

MS Research Update: Closer to a Cure



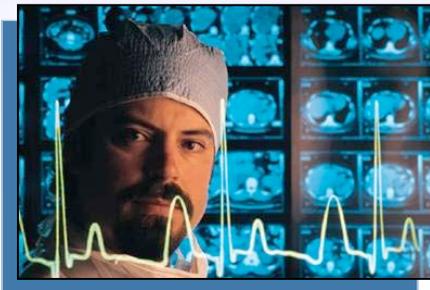
Greater Illinois Chapter

MS Research Update: Closer to a Cure



**Research and the
National MS Society**

**Targeted research
initiatives**



**Promising new
therapies on the
horizon**



Facts About the National MS Society's Research Programs



NMSS is the largest private sponsor of research in the world

NMSS supports research worldwide and trains young scientists

NMSS supports investigator-initiated and targeted research

Research is peer-reviewed to ensure merit and relevance to MS

Greater Illinois Chapter is an Important Center for Research

20 active research studies in IL funded by NMSS representing \$5.7 million

15 of these studies are basic science projects funded by the NMSS and Greater IL Chapter MS Centers

In FY04 the Greater IL Chapter contributed over \$2 million to research

In November of 2004, the Chapter committed **\$2.0 million*** to the National Promise 2010 campaign to support targeted research

*In addition to the millions the Chapter will give in FY05 to support general research

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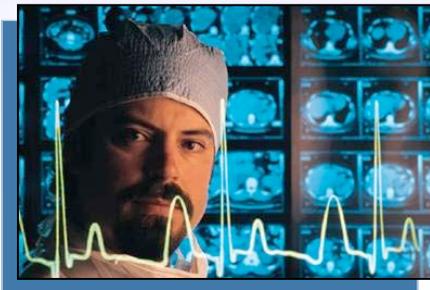


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Promise 2010 Campaign Addresses Some Important Research Areas

- Promise 2010 is a 5-year, \$30 million initiative of the National MS Society
- Targeted research areas include:
 - Pediatric MS
 - Understanding the impact of MS: Sonya Slifka longitudinal study
 - Nervous system repair and protection
 - MS Lesion project



Adults Aren't the Only Ones That Get MS



Until recently, research and treatment of MS focused on adults

- Over **5,000 children** with MS
- More **difficult to diagnose** in children than adults
- Pediatricians are **not familiar** with MS
- Effects of MS drugs in children are **unclear**
- **Few tools** for parents and children with MS

Pediatric neurologists are only now considering MS as a possibility in children



The NMSS Is Committed To Treating The Youngest People With MS

Promise 2010 will establish pediatric MS Centers across the US to:

- Set the standard for pediatric MS **care**
- Serve as centers for **research & information**
- Provide **support** for children and their families



As MS experts devise early treatment protocols for children, they may unlock the mysteries of MS in adults

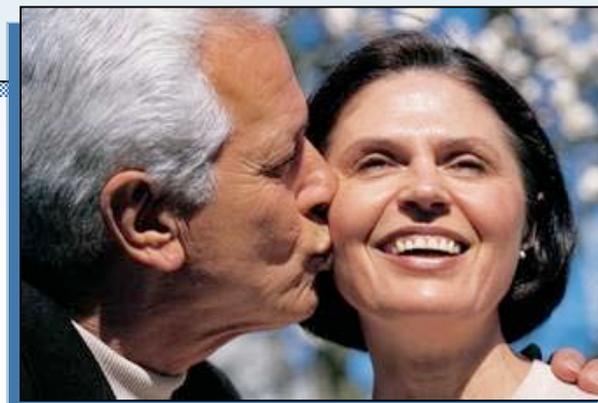
The Sonya Slifka Longitudinal Study Will Help Us Understand MS



Asking questions. Getting answers.

- Do people get the **treatments** they need?
- Why do some people **respond** to a treatment better than others?
- How does MS impact a person's **life**?
- How does MS impact the **family**?
- How do people with MS utilize **healthcare**?

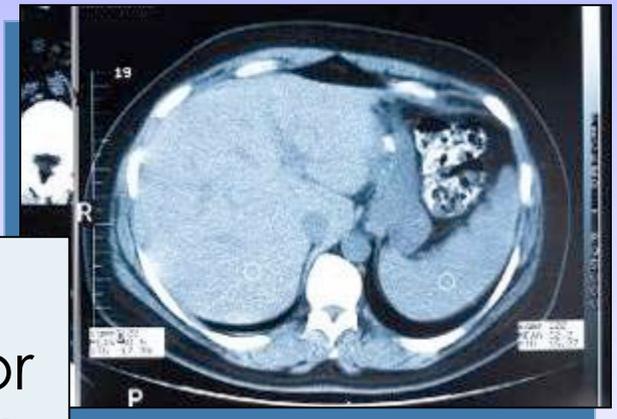
This groundbreaking study is producing vital information about the impact of MS on individuals and families



