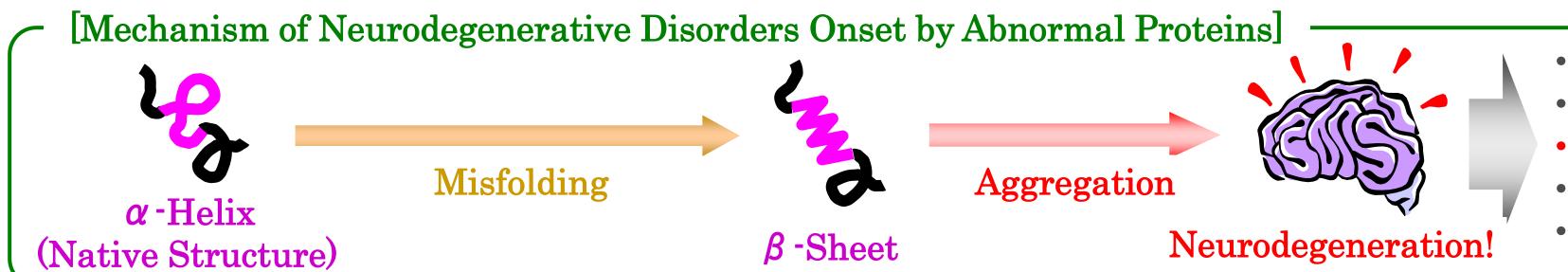
L-Arginine

~ A Therapeutic Agent for PolyQ Diseases ~

KEY INVENTION

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

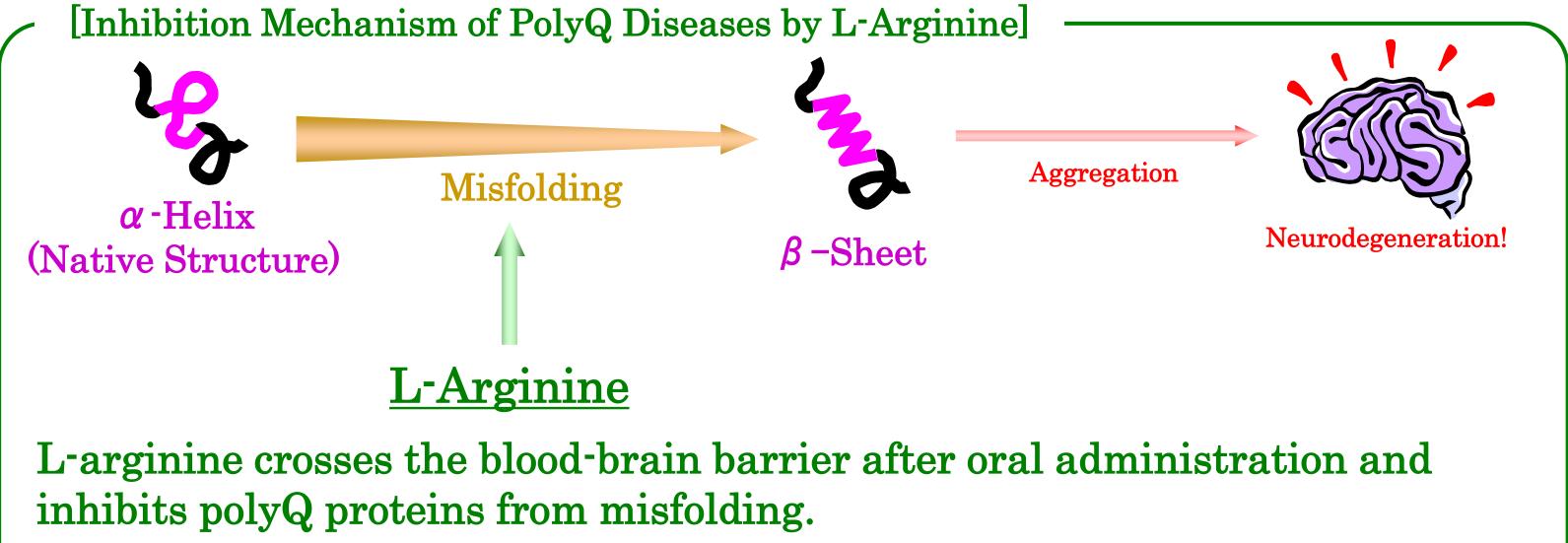
Medical Doctor-led Clinical Trials of L-arginine are ongoing as a feasibility study for polyQ diseases.



