

OPEXA THERAPEUTICS, INC.

FORM 8-K (Current report filing)

Filed 10/02/12 for the Period Ending 10/02/12

Address	2635 TECHNOLOGY FOREST BLVD. THE WOODLANDS, TX 77381
Telephone	(281) 272-9331
CIK	0001069308
Symbol	OPXA
SIC Code	2834 - Pharmaceutical Preparations
Industry	Biotechnology & Drugs
Sector	Healthcare
Fiscal Year	12/31

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
Washington, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the
Securities Exchange Act of 1934

Date of Report (date of earliest event reported): **October 2, 2012**

OPEXA THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Texas
(State or other jurisdiction of
incorporation)

001-33004
(Commission File Number)

76-0333165
(IRS Employer Identification No.)

2635 Technology Forest Blvd., The Woodlands, Texas
(Address of principal executive offices)

77381
(Zip Code)

Registrant's telephone number, including area code: **(281) 272-9331**
N/A

(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- ☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
 - ☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
 - ☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
 - ☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))
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Item 7.01 Regulation FD Disclosure

On October 2, 2012, Opexa Therapeutics, Inc. (the “**Company**”) will host an analyst briefing to be held in New York, New York, using slides containing the information attached to this Current Report on Form 8-K as Exhibit 99.1. The presentation includes an overview of the Company’s ongoing Abili-T™ clinical study for Tcelna™, the Company’s lead therapy, in subjects with secondary multiple sclerosis (SPMS), as well as an SPMS disease overview by Mark Freedman, M.D., director of the Multiple Sclerosis Research Unit at the Ottawa Hospital, member of Opexa's Scientific Advisory Board, and an investigator for Opexa’s Abili-T study. The attached materials will be made available on the Investor Relations page of the Company’s website at www.opexatherapeutics.com. The Company does not undertake to update this presentation.

Item 9.01 Financial Statements and Exhibits

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
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99.1	Opexa Therapeutics, Inc. Abili-T Analyst Briefing to be held in New York, New York on October 2, 2012.
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The information in Item 7.01 of this Current Report on Form 8-K, including Exhibit 99.1, shall not be deemed “filed” for the purposes of Section 18 of the Securities Exchange Act of 1934 or otherwise subject to the liabilities under that Section, nor be deemed to be incorporated by reference into the filings of the registrant under the Securities Act of 1933.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Dated: October 2, 2012

OPEXA THERAPEUTICS, INC.

By: /s/ Neil K. Warma

Neil K. Warma

President & Chief Executive Officer

Exhibit Index

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OPEXA THERAPEUTICS



A REVOLUTION IN
CELL THERAPY

Abili-T™ Analyst Briefing

Opexa Therapeutics, Inc.

October 2, 2012
New York, NY



Forward-Looking Statements

This presentation contains forward-looking statements which are made pursuant to the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The forward-looking statements in this presentation do not constitute guarantees of future performance. Investors are cautioned that statements in this presentation which are not strictly historical statements, including, without limitation, statements regarding the Company's clinical development plans for Tcelna™, constitute forward-looking statements. Such forward-looking statements are subject to a number of risks and uncertainties that could cause actual results to differ materially from those anticipated, including, without limitation, risks associated with the Company's capital position, the rights and preferences provided to the Series A Convertible Preferred Stock and investors in the convertible secured notes issued by the Company in July 2012 (including a secured interest in all of our assets), the ability of the Company to enter into and benefit from a partnering arrangement for the Company's product candidate, Tcelna™, on reasonably satisfactory terms (if at all), our dependence (if partnered) on the resources and abilities of any partner for the further development of Tcelna™, our ability to compete with larger, better financed pharmaceutical and biotechnology companies, new approaches to the treatment of our targeted diseases, our expectation of incurring continued losses, our uncertainty of developing a marketable product, our ability to raise additional capital to continue our development programs, including to undertake and complete any ongoing or further clinical studies for Tcelna™, the success of our clinical trials, the efficacy of Tcelna™ for any particular indication, such as for relapsing remitting MS or secondary progressive MS, our ability to develop and commercialize products, our ability to obtain required regulatory approvals, our compliance with all Food and Drug Administration regulations, our ability to obtain, maintain and protect intellectual property rights (including for Tcelna™), the risk of litigation regarding our intellectual property rights or the rights of third parties, the success of third party development and commercialization efforts with respect to products covered by intellectual property rights that we may license or transfer, our limited manufacturing capabilities, our dependence on third-party manufacturers, our ability to hire and retain skilled personnel, our volatile stock price, and other risks detailed in our filings with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this presentation. We assume no obligation or undertaking to update or revise any forward-looking statements contained herein to reflect any changes in our expectations with regard thereto or any change in events, conditions or circumstances on which any such statement is based. You should, however, review additional disclosures we make in our Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, and Current Reports on Form 8-K filed with the SEC.



