

Brain Therapeutic Shuttle

A blood-brain barrier shuttle and companion screening platform for developing treatments that need to reach the brain.

Value Proposition

The blood-brain barrier helps protect the brain by limiting what can pass from the bloodstream into the central nervous system. While this protection is important for health, it can also prevent treatments from reaching the areas where they are needed. As a result, developing treatments for the brain can be challenging because many of them cannot cross the blood-brain barrier in sufficient amounts to reach the brain.

The Brain Therapeutic Shuttle is part of Guided Access for Therapeutic Entry (GATE), a platform developed to address this challenge. GATE combines a small-molecule shuttle designed to improve transport small molecule therapeutics across the blood-brain barrier with a companion screening assay that evaluates the therapeutic response upon passage.

Together, these technologies support the development and evaluation of treatments that need to reach the central nervous system.

Technology Readiness Level 4

IP Information

This technology is protected under the following intellectual property:

- U.S. Patent No. 11,084,794

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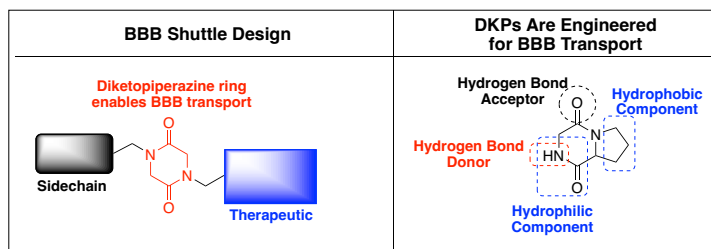


Figure 1. The Brain Therapeutic Shuttle uses a diketopiperazine (DKP) structure that can be linked to a variety of therapeutics to help them cross the blood-brain barrier.

Overview

Successfully developing treatments for the brain requires solving two challenges: getting a treatment across the blood-brain barrier and determining whether it still works after it reaches the brain.

GATE addresses both challenges through two complementary technologies:

- A blood-brain barrier shuttle that is readily conjugated to treatments to assist in passage into central nervous system.
- A screening assay that evaluates therapeutic response after barrier passage.

The technologies can be used together or independently, depending on development objectives.

Advantages

- Two complementary technologies that can be deployed together or independently
- Blood-brain barrier shuttle designed to improve delivery of treatments to the central nervous system
- Screening assay evaluates whether a treatment can cross the blood-brain barrier and still work after delivery
- Evaluates delivery and treatment effectiveness within a single experiment
- Supports development and evaluation of treatments that need to cross the blood-brain barrier

Technology Description

GATE consists of two complementary technologies: a blood-brain barrier shuttle and a companion screening method.

The shuttle uses LANL-developed diketopiperazine (DKP) molecules that can be linked to treatments. The DKP molecules are biomimetic, meaning they are designed to mimic characteristics found in natural biological systems. When attached to a treatment, they fold into shapes that change how the treatment interacts with the blood-brain barrier. This helps the treatment move through the barrier and into the central nervous system.

The companion screening method uses a human blood-brain barrier model to evaluate two questions during a single experiment: whether a treatment crosses the barrier and whether it still works after crossing. By combining both measurements into a single test, the method provides information about delivery and function at the same stage of evaluation.

The shuttle and screening method can be used together to support development of treatments that need to cross the blood-brain barrier. Because the technologies address different aspects of drug development, they may also be used independently depending on application needs.

Market Applications

- Development of treatments that need to reach the central nervous system
- Drug discovery and preclinical screening
- Neurological and neurodegenerative disease research
- Central nervous system oncology applications
- Infectious disease and antiviral research involving the central nervous system
- Biodefense and medical countermeasure development
- Reducing animal studies and therefore cost savings

Contact

To learn more or to discuss potential interest in this technology, please contact the Feynman Center for Innovation at licensing@lanl.gov.