The MS Lesion Project: Finding Better Ways To Treat MS

Researchers have discovered **four types of lesions** indicating MS may be a family or syndrome of **diseases**

Understanding the what underlies may lead to **different treatments** for people with certain subtypes of MS



MS Lesion Project is the most extensive effort to find patterns of MS damage and correlate to clinical symptoms, MRI scans and responses to therapy

Reversing The Ravages Of MS: Repair & Protection Initiative

Aggressive goals to make tissue repair & protection a reality

- Move research out of test tubes and lab mice and into **human model testing**
- Monitor tissue repair and protection in humans to **determine effectiveness**
- Ensure repaired **tissue is protected** from future MS attacks



Leading researchers believe treatments to protect nerves and rebuild myelin are on the horizon

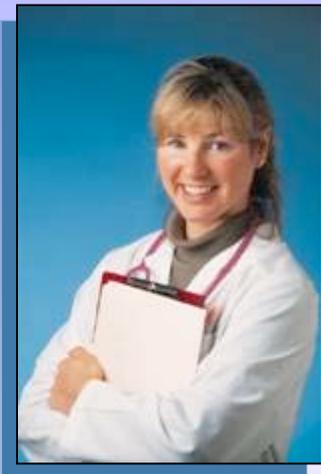
The Stem Cell Story



The National MS Society supports research done in the highest ethical fashion and within the bounds of federal, state & local regulations using all human cell types

- Stem cells are a possible source for **repair or replacement** strategies in MS
- Exploration of stem cell use in MS is in its **infancy** although **encouraging**
- Researchers in Italy found that mice with an MS-like disease **recovered their ability to move** after being injected with adult mouse neural stem cells
- **Humans pose more challenges** than mice, but the Society's scientific advisors are **optimistic**

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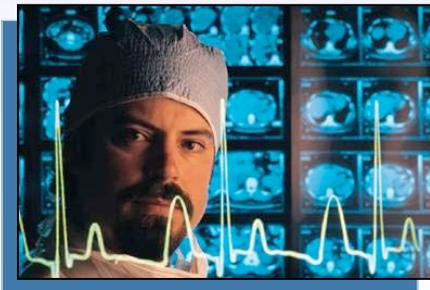


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Promising New Therapies On The Horizon



**Genetic Research
May Yield New
Therapies**

**Estrogen May Help Protect
Against MS Attacks**

**Cholesterol-
Lowering Drugs
May Help In MS**

**Symptom
Management Drugs
Being Studied For MS**

Genetic Research May Bring About Novel Therapies



Lawrence
Steinman, M.D.
Stanford
University

DNA vaccine for MS: Will genetics research lead to an MS vaccine?

- Dr. Steinman and colleagues are using **genetic research** to determine whether a treatment may be possible that would “**retrain**” the **immune system** to stop attacking myelin
- “**Epitope spreading**” is a domino-like attack against myelin that may be related to relapses
- Mice treated with a **customized DNA “cocktail”** designed to get the immune system to tolerate, instead of attack, myelin, had **reduced epitope spreading** and **fewer relapses**

***Learn more about Dr. Steinman's
research on the potential of
customized DNA vaccines at the***

***2005 Fannie & Charles Penikoff
Research Symposium***

May 14, 2005 at these locations:

Hyatt in Chicago

Clocktower Hotel in Rockford

Holiday Inn in Bloomington/Normal



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Early Study Finds Cholesterol Drug May Reduce Lesions



Currently, there is no evidence that those statins at the doses used to lower cholesterol are of any value in the treatment of MS

- In a small study, **Simvastatin (Zocor®)** appeared to **reduce the number** and volume of “gadolinium enhancing” MRI lesions
- Previous studies have shown **statin drugs may be beneficial** in treating MS
- The mechanism of statin drugs appears to be **immune system modulation**, not cholesterol-lowering
- Larger **clinical trials are on the horizon** to necessary to determine effectiveness

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Estrogen May Protect Against Immune System Attacks

Many women with MS who become pregnant experience a “holiday” from symptoms, particularly during 2nd and 3rd trimesters



Rhonda
Voskuhl, M.D.
UCLA

- Rhonda Voskuhl, MD, has shown that **estrogen** was **beneficial in mice** with an MS-like disease
- Early indication that estrogen may be of **benefit in women** with MS
- **Clinical trials** are investigating the effects of estrogen in women with MS
- Additional research is determining how estrogen alters **immune responses**

