

NEXT GENERATION GENE THERAPIES FOR DMD

デュシェンヌ型筋ジストロフィーにおける
次世代遺伝子治療

Date : 令和 7 年 7 月 23 日 16:00~17:00

Venue : 研究所 3 号館 1 階 セミナールーム

(Seminar Room, 1st floor, Bldg. 3)

Dr. Jeffrey S. Chamberlain

- Professor, Dept. of Medicine, Division of Medical Genetics, University of Washington
- Director of the Seattle Wellstone Muscular Dystrophy Specialized Research Center
- Former president of American Society of Gene Cell Therapy
- McCaw Endowed Chair in Muscular Dystrophy

Dr. Jeffrey Chamberlain is a world-renowned expert in gene therapy research for muscular dystrophies, including **Duchenne Muscular Dystrophy (DMD)**. His work focuses on the development of gene therapy using AAV vectors carrying dystrophin genes. Recently, in his laboratory, a novel strategy has been developed to **overcome the packaging size limitation of adeno-associated virus (AAV)** vectors by splitting the large dystrophin gene into separate AAVs. [Nature 632(8023):192-200,2024] His research has significantly advanced the clinical application of treatments for DMD.

Representative papers include the following.



- "Expression of the murine Duchenne muscular dystrophy gene in muscle and brain" **Science** 239 (4846), 1416–1418 (1988)
- "Overexpression of dystrophin in transgenic mdx mice eliminates dystrophic symptoms without toxicity" **Nature** 364 (6439), 725–729 (1993)
- "New mdx mutation disrupts expression of muscle and non-muscle isoforms of dystrophin" **Nature Genetics** 4 (1), 87–93 (1993)
- "Dp71 can restore the dystrophin-associated glycoprotein complex in muscle but fails to prevent dystrophy" **Nature Genetics** 8 (4), 333–339 (1994)
- "Interactions between β 2-syntrophin and a family of microtubule-associated serine/threonine kinases" **Nature Neuroscience** 2 (7), 611–617 (1999)
- "Modular flexibility of dystrophin: implications for gene therapy of Duchenne muscular dystrophy" **Nature Medicine** 8 (3), 253–261 (2002)
- "rAAV6-microdystrophin preserves muscle function and extends lifespan in severely dystrophic mice" **Nature Medicine** 12 (7), 787–789 (2006)
- "Pericytes of human skeletal muscle are myogenic precursors distinct from satellite cells" **Nature Cell Biology** 9 (3), 255–267 (2007)
- "Split intein-mediated protein trans-splicing to express large dystrophins" **Nature** 632 (8023), 192–200 (2024)

連絡先： 遺伝子疾患治療研究部（青木 内線 5221）