

シンポジウム

## 革新的医療研究開発で挑む神経変性疾患

### — プリオン病治験体制の確立に向けて —

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（2015年2月14日(土) 於：名古屋国際会議場）

## Overcoming neurodegenerative diseases by innovative therapeutic developments

### — Toward the establishment of the clinical trial system for prion diseases —

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Organized by : Gifu University

Co-Organized by : ARO Council

Foundation for Biomedical Research and Innovation

Supported by : Ministry of Education, Culture, Sports, Science and Technology  
Ministry of Health, Labour and Welfare

(Saturday, February 14, 2015, Nagoya Congress Center, Aichi, Japan)

#### Abstract

According to the estimation of the Ministry of Health, Labour and Welfare (MHLW), approximately 7 million people may suffer from dementia in 2025 in Japan, which is approximately 20% of the people 65 years old or older. In the new dementia care program proposed by MHLW termed 'New Orange Plan' announced on January 27, 2015, they declared the promotion of the development of prophylaxis, diagnosis and treatment for dementia. On April 1, 2015, AMED (Japan Agency for Medical Research and Development) started to work on the financial support of the development of therapeutic drugs and medical devices. In this symposium, we will mainly focus on the prion diseases, a rare disease in dementia, and especially their therapeutic drug development.

In neurodegenerative diseases such as Alzheimer's, abnormal proteins replicate by themselves and kill the nerve cells of the brain. For their treatment, one of the promising strategies will be the stabilization of the normal structure by binding of specific ligands. To do that, we logically designed a medical chaperone whose efficacies for prion diseases and safeties were demonstrated according to ICH standards. As a result, the first-in-human clinical trial system for prion disease is going to be established and the Phase I/II trial will start soon. It will become the first step of the systematic therapeutic drug developments for the rare intractable diseases. I hope that the international clinical trial for rare diseases would be also promoted with this opportunity.

#### Key words

prion diseases, medical chaperone, clinical trial, dementia, intractable diseases

*Rinsho Hyoka (Clinical Evaluation)*. 2017 ; 44 : 659-735.