Cambridge)
- Employees: 82 employees, 68 researchers (70% Ph.D.)
- Revenues (2016): around EUR 10,0m



## Business model

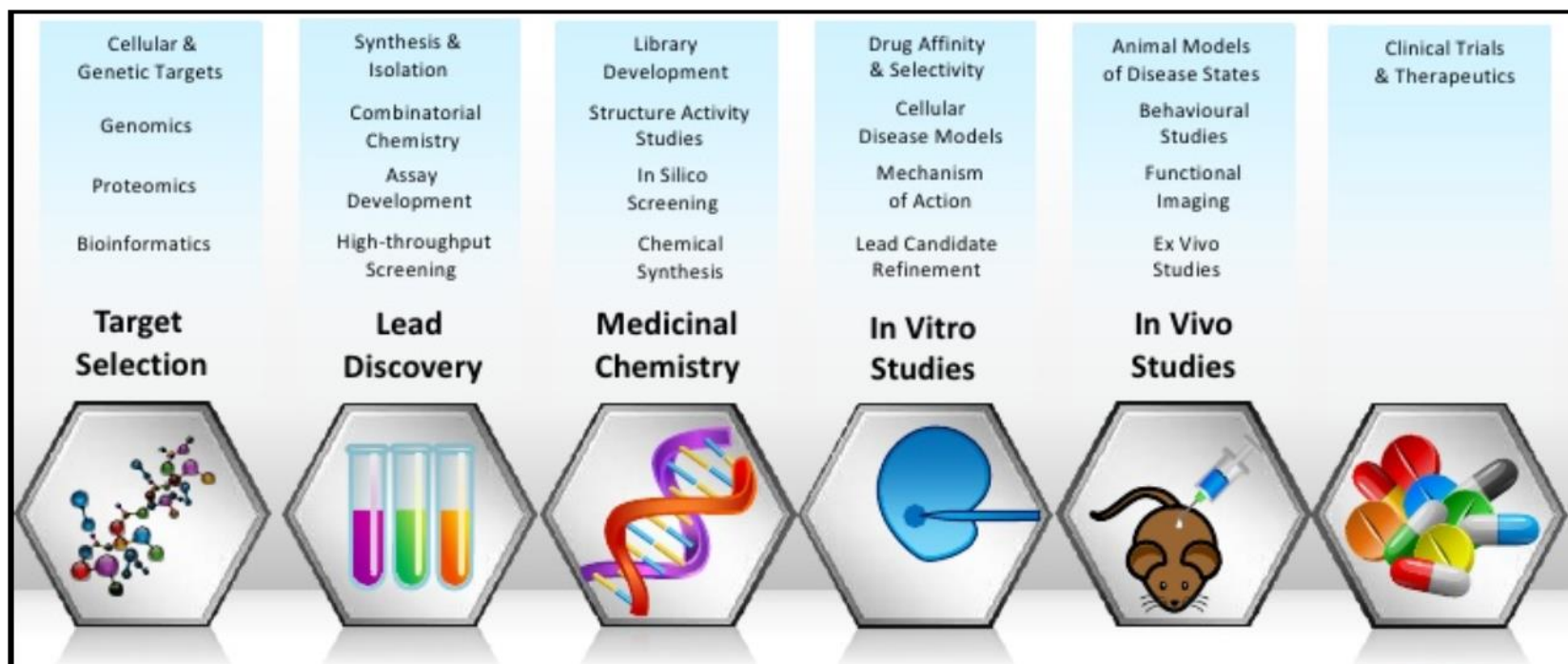
- **i-PRO** (innovative Partner Research Organization): early-stage discovery services, technologies, innovation and lead discovery platforms for applications in the life sciences industry.