Abili-T Study

- Opexa initiates study in Secondary Progressive MS (SPMS) patients
- First ever personalized T-cell therapy for MS patients
- Fast Track designation for SPMS
- Success in SPMS could also have positive impact on development of treatment for Relapsing Remitting MS (RRMS)



Tcelna Highlights

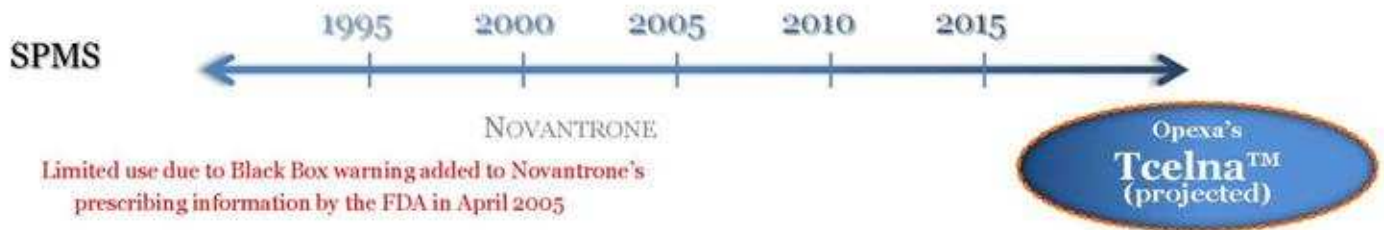
- 5 clinical studies completed (in Relapsing Remitting and Secondary Progressive MS patients)
 - 302 patients total
 - 142 treated with Tcelna in Opexa-sponsored research
- Over 850 Tcelna preparations have been successfully manufactured
 - reproducible and consistent
 - commercially viable process
- Safety demonstrated and clinical efficacy promising across broad spectrum of patients for relapsing and progressive MS
- Multiple commercial opportunities:
 - Secondary Progressive MS
 - Promising efficacy shown in slowing disease progression and reducing relapse rates significantly differentiates Tcelna
 - Fast track designation
 - Relapsing Remitting MS
 - FDA concurrence for Phase 3 studies
 - Clinical benefit shown with reduction of relapse rates and improvement in disability
 - Mechanism supports combination therapy
- Dose and regimen have been confirmed as effective and practical



Opexa's Focus: Filling the Void in SPMS Treatment

Over 1 million people affected by SPMS worldwide...

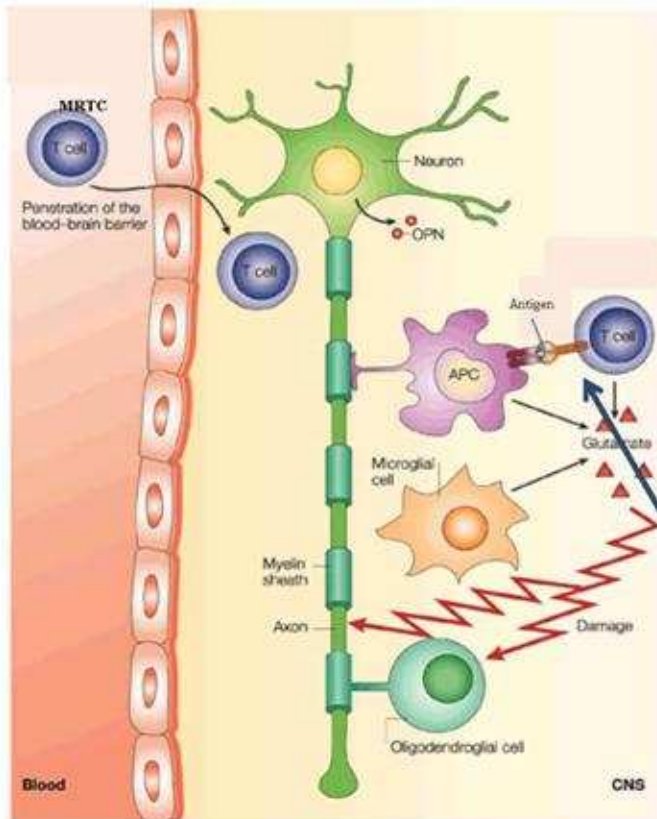
...only ONE FDA approved product for SPMS



