神経研究所　国際セミナー

日時：2018 年 12 月 12 日（水）　14:00～16:00

場所：教育研修棟　1階　ユニバーサルホール

プログラム

開会の挨拶　　**14:00～14:10**　　**理事長　水澤　英洋**

特別講演 1：　　**14:10～14:45**

Clinical development of NS-065/NCNP-01 (Viltolarsen), exon 53 skipping drug, in patients with Duchenne muscular dystrophy

小牧　宏文 TMC 長　　トランスレーショナルメディカルセンター

座長　青木　吉嗣

特別講演 2：　　**14:45～15:45**

Nucleic acid therapies for neuromuscular diseases

Matthew JA Wood 教授　University of Oxford

座長　武田　伸一

閉会の挨拶　　**15:45～16:00**　　**所長　和田　圭司**

担当・連絡先：遺伝子疾患治療研究部(内線 5224　Tsoumpra M, Hashimoto Y)

NCNP神経研究所 International Seminar

Nucleic acid therapies for neuromuscular diseases

Professor Matthew JA Wood

Department of Physiology, Anatomy and Genetics
Associate Head, Medical Sciences Division
University of Oxford

Date:2018年12月12日（水） 14:00～16:00
Venue:教育研修棟1階 ユニバーサルホール

The main focus of research in Professor Wood's laboratory is the study of RNA biology and the development of RNA-based therapy for neuromuscular diseases. He is a member of the International Duchenne Exon Skipping Consortium which facilitates synergy between academic and industrial bodies in order to enhance the development of novel RNA-based therapies for Duchenne muscular dystrophy. He is also a founding member and currently the Co-PI of the UK muscular dystrophy consortium known as MDEX. Furthermore, he is a member of the Oxford Parkinson's Disease Centre, and the Oxford Stem Cell Institute multidisciplinary teams and is currently co-leading a large EU Innovative Medicines Initiative to develop new strategies for delivery of drugs to the brain.

Summary:

The development of effective therapies for neuromuscular disorders such as Duchenne muscular dystrophy (DMD) is hampered by considerable challenges: skeletal muscle is the most abundant tissue in the body, and many neuromuscular disorders are multisystemic conditions. However, despite these barriers, there has recently been substantial progress in the search for novel treatments. In particular, the use of antisense oligonucleotides, which are designed to target RNA and modulate pre-mRNA splicing to restore functional protein isoforms or directly inhibit the toxic effects of pathogenic RNAs, offers great promise and these approaches are now being tested in the clinic (Muntoni and Wood, Nat Rev Drug Discov. 2011)

Today, Professor Wood will introduce the predominant therapeutic strategies in the antisense field whilst highlighting recent clinical findings that demonstrate the significant potential of these approaches for the development of novel therapies in neuromuscular diseases

担当・連絡先：遺伝子疾患治療研究部（内線 5221 Tsoumpra M, Hashimoto Y）