

# AB103: Development of a Lead Gene Therapy Candidate for Parkinson's Disease Based on $\alpha$ -Synuclein Aggregation Inhibition

**ABrain Inc.**

**A $\beta$ Brain**

Ingenious Solutions to Neurodegenerative Disease

| NEUROLOGY                | Lead                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                       |
|--------------------------|----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Product Type             | Gene & Nucleic acids                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                       |
| Indication               | Parkinson's Disease                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                        |
| Target                   | $\alpha$ -synuclein                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                        |
| MoA(Mechanism of Action) | AAV9-mediated expression of AB103 blocks pathological $\alpha$ -synuclein aggregation and seed propagation by competitively interfering with $\beta$ -sheet transition and fibril maturation.                                                                                                                                                                                                                                                                                                                                                                              |
| Competitiveness          | AAV9-AB103 offers a differentiated disease-modifying strategy by providing sustained intracellular inhibition of pathological $\alpha$ -synuclein aggregation and seed-mediated propagation. Unlike antibody or $\alpha$ -synuclein-lowering therapies, AB103 is designed to act inside CNS cells while preserving physiological $\alpha$ -synuclein expression. Through single-dose AAV9-mediated delivery, AB103 may provide durable target engagement and potential advantages over small-molecule aggregation inhibitors and neurotrophic factor-based gene therapies. |
| Development Stage        | Lead                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                       |
| Route of Administration  | Intracerebroventricular (ICV) injection                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                    |

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