Treating SPMS with a
Safe and **Effective** Therapy



Role of Myelin-Reactive T-cells in Multiple Sclerosis



Nature Reviews | Immunology

- MRTCs cross the Blood Brain Barrier (BBB) into the central nervous system (CNS) from the periphery and respond to antigen presenting cells (APC) bearing myelin antigens
- Macrophages and microglial cells release OPN, IL-12/23, accompanied by T-cell dependent secretion of IFN- γ , IL-17 and tumor-necrosis factor alpha (TNF α) in addition to direct T-cell mediated cytotoxicity

Result $\rightarrow$ *Destruction of the myelin sheath*

- T-cells that clonally accumulate in the lesion are driven by defined protein antigens

Opexa's Strategy

Identify which *specific* antigens within each *individual* patient may be targeted by the immune response



Production of Tcelna: A two-step process

The Epitope Profiling Assay (EPA)

- Screen peripheral blood for Myelin-Reactive T-cells (MRTCs), and mapping of immunodominant epitopes to MBP, MOG and PLP
 - 109 overlapping peptides encompassing MBP, MOG and PLP
 - Interferon gamma response to individual peptide pools defines positive response in 7 day assay

ImmPath™ Process

- Procure unit of blood from which up to six T-cell lines reactive with immunodominant myelin peptides are generated and pooled as a patient-specific **Tcelna** product
 - Manufacturing performed under GMP/GTPs in functionally closed system
 - Process generates a year of **Tcelna** doses from a single unit of blood



Proprietary Assay Enables Annual Personalized Treatments

Year 1

Conduct analysis of 109 peptides from all three key myelin proteins (MBP, MOG, PLP)

Patient XX	Peptide 1	Peptide 2	Peptide 3	Peptide 4	Peptide 5	Peptide 6	Peptide 7	Peptide 8	Peptide 9	Peptide 10	Peptide 11	Peptide 104	Peptide 105	Peptide 106	Peptide 107	Peptide 108	Peptide 109
MBP																	
MOG																	
PLP																	

Year 2

Patient XX	Peptide 1	Peptide 2	Peptide 3	Peptide 4	Peptide 5	Peptide 6	Peptide 7	Peptide 8	Peptide 9	Peptide 10	Peptide 11	Peptide 104	Peptide 105	Peptide 106	Peptide 107	Peptide 108	Peptide 109
MBP																	
MOG																	
PLP																	

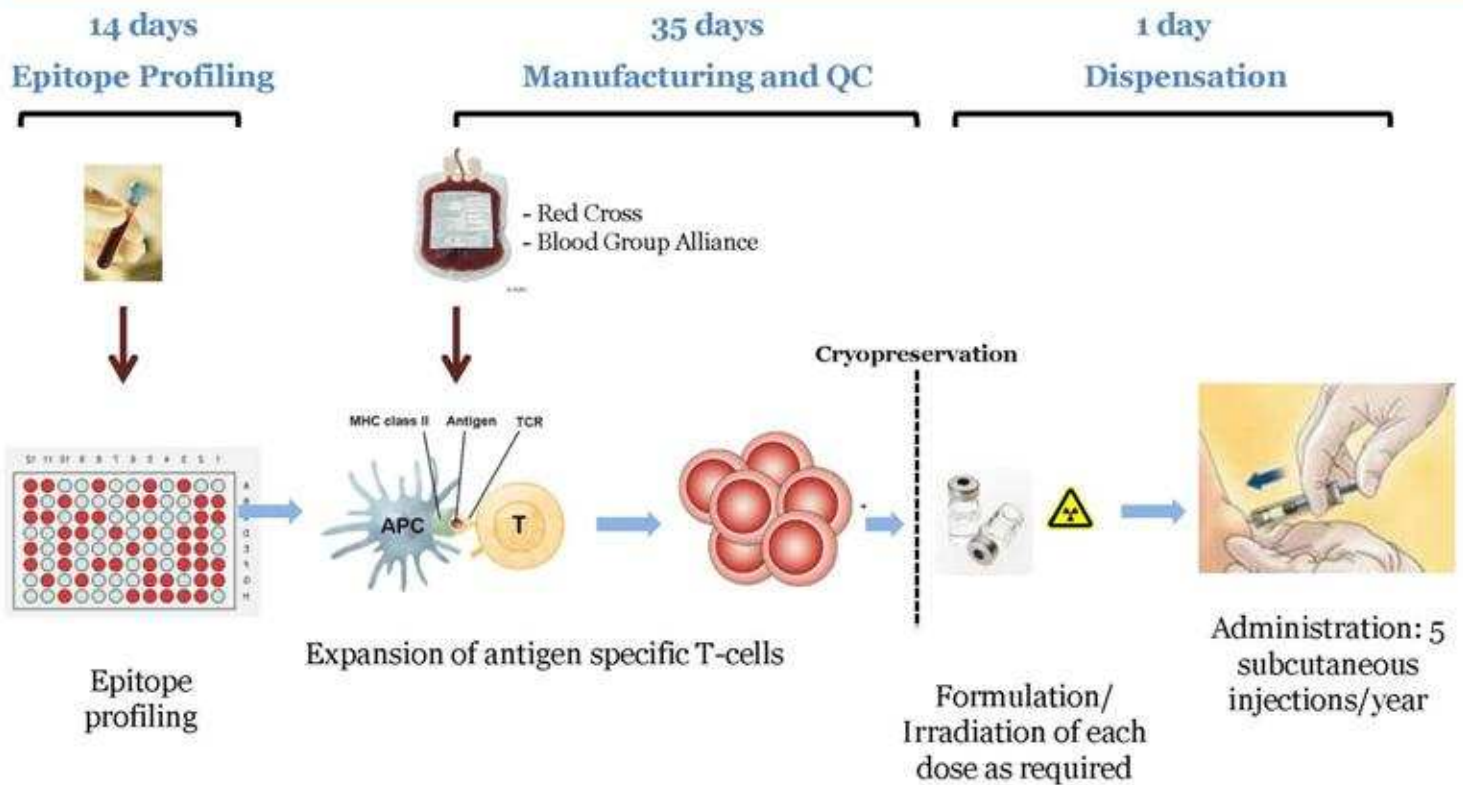
Re-assess epitope profile annually to identify epitope drift

