Assessment of Ultrsound-facilitated Neurotherapeutics in Alzheimers Disease

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Country

USA

Title of project or programme

Assessment of Ultrsound-facilitated Neurotherapeutics in Alzheimers Disease

Source of funding information

NIH (NIA)

Total sum awarded (Euro)

€ 3,190,744.95

Start date of award

01/08/2011

Total duration of award in years

6

The project/programme is most relevant to:

Alzheimer's disease & other dementias

Keywords

Acquired Cognitive Impairment... Aging... Alzheimer's Disease... Alzheimer's Disease including Alzheimer's Disease Related Dementias (AD/ADRD)... Bioengineering... Biotechnology... Brain Disorders... Cerebrovascular... Clinical Research... Clinical Research - Extramural... Dementia... Diagnostic Radiology... Neurodegenerative... Neurosciences

Research Abstract

? DESCRIPTION (provided by applicant): After cancer and heart disease, neurodegenerative diseases, such as Alzheimer's, Parkinson's, multiple sclerosis (MS) and amyotrophic lateral sclerosis (ALS), take more lives each year than any other illness. Although some effective treatments are available, most of those diseases remain undertreated. This is mainly because the blood-brain barrier (BBB) limits the delivery of systemically-administered drugs. As a result, only 5% of the more than 7000 small-molecule drugs available can currently treat Central Nervous System (CNS) diseases. Safe and localized opening of the BBB has been proven to present a significant challenge. Focused Ultrasound (FUS), in conjunction with microbubbles, remains the sole technique that can induce localized BBB opening noninvasively and safely. Prior to our studies reported herein, the bioactivity and therapeutic potential of the trans-BBB delivery was unknown. In this renewal study, we aim at determining 1) the role of the FUS technique in the treatment of Alzheimer's disease (AD) in established mouse models (aim 1), 2) build a new FUS system with neuronavigation capabilities for demonstrating proof of concept of neuroprotection in NHP (aim 2) and 3) build a clinical system and test initial feasibility and safey in a small cohort of patients (aim 3). In this renewal, the primary objective is thus to challenge the FUS technique for demonstrating therapeutic effects in AD models, translating those in large animals and demonstrating clinical feasibility. The underlying hypothesis of the proposed study is thus that the neurotherapeutic potential demonstrated in the previous funding period translates to neurotherapeutic effects in Alzheimer's disease including initial clinical feasibilit. The multi-disciplinary team assembled encompasses all critical specialty areas involved, such as ultrasound and microbubble engineering as well as MRI, PET and fluorescence brain imaging, drug delivery, behavioral assessment and mouse model development as they pertain to neuroscience and neurology. The specific aims are thus to: 1) Demonstrate neuroprotection and neurorestoration in the AD mouse model in vivo; 2) Build a new system for accurate targeting and demonstrate therapeutic effects in NHP in vivo; and 3) Build a new system for accurate targeting in humans in vivo.

Lay Summary

PUBLIC HEALTH RELEVANCE: One of the primordial reasons why Alzheimer's remains undertreated is that current treatments of neurological and neurodegenerative diseases are limited due to the lack of a truly noninvasive, transient, and regionally selective brain drug delivery method. For the purpose of this study, we will apply FUS for the effective treatment of Alzheimer's disease and employ gene delivery due to its great promise and associated low blood-brain barrier impermeability.

Further information available at:

Types:
Investments > €500k

Member States:
United States of America

Diseases:
Alzheimer's disease & other dementias

Years:
2016

Database Categories:

N/A

Database Tags:
N/A