

## The Myelin Repair Foundation's Translational Medicine Platform

*Translating Scientific Discoveries into Faster, Effective New Patient Treatments*

### THE PROBLEM:

#### Patients Waiting Endlessly for a Cure

The Myelin Repair Foundation (MRF) is addressing a significant and extremely difficult medical research problem. This challenge is known as the translational gap or the “valley of death” — the vast divide between university labs and the entities that can take scientific discoveries and turn them into therapies for patients.

For serious consideration of potential drugs developed in academic laboratories, the pharma industry requires comprehensive validation of the target prior to its acceptance into the company's pipeline. Pharma companies are reluctant to invest resources to replicate and validate research studies that often lead to the disappointing conclusion that the results are not in fact consistently reproducible. It has been easier for the industry to invest in already-successful therapeutic approaches to disease, therefore shying away from investigating new therapeutic pathways. In addition, there is no incentive for academic labs to conduct tedious replication studies to confirm validation of research results.

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In the current drug development system, the chance that any single discovery will actually reach patients in the clinic is **less than one in 1,000**.

The deepening gap between academic labs and pharma over the past several decades has been a major factor in the decrease in new drug approvals, with potential therapeutics unable to move forward to the clinic.

Academia  
DISCOVERIES

Pharma  
THERAPIES



The diagram shows a dark, jagged valley between two hills. The left hill is labeled 'Academia DISCOVERIES' and the right hill is labeled 'Pharma THERAPIES'. The valley itself is labeled 'VALLEY OF DEATH' in large, bold, white letters. The letters 'TMP' are faintly visible in the background of the valley.

### OUR SOLUTION:

#### The Translational Medicine Platform (TMP)

The Myelin Repair Foundation's Translational Medicine Platform (TMP) de-risks new disease approaches for the pharmaceutical industry, clearing the pathway to clinical trials, accelerating therapeutics to patients. Led by MRF scientists with over a combined century of pharma experience, the Translational Medicine Platform establishes a new and comprehensive model for drug development to meet patient need. This ground-breaking and systemic solution facilitates the smooth transfer of promising drug discoveries from the lab bench to the pharma industry for myelin repair therapeutics for multiple sclerosis, establishing an efficient, effective drug discovery paradigm for all diseases.

The TMP bridges the “valley of death” by industrializing the target validation process through a methodical assessment gauging the readiness of myelin repair drug targets for clinical trials. Specifically, the Myelin Repair Foundation designed the Translational Medicine Platform to:

- Assess drug candidates from industry, other academic labs, and from the MRF's own research lab, using a superior array of research tools and models developed by the MRF, which are applied in a methodical, strategic and consistent manner;

## MRF Translational Medicine Platform

- Develop—and continue to cultivate—new animal and human research tools and models for myelin, along with biomarkers for myelin repair;
- Evaluate hundreds, if not thousands, of compounds systematically to assess many compounds simultaneously—freeing MRF from depending on any one discovery to make achieve FDA approval. As a result, the MRF will be able to advance many potential drug targets for “multiple shots on goal”;
- Advance therapeutics that show consistent results for myelin repair in the TMP, helping MRF

scientists identify which promising scientific discoveries will most likely improve human health, increasing the chances for clinical trials success

### What This Means

The TMP will not only accelerate getting a myelin repair treatment to MS patients, but also will demonstrate the utility of this type of target evaluation “engine” for all other diseases. The TMP is applicable to speed new treatments for all diseases, designed to change the “status quo” of how all medical research is conducted. ■

## The MRF Translational Medicine Center

The Myelin Repair Foundation’s Translational Medicine Center (TMC) is the centerpiece of the industry-focused translational medicine platform. Launched in the final quarter of 2011, the Translational Medicine Center’s MRF staff scientists evaluate the



most promising myelin research by utilizing and building upon the biological assays originally developed in MRF’s core academic labs. The TMC provides access to MRF-developed assays and evaluates discoveries made in neuroscience labs anywhere in the world. This capability at the TMC accelerates the validation process for a myriad of myelin repair discoveries beyond even those achieved within MRF.

industry an unprecedented and hugely significant benefit in an industry-rigorous laboratory facility setting that can drastically accelerate the delivery of effective myelinating drugs to patients.

Led by MRF research staff with in-depth expertise moving therapeutic compounds into clinical trials, the TMC offers the

### A New Role for Non-Profits to Accelerate New Treatment Discoveries

The MRF is a non-partisan, nonprofit organization connecting various entities in the drug discovery continuum in order to achieve a singular goal: shortening the time to market for new medicines. The MRF is in a unique position to respond quickly and effectively to drug discovery challenges, identifying the fastest, most effective routes across the “valley of death”. The Myelin Repair Foundation’s Translational Medicine Platform facilitates improved collaboration between academia, nonprofit organizations and pharma companies to accelerate the development of new patient treatments to the clinic.



*Collaboration. Acceleration. Results.*

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