Develop new formulation based on evolved epitope profile

Year 3

Patient XX	Peptide 1	Peptide 2	Peptide 3	Peptide 4	Peptide 5	Peptide 6	Peptide 7	Peptide 8	Peptide 9	Peptide 10	Peptide 11	Peptide 104	Peptide 105	Peptide 106	Peptide 107	Peptide 108	Peptide 109
MBP																	
MOG																	
PLP																	

Tcelna Manufacturing





Opexa Management Team

- **Neil Warma, President & CEO, Director**
 - 19+ years international healthcare experience with large and emerging biotechnology companies
 - 9 years of Senior Management with Novartis Pharmaceuticals
 - Formerly, President & CEO and Member of the Board of Directors of Viron Therapeutics, a private clinical stage biopharmaceutical company developing a novel class of protein therapeutics
 - Co-founder and President of MedExact Inc., a medical Internet company, which was successfully sold
 - Neuroscience and MBA degrees
- **Jaye Thompson, Ph.D., SVP, Clinical Development & Regulatory Affairs**
 - President and founder of SYNERGOS, Inc., a full service contract research organization which was successfully sold to inVentiv Health in 2006
 - Directed and managed statistical analysis, data management, report writing and the conduct of clinical trials for a wide variety of indications including MS
 - PhD, Biostatistics
- **Don Healey, Ph.D., VP, Scientific Development and Manufacturing**
 - Over 25 years experience in cellular immunology and immune regulation in academic and biotech environments
 - Former Director of Immunology for Argos Therapeutics, responsible for the development of novel autologous dendritic cell therapies for the treatment of renal carcinoma and HIV
 - Post-doctoral positions in the Department of Pathology, Cambridge University, UK, conducting studies on immunoregulation in animal models of autoimmunity, including Type I Diabetes and Multiple Sclerosis
- **Donna Rill, SVP Operations and Quality Systems**
 - 30 years of extensive clinical and research laboratory experience in cell and gene therapy research and clinical application, immunological techniques and assessments
 - Designed, and validated cGMP Cell & Gene Therapy Laboratories, cGMP Vector Production facilities, and Translational Research Labs
 - Actively involved in the development of cell based therapies as well as the formulation and review of related regulatory guidelines for over 20 years
- **David Jorden, (Acting) Chief Financial Officer, Director**
 - 20+ years of public and private company investment experience; Morgan Stanley PWM, Genometrix, Inc., CFO, principal at Faye Sarofim & Co.
 - Received a MBA from Kellogg School of Management at Northwestern University and a BBA from the University of Texas
 - Chartered Financial Analyst and Certified Public Accountant designations



Scientific Advisory Board

➤ **Dawn McGuire, M.D., FAAN (Chair)**

- Advisory Council of the Gill Heart Institute
- American Academy of Neurology
- National Institute of Neurological Disorders and Stroke of the National Institutes of Health

➤ **Doug Arnold, M.D.**

- James McGill Professor Neurology and Neurosurgery at the Montreal Neurological Institute of McGill University