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Donepezil (Aricept®) May Improve Memory In People With MS



Clinical trials are underway to test long-term effectiveness of cognitive enhancers

- Cognitive changes occur often in MS, commonly with memory
- In a small study, Dr. Lauren Krupp and colleagues at Stony Brook reported that patients on donepezil had better word recall than those on placebo
- Donepezil is used to improve memory in Alzheimer's disease
- A larger study is underway to test the safety and benefits of donepezil in people with MS

New Drug Shows Promise In Treating Pseudobulbar Affect



Early results indicate that the drug reduces the number and severity of episodes of pseudobulbar affect

- Pseudobulbar affect refers to **uncontrollable laughing** and/or **crying**
- Pseudobulbar affect affects an estimated **10% of people with MS**
- Clinical trials are investigating the **safety and effectiveness** of a new drug (Neurodex®) in people with MS
- If approved, would be the **first treatment** for pseudobulbar affect

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Update



Tysabri Update

- On Monday, February 28, Biogen Idec and Elan voluntarily suspended the marketing of TYSABRI
- The 2 adverse events that led to this action were reported in patients who were taking TYSABRI in combination with AVONEX
- These events involved a rare neurological disorder called progressive multifocal leukoencephalopathy (PML)

PML Overview

- PML is a rare, progressive disease of the brain
- PML mostly affects people who have a weakened immune system
- It is caused by activation of a virus called “JC virus”
- This virus is present in an inactive form in most healthy adults. The factors leading to activation are not fully understood.
- PML is not contagious

PML Diagnosis

- PML is a very rare disease and can be difficult to diagnose
- There is no known cure for PML
- Biogen Idec and Elan will inform your healthcare professional of new information and developments
- If you have questions regarding PML, you should contact your healthcare professional

Update Continued

- To date, there have been no reported cases of PML in patients receiving TYSABRI monotherapy
- It is unknown if there is a relationship between use of TYSABRI and the development of these specific adverse events
- At this time, all dosing with TYSABRI in clinical trials has been suspended
- There are more than 650,000 patient years of experience on AVONEX and no reported cases of PML

Next Steps

- There are currently 5 treatment options for people with MS that have all been proven safe and effective
- Work with your healthcare professional to find the treatment option that best fits your situation
- Biogen Idec and Elan are continuing to work with clinical investigators, experts, and regulatory authorities
- For additional information:
 - Visit FDA website at www.fda.gov
 - See NMSS website at www.nmss.org for additional information and a learn online webcast with transcripts
 - Visit www.tysabri.com or www.biogenidec.com for additional updates

Promising New Therapies On The Horizon

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Estrogen May Help Protect

**What Else Is On The
Horizon?**

MS

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What Else Is On The Horizon?

Oral disease-modifying drugs



Focus on wellness



Rehabilitation strategies



Combination therapies



Where Can You Go For More Information About Research?



www.nmss.org

**Information Resource Center
1-800-FIGHT MS**

**Look For Research
Highlights in MS
Connection & InsideMS**



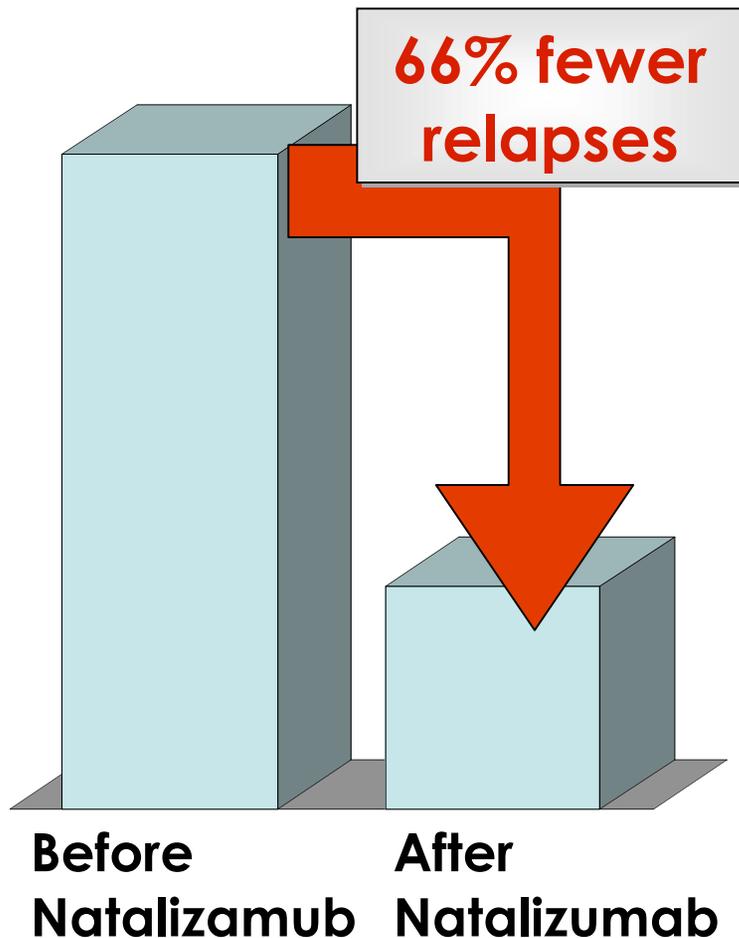
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Additional Information