## Affiliated Companies

- **IMAX** Discovery GmbH (Dortmund, Germany) (50% stake)
- Hit Discovery Constance (**HDC**) GmbH (Constance, Germany) (40% stake)
- **Acousia** Therapeutics GmbH (Tübingen, Germany) (15% stake)



# Axxam & the Drug Discovery Process



**Axxam**

Early Drug Discovery



# Discovery Services Platforms

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## Assay Development for HTS

(cell-based & biochemical)

## High-Throughput Screening

(FLIPR based, radiometric, HCS)

## Ready-for-Use Cell Line

(180 HTS grade assays *off-the-shelf*)

## Compound Collection AXX<sup>DIV2.0</sup>

(240.000 novel and diverse compounds)

## Compound Profiling & Hit Follow-up

(selectivity, specificity, electrophysiology)



# Business and Market Distribution



## Pharma and Biotech Companies

Major biotech and pharmaceutical companies

## Foundations & Translational Units

Alzheimer's Drug Discovery Foundation, Fast Forward, MD Anderson Cancer Center, NEOMED Institute, New York University, Broad Institute of MIT and Harvard, KU Leuven, LDC of the Max Planck Society etc ...

## Agrochemical Companies

Top three companies worldwide

## Flavor & Fragrance Companies

Eight out of 10 leading companies worldwide

## Consumer Companies

A leading non-alcoholic beverage company

A leading pet food company

Major producers of food ingredients

