

# Myelin Repair Foundation

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## Research Areas



## At a Glance

- Status: **Active Consortium**
- Year Launched: **2002**
- Initiating Organization: **Myelin Repair Foundation**
- Initiator Type: **Nonprofit foundation**
- **Rare** disease
- Location: **North America**

## Abstract

The Myelin Repair Foundation (MRF, [www.myelinrepair.org](http://www.myelinrepair.org)) is a nonprofit foundation focused on accelerating the development of myelin repair therapeutics for multiple sclerosis. MRF is working to accelerate the development of therapeutics through a collaborative model called the Accelerated Research Collaboration™ (ARC). The ARC framework coordinates and manages the entire therapeutic development continuum from discovery biology to U.S. Food and Drug Administration (FDA) approval. The model works by coordinating multidisciplinary basic research from academic and government laboratories, systematically validating and de-risking potential compounds/targets, and collaborating with pharma partners to increase the probability of successful programs.

## Mission

MRF focuses on developing myelin repair treatments to improve the lives of people suffering from multiple sclerosis (MS). To this effect, MRF has defined a 15-year research plan to develop a drug or drugs. MRF believes its ARC model can subsequently be used to accelerate the treatment for all diseases.

## Consortium History

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MRF was established in 2004 by MS patient Scott Johnson at a time when little was known about myelin repair. Its laboratory facility, the Translational Medicine Center, was launched in 2011 to advance myelin repair discoveries toward commercial development. Staffed by MRF personnel, its research activities build upon the basic research conducted within the Discovery Biology program by bridging the translational gap. It focuses on validating and assessing the targets/compounds identified by the MRF team, by non-MRF affiliated academic research laboratories, and by industry for clinical trial readiness.

Recognizing the incentives and limitations of academic scientists, commercial biopharma, and government regulators, MRF uses the ARC model to develop a clear pathway from bench to bedside by leveraging its nonprofit position to build and manage collaborations at various points and among various participants in the therapeutic development continuum.

In 2012, MRF had two candidates in Phase 1 clinical trials. The first trial, conducted at the Cleveland Clinic, examines the efficacy of a new myelin repair therapeutic pathway with mesenchymal stem cells. This is in addition to a peptide-coupled tolerance study that is currently in Phase 1 clinical trials in Hamburg, Germany.

## Structure & Governance

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A Board of Directors consisting primarily of business executives is used to guide MRF's overall mission. The Executive Team manages day-to-day operations as well as long-term strategy. The principal investigators that MRF funds conduct studies from their respective academic institutions and collaborate to investigate scientific challenges in myelin. MRF's research staff of eight scientists work with the MRF-supported principal investigators and guide MRF's discovery biology research, translational research, pharma partnerships, and clinical programs.

The research plan is divided into three components:

- Discovery Biology is a collaboration that convenes [principal investigators](#) with complementary knowledge in neurobiology, immunology, and myelin research. Their research is organized by a

research plan that addresses seven key areas that are believed to hold the answers to myelin repair. The plan is reviewed and revised annually by a [Scientific Advisory Board](#).

- Translational Medicine Platform is a collaborative framework that replicates and validates the findings from Discovery Biology by creating novel assays and identifying biomarkers. This platform is used to de-risk findings from MRF's principal investigators, other academic labs, and biopharma partners. It also ensures the clinical relevance of the experiments conducted by the Discovery Biology team and helps set priorities for further development of the most promising therapeutic targets.
- Clinical Development is a framework that focuses on advancing the most promising drugs evaluated by the Translational Medicine Platform into clinical trials. It also assists in designing and performing clinical trials. The end objective is to ensure a positive patient outcome.

Advisory boards provide expert guidance to each of the three components of the therapeutic development continuum:

- The Scientific Advisory Board, consisting primarily of global academic experts, provides oversight and input to the MRF academic research consortium, consisting of the MRF principal investigators and sponsored researchers within the Discovery Biology program.
- The Drug Discovery Advisory Board, consisting primarily of seasoned pharmaceutical experts, provides oversight to the activities within the Translational Medicine Platform, which includes biomarker research, development of assays, and drug discovery in its Translational Medicine Center and contract research organization (CRO) partners.
- The Clinical Advisory Board, consisting primarily of leading clinicians with clinical trial experience, is focused on clinical development of the drug discovery efforts emerging from the Translational Medicine Platform, with a focus on clinical trials and patient outcomes.

The Discovery Biology team includes the labs of myelin repair experts from Northwestern University, Case Western Reserve University, Stanford University, University of California, San Francisco, and the University of Chicago.

## Financing

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Between 2004 and 2012, MRF raised more than \$50 million from individuals and other foundations. MRF spends approximately 80 percent of its annual budget on research. MRF funds its research activities in a collaborative system where scientific data are shared in real time to speed research discoveries and provides the researchers and their respective institutions with royalty payments if a therapy is commercialized from their efforts.

## Intellectual Property

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Protecting the intellectual property developed within MRF-funded laboratories is critical to insuring that important discoveries advance quickly to patients. Such protection affords more freedom to operate and greater opportunity for industry partnerships to link MRF's brain trust with commercial biotech and pharmaceutical companies interested in developing MRF-based discoveries.

The universities and principal investigators that MRF funds have transferred the licensing rights pertaining to any inventions emerging from this work to MRF. In this agreement, MRF would be the sole executioner of any licensing rights, with the agreement that any royalties would be divided such that MRF would get half to fund future research and the universities would split the remaining half.

## Patent Engagement

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MRF serves patients primarily via developing treatments to improve patients' lives.

MRF measures its impact by the progress it is making in reaching the goal of developing multiple sclerosis drugs:

- More than 100 potential myelin repair treatment drug targets identified
- Many new tools and assays developed, advancing research for potential multiple sclerosis therapeutics.

- Recipient of nine U.S. patents, with 13 additional applications filed
- More than 110 scientific articles and reviews published in peer-reviewed journals
- Sponsored MRF research has led to two clinical trials
- Recognized as one of the top 10 most innovative biotech companies by Fast Company magazine in 2011 (the only nonprofit recognized)
- Two compounds currently tested in Phase I clinical trials

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## Data Sharing

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MRF researchers share data in an online cloud-based system. The MRF research staff oversee the collaboration and data exchange among these scientific experts and their labs. The MRF Translational Medicine Center houses novel biological assays originally developed in the core academic labs and makes the myelination assays available to MRF's academic research consortium members, other academic labs, and biopharmaceutical partners.

## Sponsors & Partners

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Case Western Reserve University

National Institutes of Health/National Institute of  
Neurological Disorders and Stroke

Northwestern University

Stanford University

University of California, San Francisco

University of Chicago

University of Melbourne

MRF also funds studies at a number of contract research organizations (CROs) for critical validation and drug discovery studies.

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Updated: **04/08/2016**