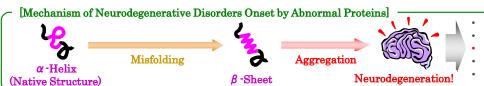
L-Arginine

~ A Therapeutic Agent for PolyQ Diseases ~

KEY INVENTION

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

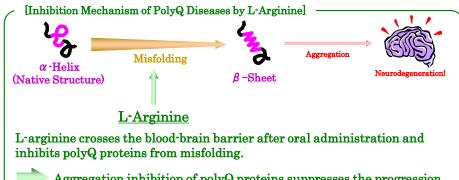
Medical Doctor-led Clinical Trials of L-arginine are ongoing as a feasibility study for polyQ diseases.



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

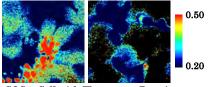
SUMMARY of INVENTION

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



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):

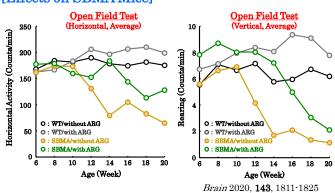
A slowly progressive cerebellar ataxia
[Symptoms] Gait disturbance, Dysarthria, etc.

• Spinal & Bulbar Muscular Atrophy (SBMA): A neurological disorder in which motor neurons gradually decrease [Symptoms] Muscle Weakness and Atrophy, etc.



Rotarod Test (Average) 200 200 0: WT/without ARG 0: WT/without ARG 0: WT/with ARG 0: SCAI/with ARG 0: SCA

[Effects on SBMA Mice]



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

APPLICATION expected

- O 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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Licensable Patent

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