Several cosmetic companies

## Market presence:

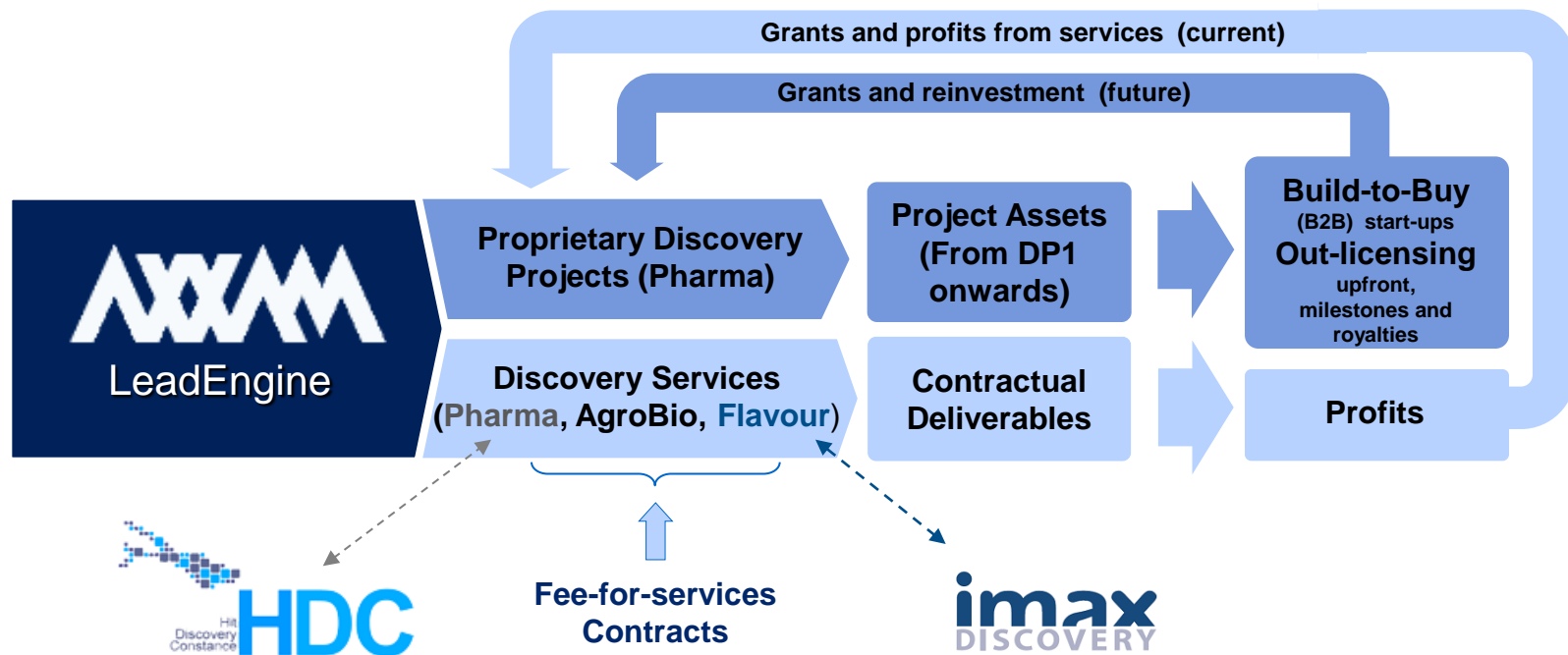
>92% international, ≈8% Italy

## Business segments:

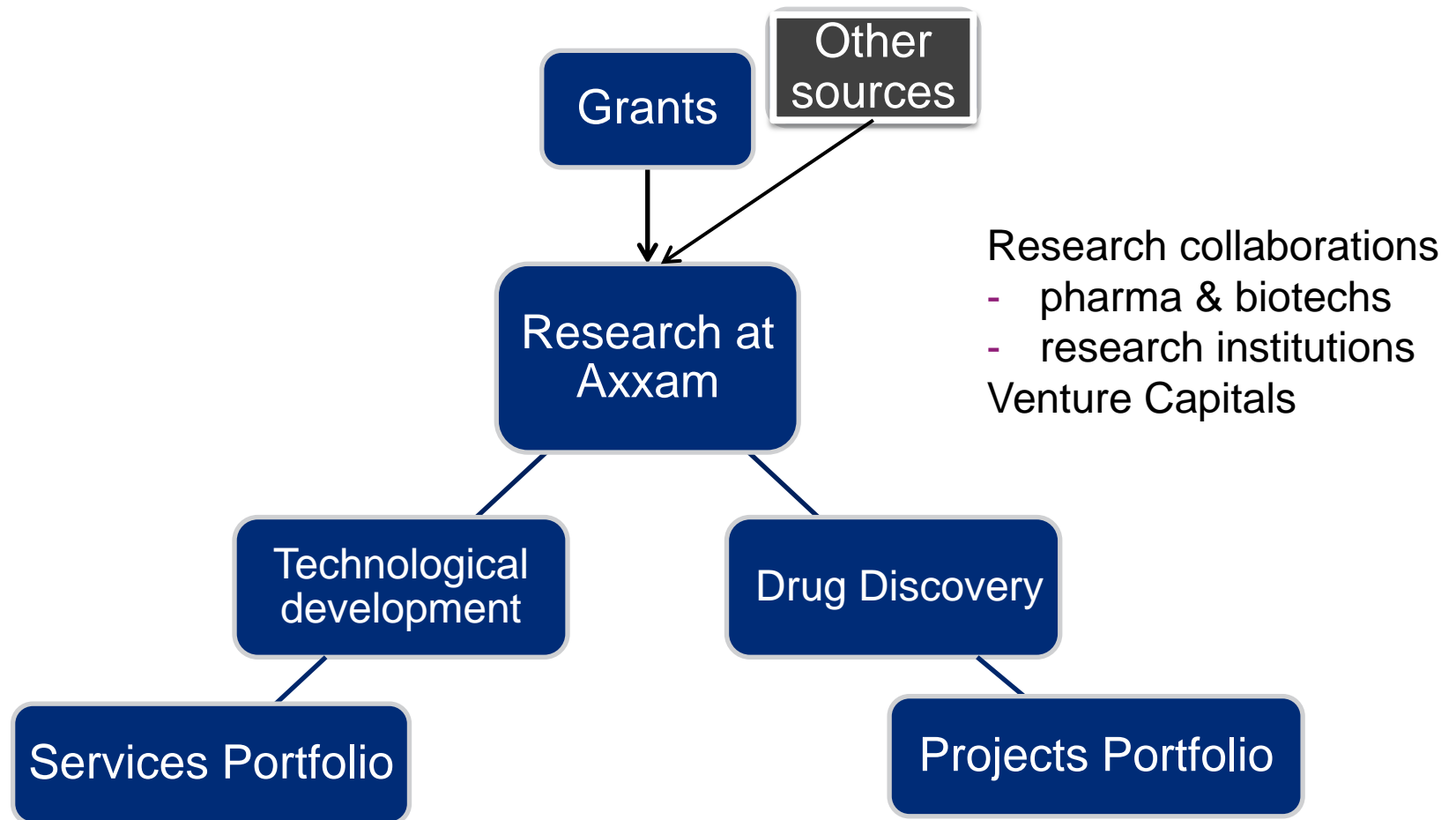
80% Pharma-20% others



# Axxam Business Model



# Grant Strategy



# Axxam Grant Operational Strategy in Pills

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- A systematic process for finding and selecting opportunities.
  - ongoing plan and scouting for identifying high-percentage opportunities
  - tracking opportunities that have recurrent calls or that are likely to recur (pre-notice could be short!)
- Careful evaluation and consideration of grant/funding priorities (usually well documented).
  - funding is a match between the needs/goals of one's organization and those of a funding agency
  - map of research activities/goals versus agencies funding priorities
    - shared with the company board
    - reflected in the application; relevance to the call
- Evaluation of investments necessary to run the selected initiatives and of the expected return on investments
  - both for the company and for the funding agency
  - reflected in the application; impact



# Track Record for International Funding



Alzheimer's  
**Drug Discovery**  
Foundation



innovative  
medicines  
initiative



Horizon 2020  
European Union Funding  
for Research & Innovation



**Fast Forward**  
Speeding Treatments To People With MS



# Innovative Medicines Initiative 2

## *Europe Partnership for Health*

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A **European Public-Private Partnership** focused on unmet medical needs common to all stakeholders of the Life Science ecosystem **with the objective to advance the development of, and accelerate patient access to, personalized medicines.**






European Federation of Pharmaceutical  
Industries and Associations

*Courtesy of Dr Elisabetta Vaudano – IMI2*



# IMI2 overall objectives – Fits for Axxam?

- 
- **improve the current drug development process** through development of tools, standards & approaches to assess **efficacy**, **safety** & **quality** of health products.
  - **develop diagnostic & treatment biomarkers** for diseases clearly linked to clinical relevance & approved by regulators
- 
- **reduce time to clinical proof of concept** (e.g. for cancer, immunological, respiratory, neurological/neurodegen. diseases)
  - **increase success rate in clinical trials** of priority meds (WHO)
- 
- **develop new therapies** for diseases with **high unmet need**, (e.g. Alzheimer's) & **limited market incentives**
  - **reduce failure rate of vaccine candidates** in phase III trials through new biomarkers for efficacy & safety checks

