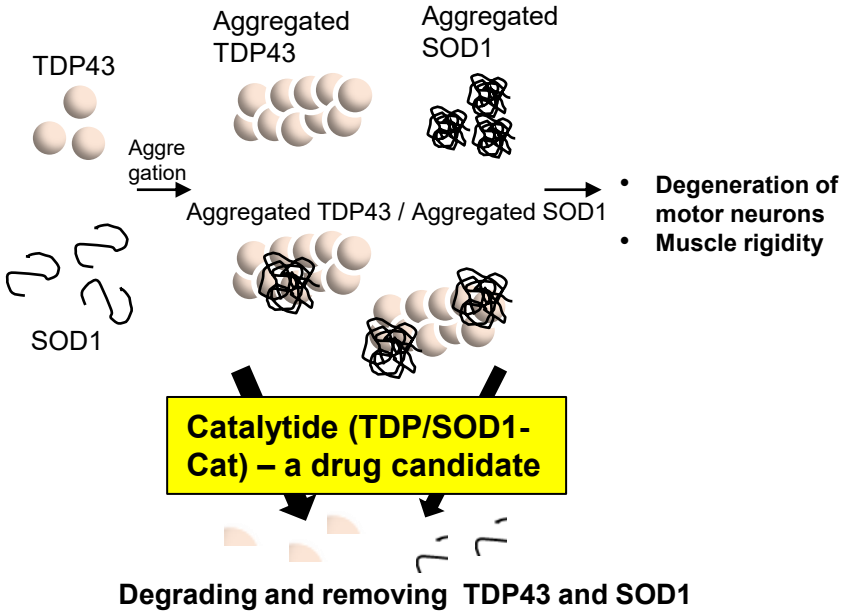
[Development of Amyotrophic Lateral Sclerosis Treatment]

<Current Treatments>

- Amyotrophic lateral sclerosis (ALS) is a progressive neurodegenerative disease caused by abnormal aggregation of TDP43 and SOD1; it is an extremely serious intractable disease that leads to death within a few years of onset due to respiratory muscle paralysis.
- No cure to date. Existing treatments are limited to symptomatic therapy.

There is a need for new approaches to break down and remove aggregated TDP43 or SOD1.



<Research Goal>
To develop a cure for ALS by using Catalytide which breaks down and removes abnormal aggregated proteins.

Results to Date

SOD1-related:

- Identification of nuclei of SOD1 aggregation
- Identification of Catalytides that degrade SOD1

TDP-43-related:

- Identification of nuclei of TDP aggregation

R&D Plan

SOD1-related:

(1) Structural optimisation of Catalytide for the degradation of Ag-SOD1

Structurally optimise the identified Catalytide and dtermine final compounds (SOD1-Cat).

(2) Evaluation of the pharmacological effects of SOD1-Cat

Evaluate pharmacological efficacy using SOD1 mutant model mice.

(3) Screening of Catalytides targeting Ag-TDP

Screen and structurally optimise Catalytides exhibiting Ag-TDP-degrading activity. Identify final compounds (TDP-Cat).

(4) Evaluation of pharmacological efficacy of TDP-Cat

Evaluate pharmacological efficacy using ALS model mice.

Timeline