AVIADOBIO
Chasing **Cures.** Delivering **Hope.**™

Frontotemporal dementia with progranulin mutations research (FTD-GRN)

AVIADOBIO: CHASING CURES. DELIVERING HOPE.™

At AviadoBio, we are relentlessly chasing cures by developing and translating groundbreaking science and precision delivery into life-changing medicines across neurodegenerative diseases, beginning with frontotemporal dementia (FTD).

With our deep understanding of the brain and our suite of proprietary gene therapy platforms and delivery technologies, we are working to overcome the challenges of delivering the right drug to the right place. Our innovative, neuroanatomy-led approach is designed to maximize the therapeutic potential of gene therapy to potentially delay or even halt the progress of neurodegenerative diseases - conditions that deprive people of the prime years of their lives and have a devastating impact on patients, families, and loved ones.

WHAT IS FRONTOTEMPORAL DEMENTIA?

Frontotemporal Dementia (FTD) is a devastating form of early-onset dementia that varies in each individual. Symptoms of FTD can include changes in personality or uncharacteristic behaviors, progressive loss of language, loss of executive function and cognitive abilities, apathy, and reduced mobility.[1-3] FTD causes a substantial reduction in life expectancy, and on average people with FTD die 3-13 years from diagnosis.[4-7]

FTD can be classified as sporadic or familial. Sporadic FTD is more common and occurs when only one person in a family is affected. Familial FTD is an inherited form of the disease. While some familial FTD has no known cause, a strong family history is found in about one-third of cases. Most inherited FTD is driven by autosomal dominant disease-causing mutations in three genes, including GRN.[8] Mutations in GRN account for about 10% of all FTD cases.[9]

Access to genetic counseling and testing is an important step for patients with FTD to determine if their disease is the result of a genetic mutation. While there are currently no approved disease-modifying treatments for FTD, learning whether FTD is caused by a genetic mutation may help clinicians to determine if patients are eligible for any clinical trials.

WHAT IS A PROGRANULIN MUTATION?

This is a mutation in the GRN gene. Genes are instructions that tell the body's cells what to do. Sometimes, genes contain errors called mutations.

The GRN gene tells the body to make progranulin, which is a protein that plays an important role in the healthy functioning of cells in the brain. When there is a mutation in the GRN gene, the body may not make enough progranulin, which can lead to cell death in the brain and the symptoms of FTD.

WHAT IS AVB-101?

AVB-101 is an investigational one-time therapy designed to deliver a functional copy of the GRN gene directly to the brain, thereby potentially restoring progranulin levels and stopping disease progression in patients with FTD-GRN. It is delivered into the brain using a neurosurgical procedure. AVB-101 has been granted orphan designation by the U.S. Food and Drug Administration and the European Commission.

ASPIRE-FTD CLINICAL TRIAL FOR FTD-GRN

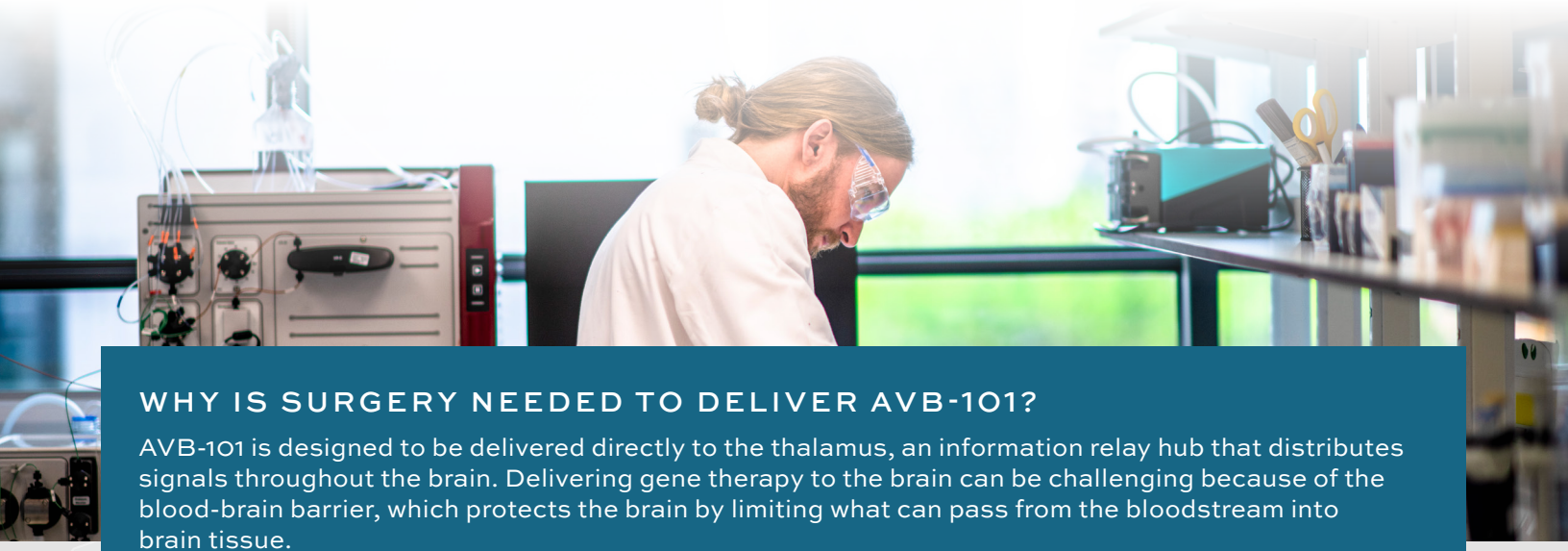
All clinical trials have specific eligibility criteria for participants to ensure that research is well-controlled. For FTD clinical trials, this may include a specific genetic profile for FTD, how quickly the disease is advancing, and how far the disease has already progressed. People with advanced FTD may be excluded from the opportunity to participate in certain trials. Not all patients will qualify for all studies.

If you are interested in participating in a study, it is critical that you speak to your neurologist to learn more about opportunities that might be best for you.

ASPIRE·FTD

ASPIRE-FTD is a Phase 1/2 open-label, multi-center study designed to evaluate the safety and preliminary efficacy of AVB-101 in patients with FTD-GRN. Individuals may be eligible to participate if diagnosed with FTD-GRN (confirmed with a genetic test), among other criteria.

For more information about the ASPIRE-FTD study and to find clinical trial sites, visit: aspire-ftd.com and clinicaltrials.gov/study/NCT06064890.



WHY IS SURGERY NEEDED TO DELIVER AVB-101?

AVB-101 is designed to be delivered directly to the thalamus, an information relay hub that distributes signals throughout the brain. Delivering gene therapy to the brain can be challenging because of the blood-brain barrier, which protects the brain by limiting what can pass from the bloodstream into brain tissue.

A minimally invasive neurosurgical procedure is used to deliver AVB-101 directly to the thalamus, bypassing the blood-brain barrier and enabling distribution across the brain. This targeted approach focuses treatment where it is needed the most.

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