シンポジウム

[4S02m]神経疾患遺伝子治療の最先端

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2022年7月3日(日) 09:00 〜 11:00 第2会場 (沖縄コンベンションセンター 会議場A1)

[4S02m-04]ALSの遺伝子治療

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キーワード：ALS, GENE THERAPY, MITOCHONDRIA, RIBOSOMES

Amyotrophic lateral sclerosis is a fatal neurodegenerative disease that affects motor neurons of the whole body to cause muscle weakness of the limbs and the trunk, dysarthria, dysphagia and respiratory failure. There is no effective treatment so far. Although the cause of the disease has not been fully understood, it is known that mislocalization and abnormal deposition of superoxide dismutase (SOD)1 or TAR-DNA binding protein (TDP)-43 occur in neurons of most ALS patients. SOD1 is an antioxidative enzyme localizing mainly in the cytoplasm, but ALS-linked mutant SOD1 mislocalizes in mitochondria and disrupts the function of the apparatus. TDP-43 is an RNA binding protein involved in the metabolism and transport of various RNAs, and it has been reported that disruption of the function may result in impaired activities of mitochondria and ribosomes in cell bodies or neurites of neurons. From this point of view, we aim to promote the function of mitochondria or ribosomes in ALS, and to develop a novel treatment strategy for ALS by increasing expression of genes related to these functions. We discuss the future of ALS gene therapy based on our data obtained so far.

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