➤ **Edward Fox, M.D., Ph.D.**

- Director of Multiple Sclerosis Clinic of Central Texas
- Advisory Committee, Lone Star Chapter of the National Multiple Sclerosis Society
- Consortium of Multiple Sclerosis Centers
- Clinical Assistant Professor of Neurology, University of Texas Medical Branch

➤ **Hans-Peter Hartung, M.D.**

- Chair of Neurology at Heinrich-Heine University
- PresidentECTRIMS
- European Neurological Society
- International Society for Neuroimmunology
- International Federation of Multiple Sclerosis Societies
- World Health Organization Advisory Board on Multiple Sclerosis



Scientific Advisory Board

➤ **Clyde Markowitz, M.D.**

- Director of the Multiple Sclerosis Center at the University of Pennsylvania
- Professor of Neurology at the University of Pennsylvania School of Medicine in Philadelphia
- Chairman of the Clinical Advisory Committee for the Delaware Valley National MS Society
- American Academy of Neurology

➤ **Paul O'Connor, M.D., FRCP**

- MS Clinic Director at St. Michael's Hospital, University of Toronto
- National Scientific and Clinical Advisor for the Multiple Sclerosis Society of Canada

➤ **Mark S. Freedman, M.D., FRCP, FAAN**

- Director of the Multiple Sclerosis Research Unit at the Ottawa Hospital
- Professor of Medicine (Neurology), University of Ottawa
- Senior Scientist, the Ottawa Hospital Research Institute
 - Secretary/Treasurer - Americas Committee for Treatment and Research in MS
 - Clinical Trials Committee, National MS Society (USA)
 - Education Committee, the Consortium of MS Centres



