

<機能病態学教室 セミナー>

**Drug Development for Neurologic Diseases:
The examples of Spinal Muscular Atrophy
and Alzheimer's Disease**



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日時：2018年6月22日（金）午後1時00分-2時00分

場所：東京大学薬学部 南館4階 大学院講義室

The lecture will discuss the overall principles of drug discovery and development, and highlight the challenges associated with drug development for neurological diseases. The examples of discovery and development of Aducanumab, an investigational human monoclonal antibody targeting oligomeric and fibrillar forms of A β for the potential treatment of Alzheimer's Disease, and Nusinersin, an antisense oligonucleotide targeting the SMN2 RNA for the treatment of Spinal Muscular Atrophy, will be used.

In the section on Alzheimer's Disease, there will be an introduction of the Amyloid Hypothesis, and some of the challenges with prior studies that have targeted beta-amyloid for the treatment of AD. The discovery of Aducanumab and the pre-clinical studies that have been performed will be discussed. New data on the specificity of Aducanumab for aggregated forms of A β will be presented. Phase 1a and Phase 1b clinical trial results will then be shown. Aducanumab is currently enrolling patients into two large Phase 3 trial, including in Japan.

In the section on Spinal Muscular Atrophy, there will be an introduction on the mechanism of action of Nusinersin. The results of the Phase 1 clinical trial results, and the results of the Phase 3 clinical trial in infants with SMA will also be shown.

The lecture will close with some thoughts on the current and future state of research and development of new drugs for neurological diseases. The hope is that the lecture will stimulate discussion on this important topic.

神経疾患治療薬として SMA に対する世界初核酸医薬として承認された Nusinersen、アルツハイマー病治療薬候補である抗体医薬 Aducanumab の開発を進めている Sandrock 博士（Biogen）にセミナーをお願いしました。大学院生だけでなく、神経疾患に対する新しい創薬アプローチに興味を持つ PI・若手研究者の方々の多数のご参集をお待ちしております。なお本セミナーは大学院講義基礎薬科学特論 IV の一部として行われ、続けて鳥居慎一客員教授による「抗体医薬・核酸医薬開発の最前線」（午後 2-3 時）も行われます。

Contact

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