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## RESEARCH/CLINICAL UPDATE

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### **Major Advancements Made in 2013 Toward Understanding and Treating MS**

The National MS Society continues to pursue all promising paths to uncover solutions for everyone with MS, wherever those opportunities exist, while focusing on the three priority areas of progressive MS, nervous system repair, and wellness/lifestyle. We continue to see the fruits of previous investments, and we are committed to growing our research funding over time.

Significant research progress occurred over the course of 2013, offering new leads that are driving efforts to stop MS in its tracks, restore function, and end MS forever. We now have a better idea of what's causing MS damage and progression, understand more than ever the benefits of early and continuous treatment, and know more about what factors influence the body's brain repair mechanisms.

Here is a brief summary of significant research progress made this year, including links to details. [Go here](#) for a complete list of recent research progress and news.

### **STOPPING MS**

- The oral therapy Tecfidera was [approved](#) for relapsing MS by FDA, and [positive results](#) of peginterferon beta-1a in relapsing MS led to an application to FDA for its marketing approval. Additional studies related to [emerging therapies and other topics](#) were presented at the annual meeting of the American Academy of Neurology.
- Progressive MS:
  - The Society joined with NIH to [launch a therapy trial](#) of the re-purposed therapy ibudilast in primary-progressive and secondary-progressive MS.
  - The global Progressive MS Alliance held its first [scientific meeting](#) to identify challenges and opportunities, and released its first request for research applications to address gaps in knowledge and research tools.
- Lifestyle/Wellness:

- A study suggested that smoking increases a person's [immunity to interferon](#), which could reduce the treatment's benefits.
  - [Dietary salt](#) may stimulate immune activity in MS, according to studies supported by the National MS Society and others. The Society is investing in additional research to follow up this lead.
- Researchers found a possible “biomarker” or indicator that may help predict MS [disease progression](#). It is called Tob1, a molecule associated with immune cells, and if confirmed, it may ultimately be used to identify people who are likely to progress to full-blown MS after an initial attack.
  - There is increasing understanding that malfunctioning mitochondria, the tiny energy producers of cells (like battery packs), may contribute to nervous system damage in MS, opening up possibilities for preventing that damage. This and other reports related to understanding MS damage and its potential repair were reported at the [ECTRIMS conference](#).
  - A report was published from the International Pediatric MS Study Group's [Therapeutic Summit Workshop](#), outlining optimal trial designs for studying disease-modifying therapies in children with MS to improve care.
  - A large meeting of the National MS Society-launched [MS Outcome Assessments Consortium](#) and the U.S. Food and Drug Administration cleared a pathway for a new tool for improving and speeding clinical trials in MS.

#### **Among new efforts and collaborations to drive efforts to stop MS in 2013, the Society:**

- Partnered with the Alzheimer's Drug Discovery Foundation and the Beyond Batten's Disease Foundation to form a collaborative research network of 10 leading universities aimed at screening drugs that show potential for treating brain diseases;
- Provided co-funding to the UK MS Society for an innovative clinical trial testing nerve-protecting therapies in secondary-progressive MS;
- Together with ECTRIMS, sponsored an international workgroup revising clinical classifications of MS to enhance clinical trials and work especially in progressive MS;
- Renewed funding for an international feasibility study (“SUMMIT”) aiming at identifying risk factors that drive MS progression.

#### **RESTORING WHAT'S BEEN LOST**

This year witnessed growing evidence that exercise and rehabilitation can restore physical and mental functions and help people with MS live fuller lives. Progress is also accelerating in the field of nervous system repair, thanks in part to significant previous investments made by the National MS Society in an international [targeted initiative](#). Repairing the myelin insulation on the wire-like axons is thought to be important not only for restoring nerve signaling and function, but also for shielding axons from further harm. [View a recording](#) of a recent Webcast

related to nervous system repair and protection.

- Nervous System Repair:
  - The first Phase 2 trial was launched of “anti-LINGO” investigational therapy aimed at stimulating myelin repair in people with MS.
  - Two mouse studies in the U.S. and Italy showed potential of stem cells derived from the skin for [growing new myelin](#) and for [reducing nervous system damage](#).
  - The first [Barancik Prize winner](#) innovated technology to search on-the-shelf drugs for myelin repair potential; a clinical trial is now starting based on a drug uncovered by this system.
  - Studies from three labs provided basic clues to [nervous system damage](#) and [factors controlling repair](#) of the brain’s insulating myelin, which if confirmed could eventually be translated to promising new therapeutic approaches to stimulating myelin repair to restore function in people with MS.
  - Society-supported researchers used non-invasive [imaging](#) called PET (positron emission tomography) to visualize the loss and repair of myelin in rats over time, which can be used to identify compounds with future potential to treat MS.
- Another step toward improving MS symptoms was taken when Canbex Therapeutics, the recipient of early funding from the Society through Fast Forward, leveraged new funding to [launch a clinical trial](#) of an oral investigational therapy to treat debilitating muscle spasms (spasticity) experienced by many with MS.
- Lifestyle/Wellness:
  - A small study suggested that [aerobic exercise](#) has potential for broad benefits for people with MS including improving memory and building brain circuitry.
  - A clinical trial showed strong evidence that a specific type of [memory training](#) improves learning in people with MS and benefits other aspects of quality of life.
  - Additional studies on exercise and rehabilitation in MS were presented at the [ECTRIMS conference](#)

**To drive additional progress toward restoring what’s been lost in MS, in 2013 the Society also:**

- Initiated funding for a clinical trial to determine whether aerobic exercise can improve cognitive impairment in people with MS;
- Partnered with the University of Miami and Accera, Inc. to test novel “Medical Food” for MS [cognitive impairment](#);
- Invested in commercial therapy development programs through Fast Forward, focusing on novel therapies to protect the nervous system from MS damage and/or stimulate myelin repair. These include CuroNZ’s NRP2945, ENDECE Neural’s NDC-1308, Karo Bio AB’s ERbeta agonists, and Karyopharm Therapeutics Inc.’s Selective Inhibitors of Nuclear Export (SINE) compounds.

## ENDING MS FOREVER

Progress was made identifying some factors that may combine to increase a person's chances of developing MS, such as specific genes, infections, and lifestyle factors. None of these factors is a single cause of the disease, and it's clear that not everyone who has MS has been exposed to these factors, nor that everyone who is exposed to these factors will develop MS. These clues provide insights that may ultimately lead to ways to prevent the disease:

- A global consortium identified [48 new MS risk genes](#), with funding from the National MS Society and others, which better define the biological pathways leading to MS and may ultimately lead to ways to prevent the disease and enhance the design of better treatments.
- A study suggested that the [MS incidence is higher](#) in African-American women than previously thought, and researchers pinpointed [genetic differences between African-Americans](#) and Northern Europeans who have MS.
- Lifestyle/Wellness
  - New studies reported that [eating fatty fish](#) has the potential to reduce the risk of developing MS, and that [smoking increases the risk](#) of developing MS, as does [obesity in girls](#).

### To further efforts to end MS forever, in 2013 the Society:

- Recruited experts to launch the MS Prevalence Work Group, and engaged a consulting group, to begin work that will provide an updated estimate of MS prevalence in the US;
- Launched new research funding totaling \$2.5 million for an expanded [Network of Pediatric MS Centers](#) and data coordination hub, which can be leveraged to answer important research questions to advance our understanding of the disease in both children and adults with MS.

This has been a year of significant research progress, bringing us closer to solutions for everyone with MS. Looking ahead, the Society is committed to fueling research by increasing its annual investment to \$50 million in 2014 – because understanding and ending MS can't come fast enough.