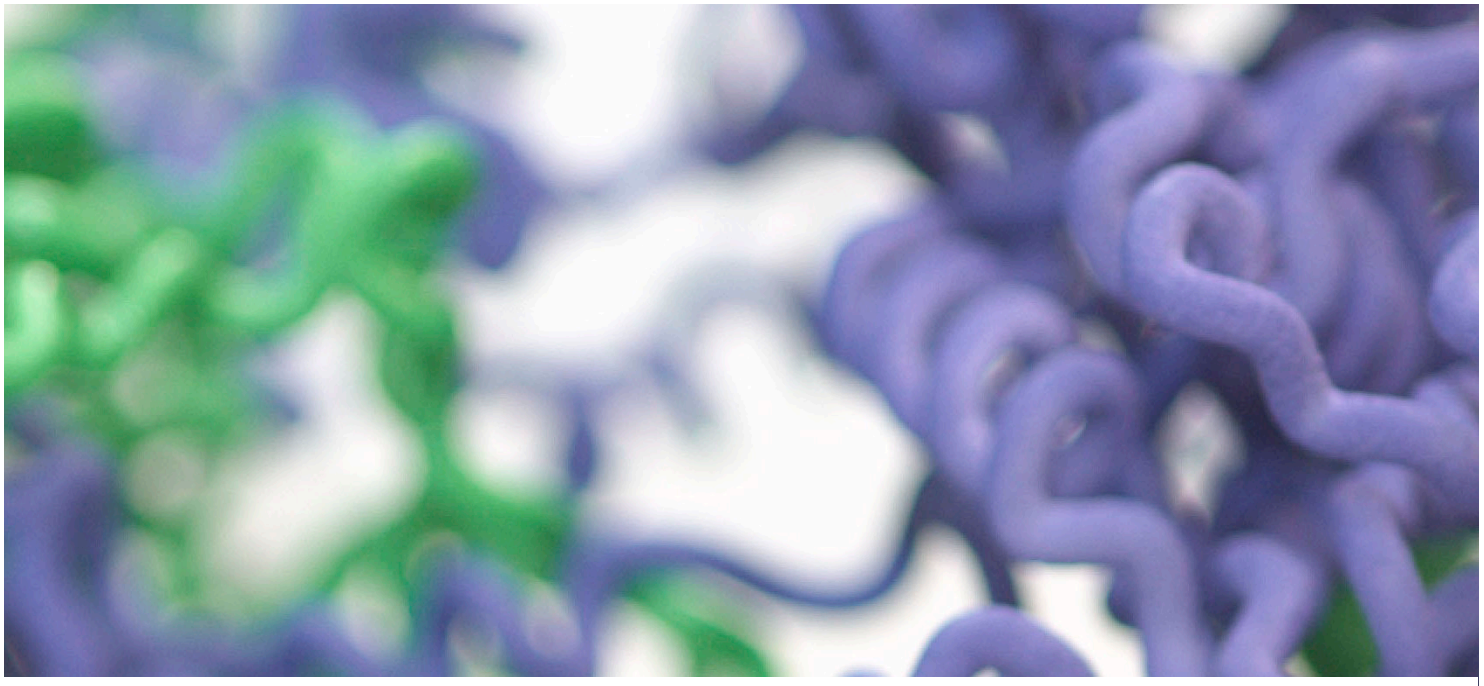

IRB BARCELONA

INSTITUTE FOR RESEARCH IN BIOMEDICINE

Advancing the frontiers of biomedical research

The Institute for Research in Biomedicine (IRB Barcelona) is an independent, non for-profit research center engaged in basic and applied biomedical science. The convergence of biology, chemistry, medicine, physics and computer science at IRB Barcelona provides a unique opportunity for the translation of basic biomedical research into innovation.

BPFO

A NOVEL AND PROMISING NEW TARGET AGAINST ALZHEIMER'S DISEASE

βPFO

A promising new target against Alzheimer's disease

βPFO is a novel amyloid-β (Aβ) oligomer preparation, which can be used to screen for new drugs against Alzheimer's Disease (AD). βPFO forms in an *in-vitro* membrane-mimicking environment and comprises an homogeneous population of β-barrel pore-forming Aβ oligomers. βPFO results from extensive research experience on protein aggregation, combined with an exhaustive screening of various physicochemical conditions that mimic a membrane environment.