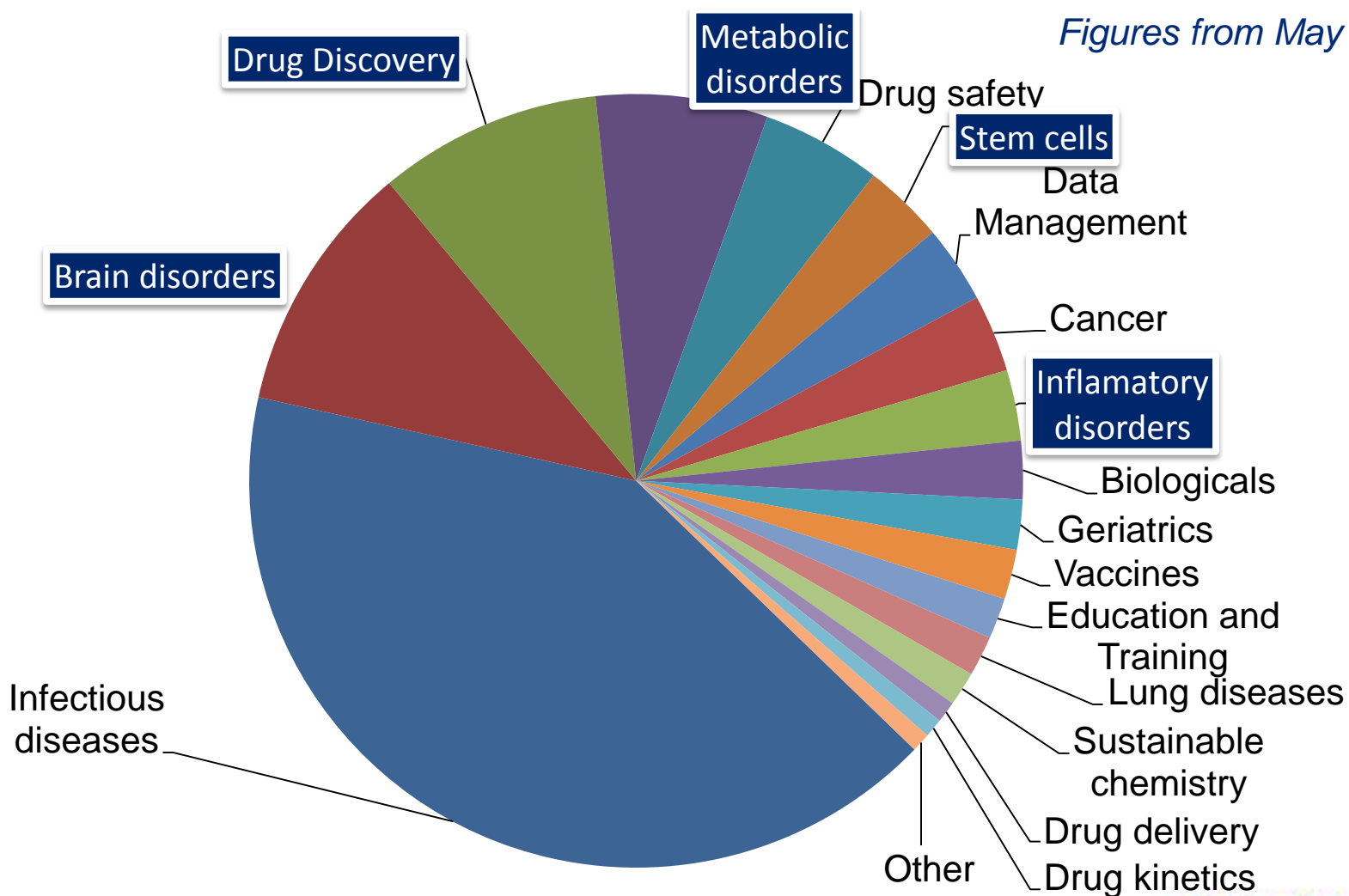
- IMI2 legislation, Article 2b

Courtesy of Dr Elisabetta Vaudano – IMI2



# IMI budget breakdown – Fits for Axxam?

*Figures from May 2016*



*Courtesy of Dr Elisabetta Vaudano – IMI2*



- High strategic fit
- Appreciated funding rate
- Very detailed calls – Easier to recognize itself in the specific call topic and identify partners with competences needed
- Two steps process
- It's an opportunity to increase and strengthen collaboration with Pharmaceutical Industries, which are Axxam major business targets
- It's an opportunity to increase collaborative networks with Research Institutions

### **Like less**

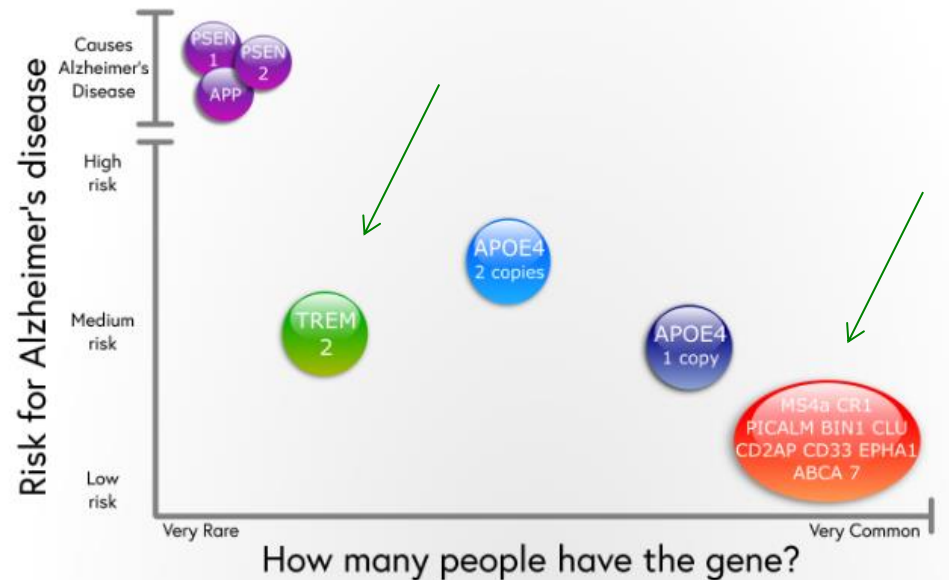
- Large consortia
- Consortium agreement; access rights could rise some issue for SMEs
- Timelines from call launch to Grant start (around 1 year).



