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Advancing Health Through Innovation.

Data sheet

3D Blood-Brain Barrier (BBB)

ALS/FTD Disease Model

Catalog Number	BBB-ALSFTD-382T	**Version**	24-well plate
Culture Format	24-well Transwell® inserts	**Mutation**	TARDBP A382T, heterozygous

Amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD) are progressive neurodegenerative disorders for which improved human-relevant experimental systems are urgently needed. The NeoBioPharma 3D Blood-Brain Barrier (BBB) ALS/FTD Disease Model provides a multicellular human neurovascular platform designed to model disease-relevant barrier, glial, vascular, and motor-neuron interactions in a controlled in vitro setting.

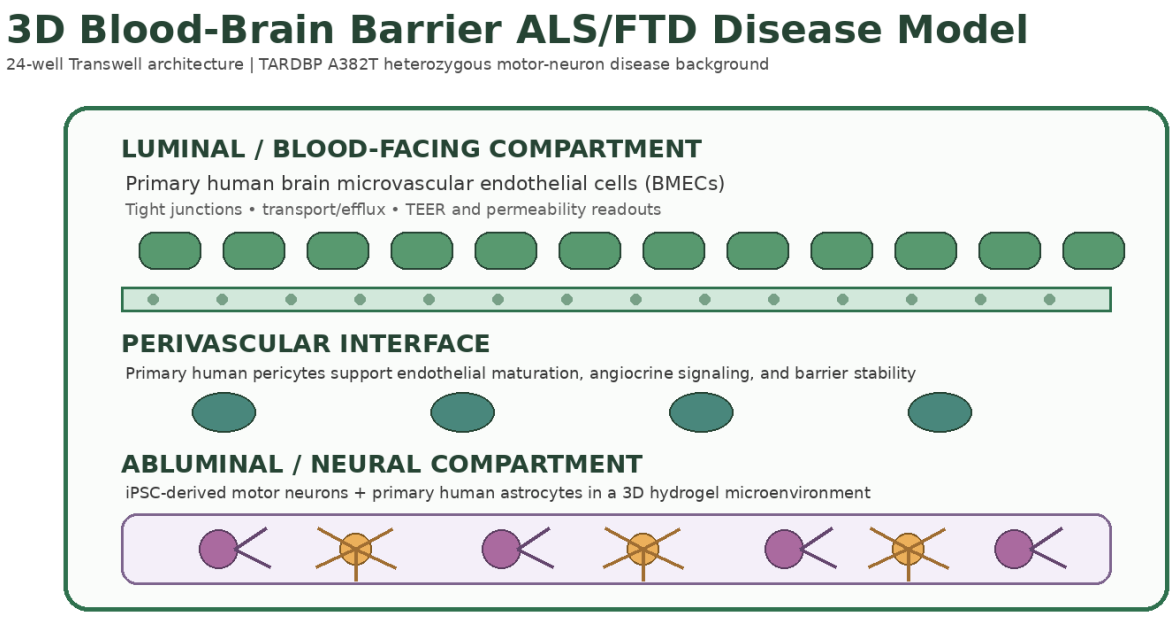

Figure 1. Conceptual architecture of the multicellular 3D neurovascular unit model.

Product positioning: A humanized 3D BBB disease model for mechanism studies, biomarker discovery, CNS permeability assessment, and preclinical therapeutic evaluation in ALS/FTD research.

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1. Product Overview

The 3D Blood-Brain Barrier (BBB) ALS/FTD Disease Model is a next-generation in vitro human cell-based platform engineered to recapitulate key features of the neurovascular unit (NVU) in the context of ALS and FTD. Unlike traditional 2D monolayers, the model integrates multiple human cell populations within a physiologically relevant three-dimensional microenvironment intended to support multicellular organization, barrier integrity, extracellular matrix interactions, and longer-term functional connectivity. The platform is designed to help bridge the translational gap between conventional cell culture, animal models, and human studies.

2. Key Features & Advantages

- Clinically relevant genetic background: motor neurons carry a heterozygous A382T mutation in TARDBP, a pathogenic variant associated with ALS/FTD.
- Human iPSC-derived disease-relevant neurons: motor neurons are differentiated from a well-characterized human induced pluripotent stem cell line to provide a controlled genetic background.
- Full neurovascular unit composition: combines motor neurons, primary human astrocytes, primary human pericytes, and primary human brain microvascular endothelial cells (BMECs).
- 3D biomimetic scaffolding: peptide-functionalized hyaluronic acid hydrogel supports cell-cell and cell-matrix signaling, tissue-like spatial organization, and extracellular matrix deposition.
- Functional barrier readouts: designed for TEER and permeability measurements, including small-molecule and biologic transport studies.
- Disease-relevant phenotypes: supports evaluation of TDP-43 cytoplasmic mislocalization/aggregation, cellular stress responses, glutamate excitotoxicity, and related ALS/FTD phenotypes.

3. Cell Composition & Genotype

Cell Type	Source / Genotype	Function in Model
Motor Neurons	Human iPSC-derived; TARDBP A382T heterozygous	Primary disease-relevant neuronal population; supports analysis of TDP-43 pathology, axonal degeneration, stress responses, and neuronal viability.
Astrocytes	Primary human	Provide metabolic and trophic support; contribute to glutamate handling, neuroinflammatory signaling, and BBB maintenance.
Pericytes	Primary human	Support endothelial maturation, capillary-like regulation, angiocrine signaling, and barrier stability.
BMECs	Primary human	Form the blood-facing barrier; express tight-junction proteins and transport/efflux systems including ZO-1, Occludin, P-gp, and BCRP.