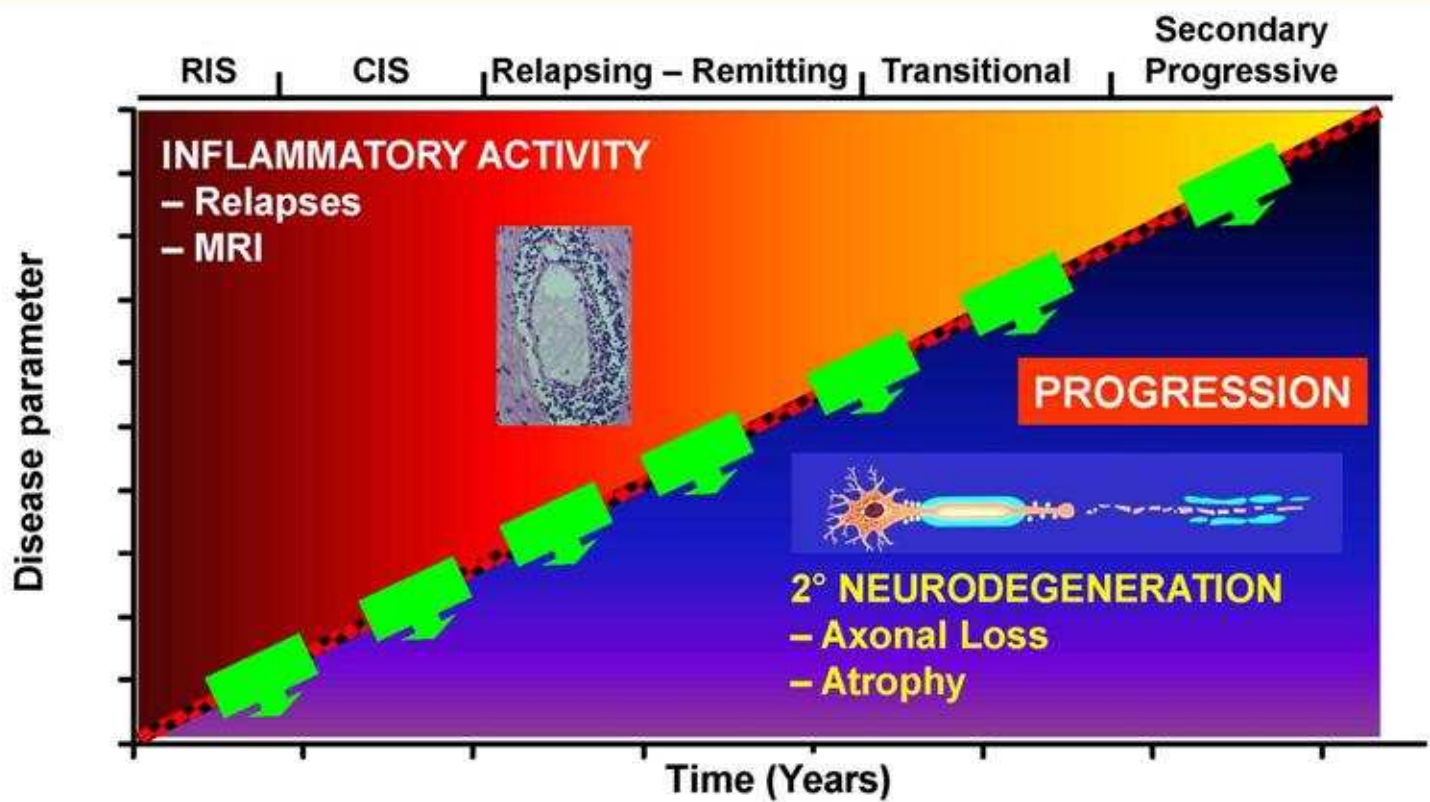
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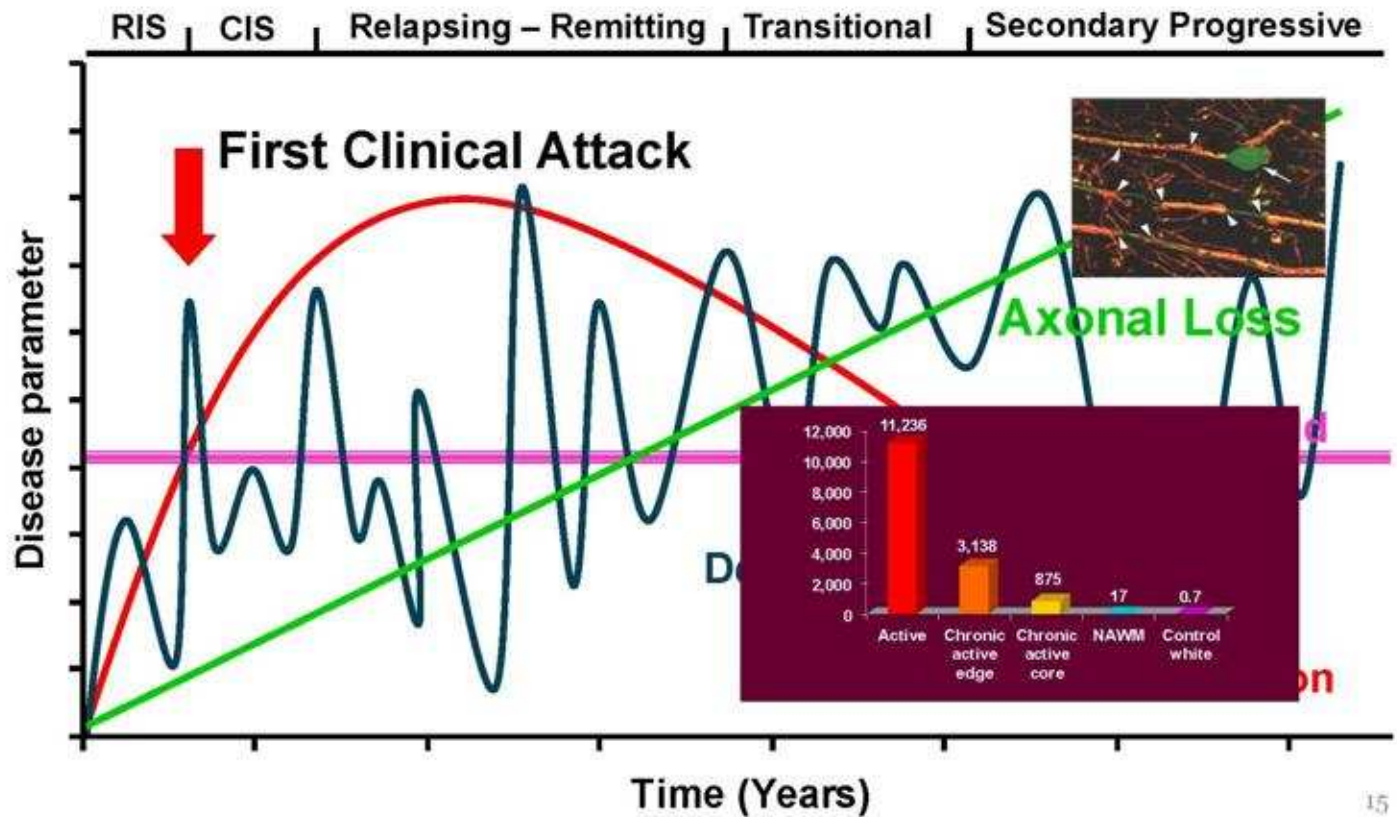
Mark Freedman, MD

