

L-Arginine

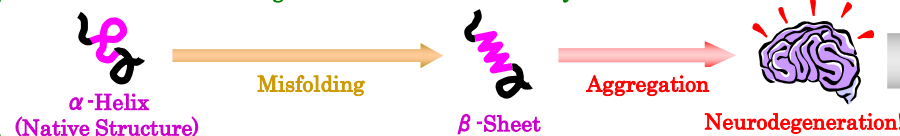
~ A Therapeutic Agent for PolyQ Diseases ~

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]

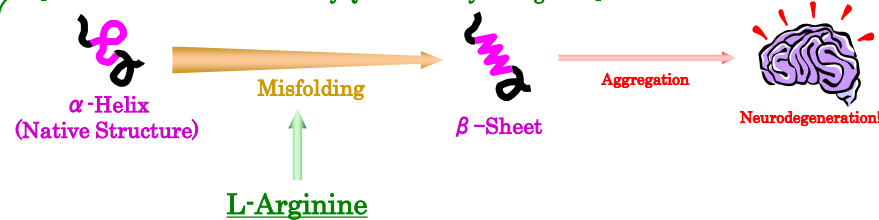


- Alzheimer Diseases
- Parkinson's Diseases
- **Polyglutamine (polyQ) Diseases**
- Amyotrophic Lateral Sclerosis (ALS)
- Frontotemporal Dementia (FTD) etc.

SUMMARY of INVENTION

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

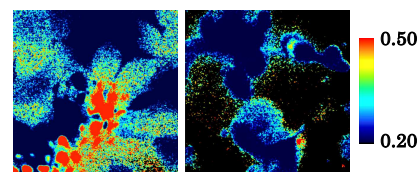
[Inhibition Mechanism of PolyQ Diseases by L-Arginine]



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.

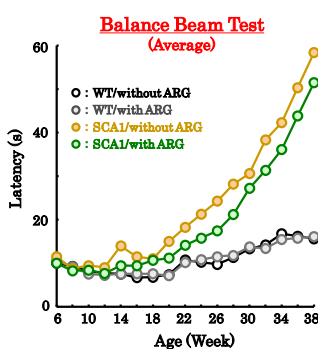
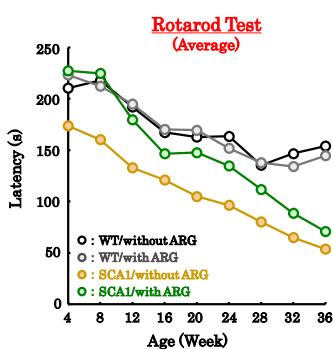
Brain 2020, 143, 1811-1825

EFFECT of INVENTION

Effects on PolyQ Disease Mouse Models

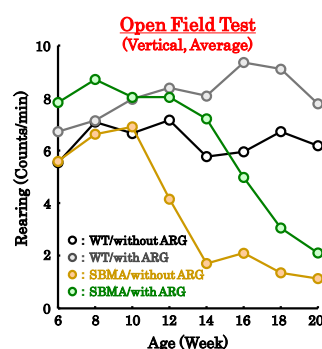
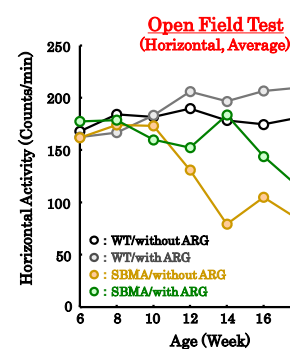
- Spinocerebellar Ataxia Type 1 (SCA1) : A slowly progressive cerebellar ataxia [Symptoms] Gait disturbance, Dysarthria, etc.
- Spinal & Bulbar Muscular Atrophy (SBMA) : A neurological disorder in which motor neurons gradually decrease [Symptoms] Muscle Weakness and Atrophy, etc.

[Effects on SCA1 Mice]



Brain 2020, 143, 1811-1825

[Effects on SBMA Mice]



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:

Pharmaceutical Composition
WO2017222040
IP Management & Licensing Group,
Department of Intellectual Property
Management, JST
TEL) +81-3-5214-8486
email) license@jst.go.jp
URL) www.jst.go.jp/chizai/