CHALLENGE

AD is the most common form of dementia and it is characterized by the loss of intellectual abilities (memory and others), serious enough to affect daily life. It accounts for 60 to 80 percent of dementia cases.

About 17% of people over 60 show mild cognitive impairment (slight but noticeable and measurable decline in cognitive abilities), leading to an increased risk of developing AD or another dementia. Nonetheless, AD is not a disease limited to older people, as up to 5 percent of people with this condition have early onset AD (also known as younger-onset AD), which often appears when someone is in their 40s or 50s.

AD has no current cure and available treatments are directed only at reducing the symptoms. These therapies slow general dementia symptoms and help improve the quality of life of AD patients only for a short period of time. Therefore, **treatments directed to AD are a well-known unmet medical need** for more than two decades.

One of the proposed processes to explain the neurotoxicity observed in AD is the formation of Aβ oligomer pores in membranes. In this context, **blockage of Aβ oligomer pores represents a new therapeutic strategy** to treat AD. βPFO aims to be a powerful approach to find these modulators.

TECHNOLOGY

A research team at IRB Barcelona has developed βPFO, a new *in-vitro* preparation that comprises pore-forming Aβ oligomers in a cell-free, membrane-mimicking environment.

βPFO preparation consists of a novel set of conditions, which allow the obtention of a **homogeneous population of Aβ oligomeric pores** embedded in micelles and/or bicelles. These structures can represent a simple, yet faithful system. Fig 1 depicts a schematic representation of the pore-forming oligomer in a bicelle membrane-mimicking environment.

βPFO will be extremely useful for the development of *in vitro* screening assays to search for pore modulators, which may represent a new hope for AD patients.

For more information: http://www.irbbarcelona.org/en/news/new-strategy-to-obtain-a-specific-type-of-amyloid-beta-aggregate-that-may-underlie-neuronal

COMMERCIAL OPPORTUNITY

According to the World Alzheimer Report 2015, the total estimated **worldwide cost of dementia has reached $818 billion** and will become a trillion dollar disease by 2018. In Europe there has been a significant increase in the costs associated with AD from $240 billion in 2010 to **$300 billion in 2015.** The greatest social costs are related to long-term professional care or home-care.

As aforementioned, there is no current cure for AD, as **all marketed products address symptom palliation**. Few drug candidates have reached clinical trials and those that have achieved, showed poor results. A new **drug developed directly against βPFO may aspire to being both a first-in-class and a blockbuster.**

The new βPFOs methodology is patent protected and **available for out-licensing.**

CURRENT STAGE OF DEVELOPMENT

βPFO development as a method per se, is completed. Additional tailoring of βPFO for a specific screening protocol may be developed in collaboration, if needed. The research group is currently working on the **detailed characterisation of the pore structure**.

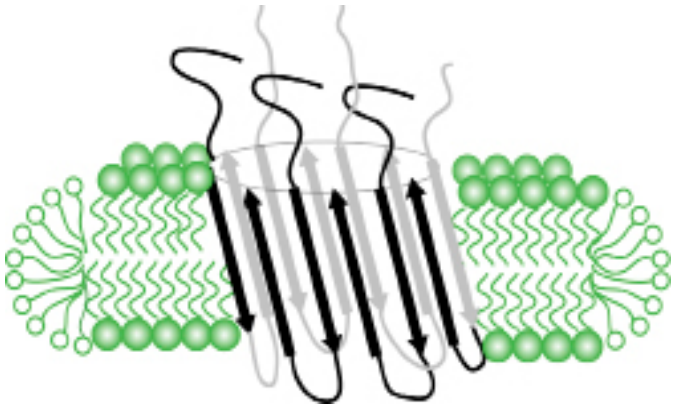

Fig 1 - Schematic representation of βPFO in a bicelle. The Aβ oligomer forming the β-barrel structure is represented in black/grey and the bicelle system (phospholipids/detergent) in green.

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