	Matched control: An isogenic gene-corrected wild-type control model is available under catalog number BBB-ALSFTD-WT.

4. Model Architecture & Culture Specifications

Parameter	Specification
Scaffold Material	Synthetic peptide-functionalized hyaluronic acid hydrogel; nominal elastic modulus 1-3 kPa, tunable.
Culture Format	24-well Transwell® inserts.
Cell Populations	Motor neurons, astrocytes, pericytes, and BMECs.
Maturation Time	Approximately 14-21 days post-seeding to establish stable barrier function and mature multicellular interactions.
Incubation Conditions	37 °C, 5% CO_2, humidified incubator; serum-free, chemically defined medium supplied.
Barrier Integrity Target (Day 21)	TEER ≥ 150 Ω·cm^2; sodium fluorescein apparent permeability (Papp) < 1.0 × 10^{-6} cm/s.

5. Quality Control & Validation

- Genotyping — Sanger sequencing confirms the heterozygous A382T mutation in TARDBP.
- Identity / lineage markers — target purity >95% for each cell population, evaluated using lineage-appropriate markers such as NeuN (neurons), GFAP (astrocytes), PDGFRβ (pericytes), and CD31/VE-cadherin (BMECs).
- TDP-43 pathology — immunofluorescence target: >40% of motor neurons demonstrating cytoplasmic TDP-43 aggregates at Day 21 versus <5% in the isogenic wild-type control.
- Barrier performance — TEER and permeability measurements assessed across independent replicates with target coefficient of variation (CV) <15%.
- Sterility and mycoplasma — negative for bacterial/fungal contamination and mycoplasma by PCR-based testing.

6. Intended Applications

Application Area	Examples of Use
Disease Mechanism Studies	Investigate TDP-43 nucleocytoplasmic shuttling, RNA-processing defects, axonal degeneration, stress pathways, and non-cell-autonomous neurotoxicity.
Biomarker Discovery	Profile exosomes, cytokines, miRNAs, and other secreted factors sampled from luminal and abluminal compartments.
Drug Permeability & Efflux	Assess CNS penetration, directional transport, and efflux behavior of small molecules and biologics across a diseased human BBB context.
Preclinical Therapeutic Evaluation	Evaluate neuroprotective compounds, antisense oligonucleotides (ASOs), gene-therapy vectors, and other experimental modalities in a human-relevant multicellular system.
Inflammatory & Metabolic Profiling	Quantify IL-6, TNF-α, reactive oxygen species (ROS), and metabolic/stress responses under basal or experimentally challenged conditions.

7. Recommended Readouts

- Barrier function: TEER, sodium fluorescein permeability, dextran permeability, junctional protein localization.
- Neuronal health: viability, neurite/axon integrity, synaptic markers, stress granules, TDP-43 localization.
- Inflammation: cytokine/chemokine panels, astrocyte activation markers, oxidative stress indicators.
- Transport biology: P-gp/BCRP activity, apical-to-basolateral and basolateral-to-apical transport.
- Molecular profiling: qPCR, immunocytochemistry/immunofluorescence, secretome analysis, targeted RNA or protein panels.

8. Storage, Shipping & Handling

Item	Requirement
Cryopreserved Cell Components	Store at ≤ -80 °C as specified on the component label. Avoid repeated freeze-thaw cycles.
Culture Media	Store at 2-8 °C. Do not freeze unless specifically indicated. Protect from prolonged light exposure.
Thawing	Use a validated 37 °C water bath and gently agitate until a small ice crystal remains; promptly transfer cells into the appropriate pre-warmed medium according to the product manual.
Shipping	Dry ice for cryopreserved components; other supplied components should be handled according to package labeling.

9. Limitations & Precautions

- For Research Use Only (RUO). Not intended for diagnostic, prophylactic, or therapeutic use in humans.
- The model is designed to reproduce selected early-to-mid disease-associated features. More advanced or chronic pathology may require extended culture (>28 days) and/or validated experimental stress paradigms.
- Test compounds with cytotoxic, detergent-like, or barrier-disrupting properties may directly alter TEER and permeability. Include vehicle, solvent, and appropriate cytotoxicity controls.
- Recommended study design: at least 3 biological replicates per condition, with at least 2 technical inserts per biological replicate.
- Performance can be influenced by operator technique, incubator conditions, assay platform, and sampling schedule. Follow the lot-specific product manual and QC documentation.

10. Technical Support & Customization

NeoBioPharma provides technical support for model setup, protocol troubleshooting, assay optimization, and data interpretation. Custom configurations, experimental endpoints, and matched control options may be available for project-specific needs.

	Document note: This datasheet is intended for informational purposes and may be updated without notice. Consult the current product manual, certificate of analysis (COA), and applicable safety data sheets (SDS) before use.

11. Scientific Background References

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9. Patent WO2019195798A1. “Human pluripotent stem cell derived neurodegenerative disease models on a microfluidic chip.”
10. University of Malaga. PhD thesis, “Human-Derived Blood-Brain Barrier In Vitro Models as Reliable Tools for Research and Therapeutics,” 2024.

Reference note: The background references above are presented as supplied for this datasheet and should be independently checked against the source record before external publication.