Pathological vs. Clinical Course of Disease





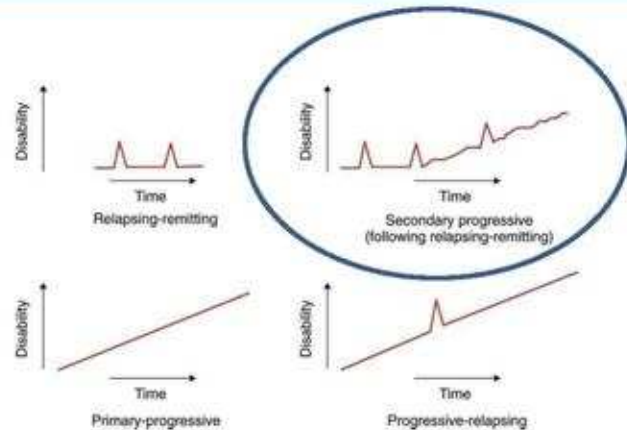
Pathological vs. Clinical Course of Disease





Secondary Progressive MS

- Patients initially experience a relapsing-remitting course then transition to SPMS
- SPMS patients experience worsening disability with or without relapses
- Within 10 years of diagnosis, approximately 50% of patients progress to SPMS
- Estimated that 85% of patients will eventually transition to SPMS
- Approximately 30-45% of MS patients can be classified as SPMS





SPMS Standard of Care

- **Only two drugs approved specifically for SPMS**
 - Novantrone (mitoxantrone) approved only in the US
 - Limited usefulness due to product's safety profile
 - Black box warning: cardio toxicity
 - Betaseron (interferon beta-1b) approved only in Canada
- **These drugs may be effective for patients who still have relapsing forms of progressive disease, however, once patients are in pure progression stage, neither is effective**
- **Symptomatic therapy may be prescribed**

The Immunopathology of SPMS: Relevance to Tcelna

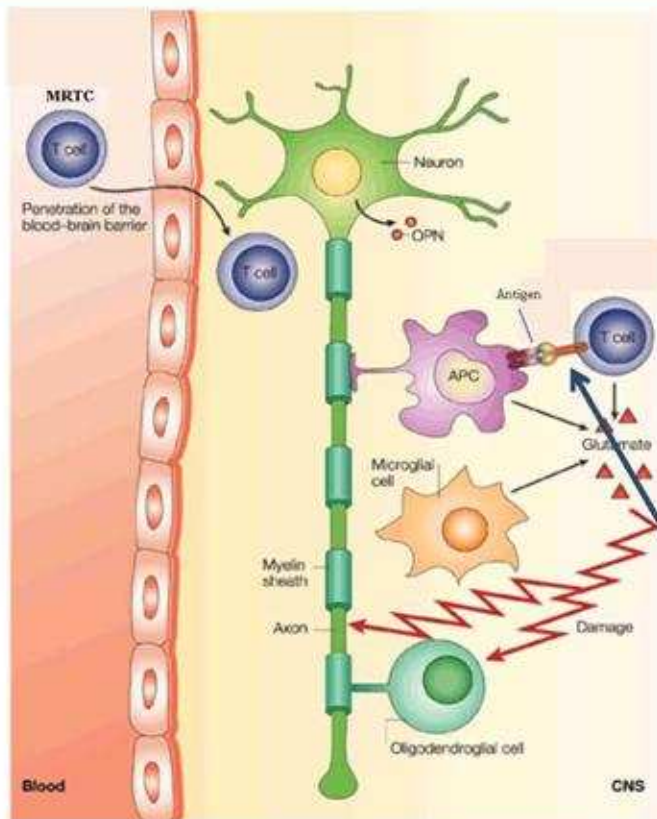


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- Compartmentalized CNS inflammatory cells
 - Persistence of meningeal mononuclear cell infiltrates associated in lymphoid follicular structures
 - Microglia activation suggests ongoing chronic innate immune responses
- Meningeal follicles close to cortical gray matter lesions suggests that adaptive immune responses involving antibody and complement contribute to progression in SPMS
- Chronic myelin-reactive cells may be contributing to the development of both innate and adaptive immune responses persisting in the CNS
- **Tcelna immunotherapy in SPMS may reduce the drivers of this chronic disease-driving immunity through both peripheral and central regulatory responses**



Role of Myelin-Reactive T-cells in Multiple Sclerosis



Nature Reviews | Immunology

- MRTCs cross the Blood Brain Barrier (BBB) into the central nervous system (CNS) from the periphery and respond to antigen presenting cells (APC) bearing myelin antigens
- Macrophages and microglial cells release OPN, IL-12/23, accompanied by T-cell dependent secretion of IFN- γ , IL-17 and tumor-necrosis factor alpha (TNF α) in addition to direct T-cell mediated cytotoxicity

