

# L-Arginine

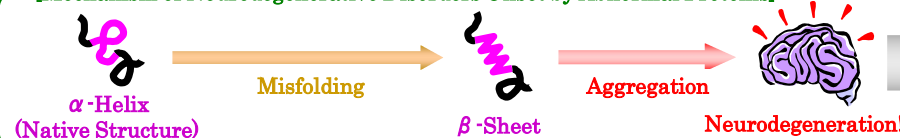
## ~ A Therapeutic Agent for PolyQ Diseases ~

### KEY INVENTION

L-arginine inhibits the aggregation of the polyglutamine (polyQ) proteins which are responsible for polyQ diseases.

➔ Medical Doctor-led Clinical Trials of L-arginine are ongoing as a feasibility study for polyQ diseases.

[Mechanism of Neurodegenerative Disorders Onset by Abnormal Proteins]

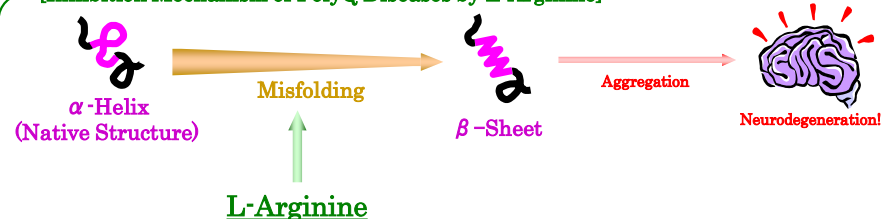


- Alzheimer Diseases
- Parkinson's Diseases
- **Polyglutamine (polyQ) Diseases**
- Amyotrophic Lateral Sclerosis (ALS)
- Frontotemporal Dementia (FTD) etc.

### SUMMARY of INVENTION

L-arginine inhibits the polyQ proteins from misfolding and aggregation.

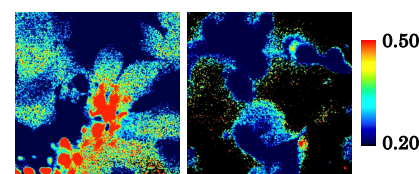
[Inhibition Mechanism of PolyQ Diseases by L-Arginine]



L-arginine crosses the blood-brain barrier after oral administration and inhibits polyQ proteins from misfolding.

➔ Aggregation inhibition of polyQ proteins suppresses the progression of polyQ diseases.

Inhibitory Effect of PolyQ Protein Aggregation by L-Arginine



COS-7 Cell with Fluorescent Proteins (Left : Control, Right : with L-Arginine)

Addition of L-arginine decreased protein aggregation.

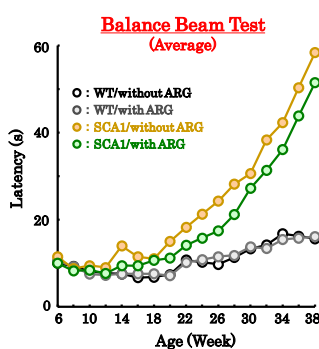
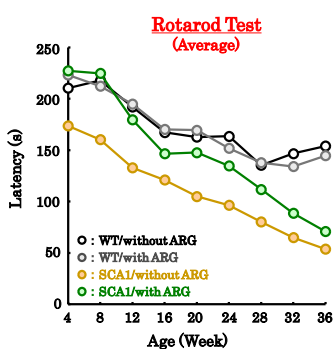
*Brain* 2020, 143, 1811-1825

### EFFECT of INVENTION

#### Effects on PolyQ Disease Mouse Models

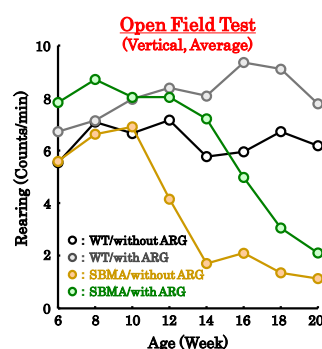
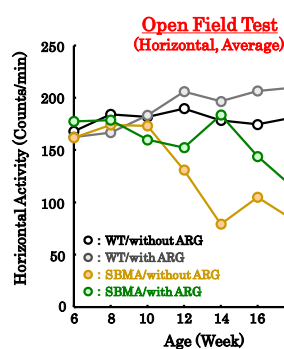
- Spinocerebellar Ataxia Type 1 (SCA1) : A slowly progressive cerebellar ataxia [Symptoms] Gait disturbance, Dysarthria, etc.
- Spinal & Bulbar Muscular Atrophy (SBMA) : A neurological disorder in which motor neurons gradually decrease [Symptoms] Muscle Weakness and Atrophy, etc.

#### [Effects on SCA1 Mice]



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#### [Effects on SBMA Mice]



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Motor dysfunction of SCA1/SBMA mice was suppressed by L-arginine administration.

### APPLICATION expected

- Application as a novel therapeutic agent (progression inhibitor) for polyQ diseases
- Application as a therapeutic agent (progression inhibitor) for other neurodegeneration disorders

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