# Brain Therapeutic Shuttle

Shuttling Charged Therapeutic Molecules Across the Blood-Brain-Barrier For Enhanced Efficacy

Los Alamos NATIONAL LABORATORY

TECHNOLOGY SNAPSHOT

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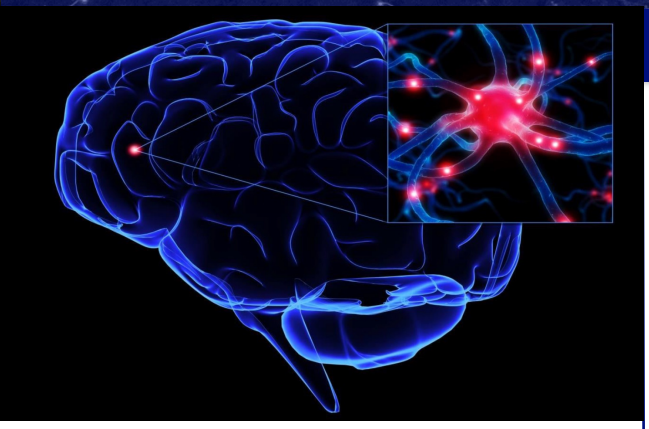

## APPLICATION AREA

Sector: Biotech, Life Sciences & Healthcare

Area: Biochemistry

Industry: Pharmaceutical

Market: Neurological Treatment Delivery; Blood-Brain-Barrier

## PARTNERSHIP OPPORTUNITIES

We are seeking a collaborative pharmaceutical partner with an interest in furthering the development of this technology into a reliable drug delivery platform. This technology is available for a:

- ✓ Cooperative Agreement
- ✓ License

## CONTACT

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## TECHNOLOGY READINESS LEVEL: 4

Process and Component Validation

## IP information

Patent pending (S167618)

## OVERVIEW

This Brain Therapeutic Shuttle is a neurological treatment delivery mechanism that enables increased efficacy due to its ability to cross the blood-brain-barrier (BBB). The technology addresses pharmaceutical companies' dissatisfaction with the low percentage of therapeutics (less than 5%) that are able to penetrate the BBB. The central nervous system is comprised of the brain and spinal cord, where the structure and function are directly affected by neurological diseases and disorders. Current treatment regimes require large and repetitive dose administration resulting in high therapeutic bioavailability in the peripheral nervous system rather than the central nervous system, consequently increasing prevalence of adverse side effects.

The Brain Therapeutic Shuttle enables the passage of small molecules across the BBB. This small biomimetic molecule is designed to penetrate the BBB and can carry therapeutic molecules across where they are released for greatest effectiveness.

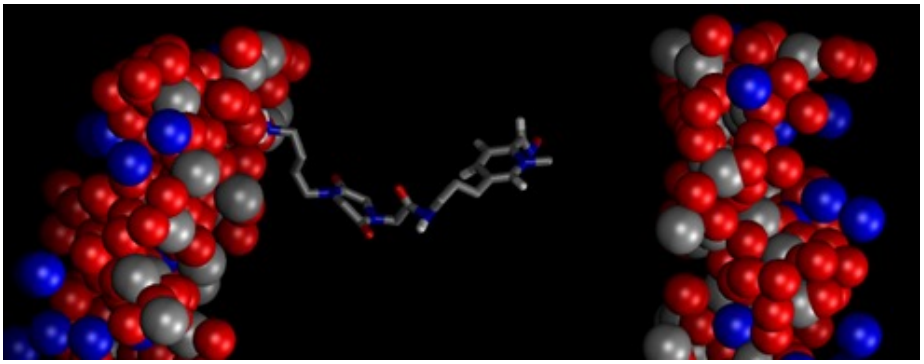
The global neurological treatment delivery market is growing rapidly due to increasing public awareness and incidence of neurological disorders as well as greater investment in solutions. The Brain Therapeutic Shuttle is an enabling technology with applications in the central nervous system pharmaceutical market, opening new possibilities for additional neurological treatments.

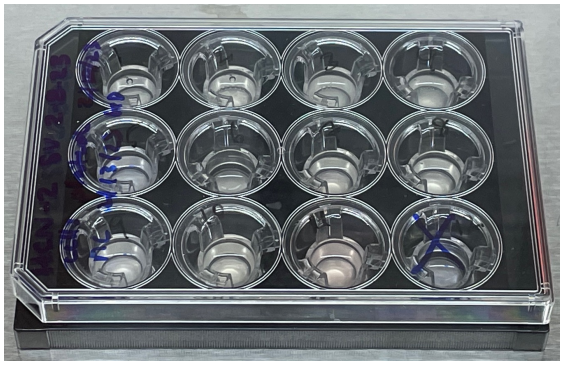
## ADVANTAGES

- ☑ Easily synthesized
- ☑ Can be tailored to specific BBB transfer mechanisms
- ☑ Biomimetic and non-toxic
- ☑ Improves bioavailability of therapeutics
- ☑ Broadens pipeline of potential therapeutics for brain disorders and neurological diseases



NNSA National Nuclear Security Administration





## TECHNOLOGY DESCRIPTION

The Brain Therapeutic Shuttle was designed to address the need for therapeutics to cross the BBB by employing the unique multidisciplinary expertise and integration at Los Alamos National Laboratory. The therapeutic shuttle is designed to fold in a predictable manner to minimize the exposure of polar residues in the hydrophobic region of the BBB, enabling charged molecules to safely pass. Thus, the brain is “tricked” into allowing a charged molecule to pass. Simulations and human BBB models have been utilized to predict and validate *in vitro* BBB passage. Fluorescence and mass spec techniques were used for detection. The shuttle structures can be easily designed and optimized to help transport other therapeutics for a multitude of treatment applications.

## MARKET APPLICATIONS

Central nervous system diseases are a group of neurological disorders that affect the structure or function of the brain or spinal cord. The global central nervous system therapeutic market size is valued at $120 billion and the global BBB market alone has exceeded $2 billion and is expected to surpass $7 billion in 2028. Advancements in diagnostics and therapeutics of central nervous system diseases are expected to increase the treatment rate globally. Moreover, the increasing prevalence of neurological disorders and the rising demand for effective therapeutics options are expected to boost market growth. The high unmet medical need has led all the major pharmaceutical companies to invest and develop novel therapies for the treatment of central nervous system diseases. This product serves as a tool to expand their drug pipeline bioavailability for treatment of neurodegenerative diseases, neurodevelopmental disorders and traumatic injuries. This technology could immediately improve treatments of Parkinson’s disease, Alzheimer’s disease, Hunter’s syndrome, Multiple Sclerosis, Epilepsy and brain cancer.

## NEXT STEPS

Los Alamos researchers have demonstrated proof-of-concept and validation that the Brain Therapeutic Shuttle can transport multiple therapeutics across the *in vitro* BBB. We are seeking collaboration partners to prepare for pre-clinical trials and expand utility of the shuttles by exploring additional therapeutic applications.