



# Fast Forward<sup>SM</sup>

## General Fund

This project is funded by the National Multiple Sclerosis Society through Fast Forward, LLC, in order to accelerate the commercial development of MS treatments. The Society and Fast Forward connect university-based MS research with private-sector drug development and fund small biotechnology/pharmaceutical companies to develop innovative new MS therapies and repurpose FDA-approved drugs as new treatments for MS.

### *Primary Investigator*

Patrizia Casaccia, MD PhD  
Icahn School of Medicine at Mount Sinai  
New York, NY  
Sharon Shacham, PhD  
Karyopharm Therapeutics,  
Natick, MA

### *Project Title*

In vivo efficacy of lead  
compounds for  
neuroprotection and  
neuroregenerative  
properties in MS

### *Amount to be Committed*

\$500,000  
Term – 14 months

## *About the Company*

Karyopharm Therapeutics Inc. is a clinical-stage pharmaceutical company founded by Drs. Sharon Shacham and Michael Kauffman focused on the new field of nuclear transport modulators. Karyopharm's Selective Inhibitors of Nuclear Export (SINE) function by trapping multiple tumor suppressor proteins in the nucleus. Preliminary evidence of anti-tumor activity across multiple tumor types has been observed by Karyopharm in preclinical studies and Phase 1 clinical trials. The Company is also testing SINEs in autoimmune, viral and dermatologic disorders. Karyopharm is located in Natick, Massachusetts.



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### *Project Background & Goals*

Multiple sclerosis occurs when an immune attack is launched on the brain and spinal cord. Long-term disability occurs when nerve fibers are destroyed. Because the disease-modifying therapies that are currently available for the treatment of MS are only partially effective, there is a clear need for new therapies that directly promote the protection of the nervous system.

Karyopharm is looking at Selective Inhibitors of Nuclear Export (SINE) compounds for development as a treatment for progressive forms of MS. These compounds inhibit release of inflammatory proteins and increase concentrations of neuroprotective factors. Preliminary data in nerve cells isolated in the laboratory and in mice with EAE, an MS-like disease, suggest both an anti-inflammatory and a neuroprotective effect of the SINE molecules. Now they are conducting extensive tests to determine the mechanism of action, possible toxic effects, and to select a leading SINE candidate.

This early-stage study can help to develop a much-needed treatment option to stop MS progression in people with progressive forms of MS.