# Call 5 Topic 7 – July 2015

## *Alzheimer's disease, modulating microglia function – TREM2 & CD33*

- In Western Europe in 2013, ~11 million people suffering from dementia; it is anticipated that there is an increase up to 21 million in 2050.
- Alzheimer's disease (AD) is an age-related chronic neurodegenerative disease; currently no effective treatment is available for AD
- Main pathological hallmarks: amyloid plaques, fibrillary tau tangles, inflammation and synaptic/neuronal loss.
- Phagocytes around plaques appear to be dysfunctional.
- Multiple GWAS studies, and integrated systems biology approaches have implicated the innate immune receptor genes TREM2 and CD33 in AD, but their exact role is still unclear.
- **Opportunity to develop new effective treatment paradigm.**



# PHAGO - a Public Private Partnership

—From innovation to industrialization—

## EFPIA Partners

abbvie

janssen  
PHARMACEUTICAL COMPANIES  
OF Johnson & Johnson

Lundbeck

Roche

AstraZeneca

Lilly

ORION

SANOFI

## SME Partners

ARTTIC  
INTERNATIONAL MANAGEMENT SERVICES

AXXAM  
INNOVATES, ENABLES, PERFORMS

life&brain

## Funding

imi



efpia

## Academic Partners

CHARITÉ  
UNIVERSITÄTSMEDIZIN BERLIN

Fraunhofer  
SCAI

UNIVERSITY OF  
CAMBRIDGE

UNIVERSITY OF  
GOTHENBURG

DZNE  
Deutsches Zentrum für  
Neurodegenerative Erkrankungen  
in der Helmholtz-Gemeinschaft

KING'S  
College  
LONDON

UCL

universitäts  
klinikum bonn

## PHAGO Objectives

- Identify druggable points of interaction in TREM2 and CD33 signalling to modulate phagocytes for treatment of AD.
  - Explore whether a decrease or an increase of brain microglia phagocytic activity and/or cytokine release causes or prevents neurodegenerative/ageing phenotypes
  - Explore the roles of CD33 and TREM2 in microglia activity/activation and generate innovative tools to investigate their function
  - Develop and validate assays targeting TREM2 and CD33

Impact for Axxam  
new technologies and new assays; increase competences on neuro-inflammation; liaison with Pharma for Hit Discovery programs