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

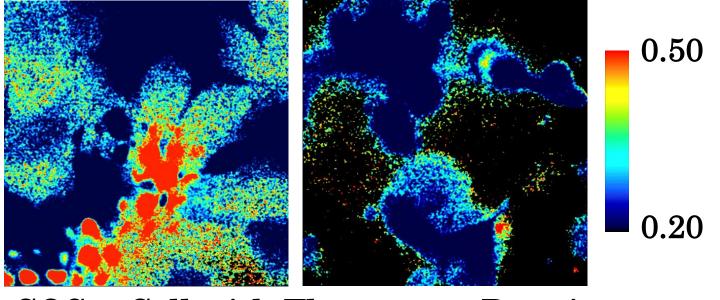
SUMMARY of INVENTION

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

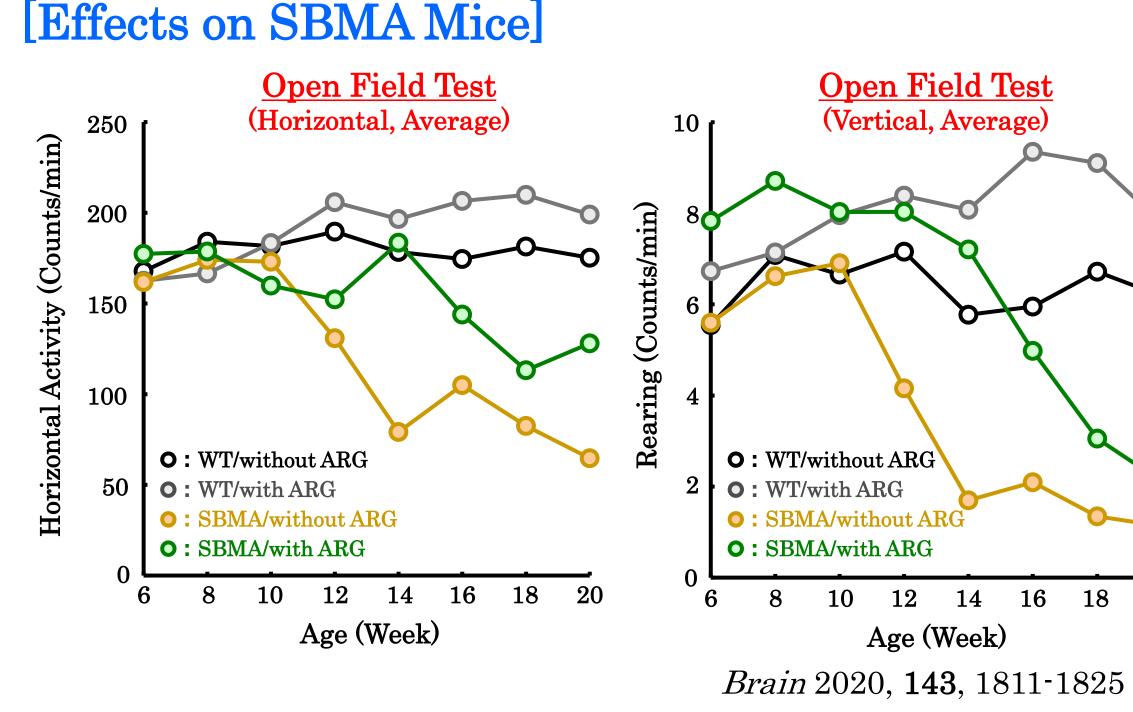
Effects on PolyQ Disease Mouse Models

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.

Effects on SCA1 Mice Balance Beam Test **Rotarod Test** (Average) (Average) **O**: WT/without ARG 200 • : WT/with ARG • : SCA1/without ARG (S) • : SCA1/with ARG **O**: WT/without ARG • : WT/with ARG • : SCA1/without ARG • : SCA1/with ARG 26 18 Age (Week) Age (Week) Brain 2020, **143**, 1811-1825



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

Representative Inventor:

Yoshitaka Nagai

(Professor, Kinki University)

Co-Inventor:

Eiko N. Minakawa (Assistant Professor, Kyoto University), et al.

Licensable Patent

Title of Invention:

International Publication No.: Contact:

IP Management & Licensing Group, Department of Intellectual Property Management, JST TEL)

Pharmaceutical Composition

+81-3-5214-8486 email) <u>license@jst.go.jp</u>

WO2017222040

URL) https://www.jst.go.jp/chizai/