- Local research studies
 - Cluster study
 - Aging & MS
- Speaker's slides
- Research updates added, as necessary

Natalizumab Significantly Reduced Relapse Rates In People With MS

Relapse Rate



Results from Clinical Trial Are Encouraging

After one year, those taking natalizumab had **66% fewer relapses** than before taking natalizumab

Natalizumab (Tysabri®) Approved To Treat Relapsing Forms Of MS



- **FDA approval** based on first year data of a 2-year clinical trial of the monoclonal antibody natalizumab
- 942 people with **relapsing-remitting MS**
 - 99 sites worldwide
 - Randomized
 - Placebo-controlled
 - Double-blind

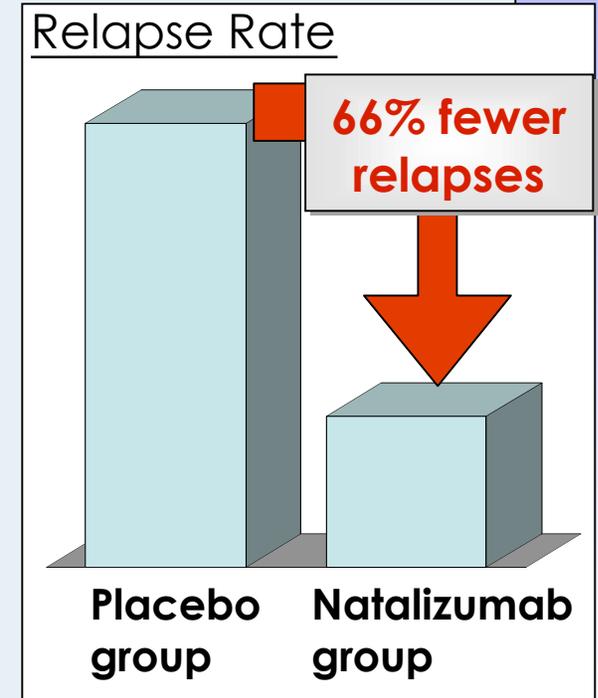
Natalizumab Works Differently Than Other Disease-Modifying Drugs

- Monoclonal **antibody** with known mechanism of action
- **Blocks** potentially **damaging immune cells** into brain and spinal cord
 - Attaches to alpha-4-integrin on immune T cells
 - Prevents T cells from passing blood-brain barrier
- Given by **monthly infusion** in a doctor's office
- Side effects may include headache, fatigue, urinary tract infection, depression, lower respiratory tract infection, joint pain and abdominal discomfort



Natalizumab Appears To Be A Promising New Therapy For MS

- Patients on natalizumab had **66% fewer relapses** than before the study
- **76%** of those on natalizumab were **relapse-free** vs. 53% of placebo group
- **60%** of those on natalizumab had **no new MRI lesions** or no newly enlarging lesions (patches of disease activity) compared to 22% of placebo
- **96%** of patients on natalizumab had **no gadolinium-enhancing lesions** (lesions that show active inflammation) vs. 68% of the placebo group



Natalizumab Was Also Tested In Combination With An Interferon

- Patients on natalizumab plus (interferon beta-1a (Avonex®)) had a 54% **additional reduction in clinical relapses** than those taking placebo plus Avonex
- 96% of those on both drugs had **no gadolinium-enhancing lesions** vs. 76% of those on placebo plus interferon
- 67% of those on both drugs were **relapse-free** vs. 53% of placebo group
- 67% receiving both drugs developed **no new or newly enlarging lesions** compared to 40% in the interferon-only group

The combination of natalizumab plus interferon beta-1a reduced clinical relapses and lesions even more than when interferon beta-1a was given alone

Next Steps and Unanswered Questions About Natalizumab

- Who is most likely to benefit from natalizumab?
- How does natalizumab compare to other disease-modifying drugs in terms of benefits or safety?
- Should natalizumab be given in combination with another therapy?
- How much will it cost? Will it be covered by health insurers?
- Results of 2 year data will provide more information about effectiveness & safety
- Talk to your physician about whether or not natalizumab is right for you