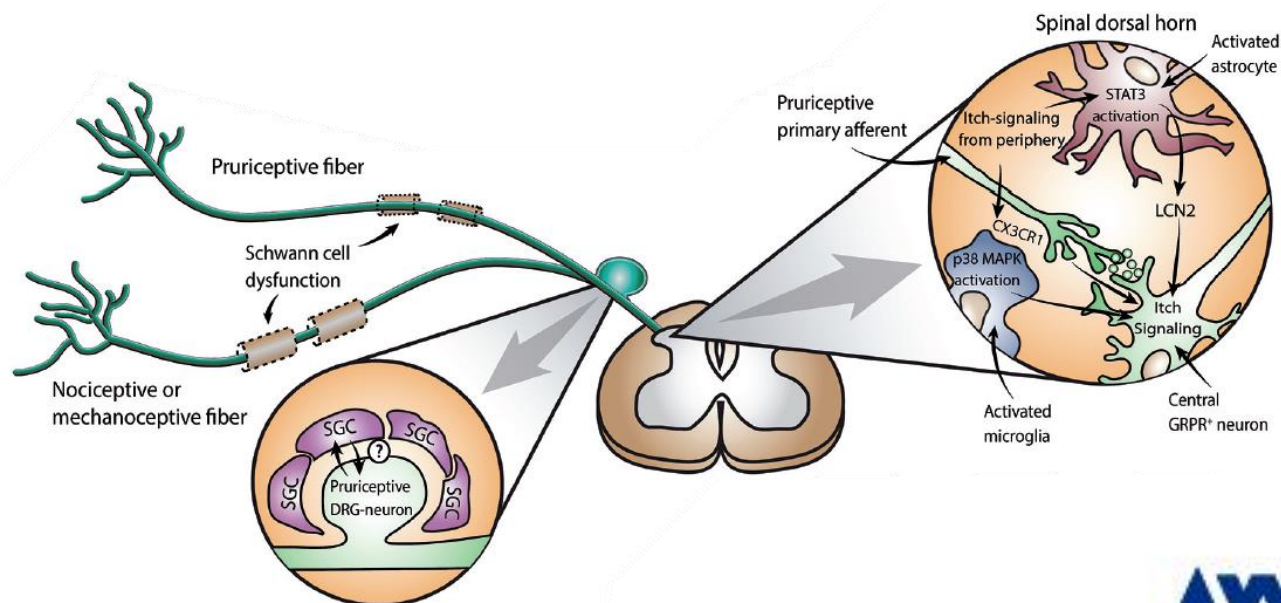
This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 115976 . This Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA



# Call 7 Topic 3 – December 2015

## *Pathological neuron-glia interactions in neuropathic pain*

- Neuropathic pain (NP) affects 6-8% of the population and current treatments are inadequate; the aging population and the diabetes epidemic suggest that this prevalence will rise in the future
- Current treatments have limited efficacy (about 30% of treated patients do not receive adequate relief) further limited by extensive side effects
- Non-neuronal cells play a very active role in sensory abnormalities
- **Lack of translation in the field → need new models and drug screening systems – Focus on human iPSC and HCS.**





NGN-PET

# Neuron-Glia Networks for Pain Efficacious Treatments(NGN-PET) Grant

**Innovation**



**KING'S**  
*College*  
**LONDON**

**Industrialisation**



**ESTEVE**

Funding





## NGN-PET Objectives

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- To understand which mechanisms are most important in clinically common neuropathic conditions, and whether these explain the high susceptibility of women to develop such symptoms.
- To develop efficient assays that capture the key mechanisms that can be used for compound screening.

The ambition of the project is to provide a translational platform of rodent and human Neuropathic Pain models for the identification and validation of **new targets** and **biomarkers**, and suitable for **assay development** and **drug screening**.

**If the project will be successful we aim at having a project prosecution.**

This project has received funding from the Innovative Medicines Initiative 2 Joint Undertaking under grant agreement No 116072. This Joint Undertaking receives support from the European Union's Horizon 2020 research and innovation programme and EFPIA





NGN-PET

## NGN-PET main expected results

- A more thorough mechanistic understanding of neuron-glia interactions
- **Targets** which affect neuron-glia interactions beneficially, and Neuropathic Pain **indicators**, both of which can be demonstrated in human and rodent models
- *in vitro* assays useful for investigating neuron-glia interactions
- *in vitro* assays suitable for **compound screening** on industrial platforms with a strong use of HTS, HCS and iPS Cell systems

Impact for Axxam

**new technologies** and new assays; increase competences on neuro-inflammation; liaise with Pharma for Hit Discovery programs



# Conclusions and perspectives

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- Get all the best from the participation to current projects:
  - Technological development
  - Scientific knowledge and research network
  - Strategic network with Pharma Industries
  - Liaise with IMI2 and institution to deeply understand the program mechanisms
- Stay tuned on the future IMI 2 calls and programs
  - <https://www.imi.europa.eu/content/future-topics> (September 2017?)
  - Annual work plans
- Get opportunities to synergize with other initiatives.

## Thank you for your attention!





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