Result $\rightarrow$ *Destruction of the myelin sheath*

- T-cells that clonally accumulate in the lesion are driven by defined protein antigens

Opexa's Strategy

Identify which *specific* antigens within each *individual* patient may be targeted by the immune response



Proposed Mechanism

- **Attenuated, patient-specific (autologous) myelin reactive T-cells (MRTC)**
 - MRTC expanded *ex vivo* in response to immunodominant peptides of MBP, MOG & PLP
- **Therapeutic sc dosing ($30-45 \times 10^6$ cells) stimulates host reactivity to the 'over-represented' MRTC inducing a dominant negative 'regulatory T-cell' response leading to:**
 - Down-regulation of similar endogenous disease-causing myelin reactive T-cells
 - Up-regulation of regulatory cells (Foxp3+ and Tr1 cells) to reduce inflammation and provide possible neuroprotection, should these gain entry to the CNS



Safety

Summary of Safety from Phase IIb (n=150)

Safety Summary	
Deaths	• No deaths occurred
Serious Adverse Events (SAEs)	• No treatment-related SAEs
Most common adverse events	• Mild to moderate injection site reaction only



SPMS Phase IIb Trial Design (Abili-T)

- **Double-blind**
- **Placebo-controlled, 1:1 randomized**
- **180 Patients**
 - SPMS population
 - Approximately 30 sites in USA and Canada
- **2 annual courses of Tcelna**
- **2 years of assessment**



Opexa Therapeutics, Inc.
2012-00 Abili-T Study
Investigator Sites



STUDY SITES:

#001 – Stanley Cohan, MD, PhD – Portland, OR
#002 – George Hutton, MD – Houston, TX
#003 – William Sheremata, MD – Miami, FL
#004 – Lily Jung-Henson – Seattle, WA
#005 – Cary Twyman, MD – Lexington, KY
#006 – Mark Cascione, MD – Tampa, FL
#007 – Ben Thrower, MD – Atlanta, GA
#008 – Jeffrey Gitt, DO – Phoenix, AZ
#009 – Daniel B Wynn, MD – Northbrook, IL
#010 – Suzanne Gazda, MD – San Antonio, TX
#011 – Clyde Markowitz, MD – Philadelphia, PA
#012 – Patricia Coyle, MD – Stony Brook, NY
#013 – Craig Herrman, MD – Indianapolis, IN
#014 – Sharon Lynch, MD – Kansas City, KS
#015 – Stephen Newman, MD – Plainview, NY
#016 – Joanna Cooper, MD – Berkeley, CA
#017 – Daniel Jacobs, MD – Orlando, FL

#018 – Angela Applebee, MD – Burlington, VT
#019 – Walter Carlini, MD – Medford, OR
#020 – Lawrence P Goldstick, MD – Dayton, OH
#021 – Matthew Baker, MD – Naples, FL
#022 – Joshua Katz, MD – Brighton, MA
#023 – Jill Conway, MD – Charlotte, NC
#024 – Edward J Fox, MD, PhD – Round Rock, TX
#025 – Kermit Lloyd, MD – Newport News, VA
#026 – S. James Shafer, MD – Vero Beach, FL
#027 – Jeanette Wendt – Tucson, AZ

#101 – Mark Freedman, MD – Ottawa, Ontario
#102 – Amit Bar-Or, MD – Montreal, Quebec
#103 – Felix Veloso, MD – Regina, SK
#104 – Anthony Traboulee, MD – Vancouver, BC



Target Population

- **Subjects with EDSS between 3.0 and 6.0**
- **Subjects must have evidence of progression in the absence of relapse in the last two years**
- **Presence of Myelin Reactive T-cells**



Efficacy Assessments

- **Primary Endpoint**
 - Whole-brain atrophy
- **Secondary Endpoints**
 - Sustained progression measured by EDSS
 - Time to sustained progression
 - T2 lesions progressing to hypointense lesions (black holes)
 - Change in EDSS
 - Annualized Relapse Rate (ARR)
 - Change in MSFC Assessment of disability
 - Change in Symbol Digit Modality Test (SDMT)
- **Exploratory Endpoints**
 - Quality of life assessment by MSQLI
 - Gd-enhancing lesion volume with increasing MTR
 - Gd-enhancing lesion volume with decreasing MTR
 - Change in MTR in normal-appearing white matter
 - Changes in T-regulatory cell repertoire and function



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Questions and Answers