

# OPEXA THERAPEUTICS, INC.

## **FORM 8-K** (Current report filing)

Filed 08/14/14 for the Period Ending 08/14/14

|             |                                                         |
|-------------|---------------------------------------------------------|
| Address     | 2635 TECHNOLOGY FOREST BLVD.<br>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): August 14, 2014**

---

**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))
- 
-

**Item 2.02 Results of Operations and Financial Condition**

On August 14, 2014, Opexa Therapeutics, Inc. (the “**Company**”) filed its Quarterly Report on Form 10-Q for the quarter ended June 30, 2014 and announced its results of operations in a press release. A copy of the press release announcing the results is furnished as Exhibit 99.1 to this Current Report on Form 8-K and is incorporated herein by reference.

On the same day, members of the Company’s management will hold a teleconference to discuss the financial results. Interested individuals desiring to participate in the teleconference may dial in approximately ten minutes before the scheduled 5:00 P.M. ET call to (253) 237-1170 or toll free at (877) 372-0867. Please reference conference ID # 81938581 or the Opexa Therapeutics Earnings Call when dialing into the call. A live webcast of the call can also be accessed via the webcast link on the Company’s website ([www.opexatherapeutics.com](http://www.opexatherapeutics.com)).

**Item 7.01. Regulation FD Disclosure.**

A copy of the Company’s updated Investor Presentation that will be available on the Investor Relations page of the Company’s website ([www.opexatherapeutics.com](http://www.opexatherapeutics.com)) is furnished as Exhibit 99.2 to this Current Report on Form 8-K and is incorporated herein by reference. 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>                                                  |
|--------------------|---------------------------------------------------------------------|
| 99.1               | Press Release issued by Opexa Therapeutics, Inc. on August 14, 2014 |
| 99.2               | Opexa Therapeutics, Inc. Investor Presentation, August 2014         |

The information in Items 2.02 and 7.01 of this Current Report on Form 8-K, including Exhibits 99.1 and 99.2, 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: August 14, 2014

OPEXA THERAPEUTICS, INC.

By: /s/ Neil K. Warma

Neil K. Warma

President and Chief Executive Officer

---

**EXHIBIT INDEX**

| <b><u>Exhibit No.</u></b> | <b><u>Description</u></b>                                           |
|---------------------------|---------------------------------------------------------------------|
| 99.1                      | Press Release issued by Opexa Therapeutics, Inc. on August 14, 2014 |
| 99.2                      | Opexa Therapeutics, Inc. Investor Presentation, August 2014         |

## Opexa Therapeutics Reports Second Quarter 2014 Financial Results and Provides Corporate Update

THE WOODLANDS, Texas--(BUSINESS WIRE)--August 14, 2014--Opexa Therapeutics, Inc. (NASDAQ:OPXA), a biotechnology company developing Tcelna<sup>®</sup> (imilecleucel-T), a novel T-cell immunotherapy for the treatment of Multiple Sclerosis (MS), today reported financial results for the second quarter ended June 30, 2014 and provided an overview of the Company's recent corporate developments.

Recent highlights include:

- **Clinical**
  - Opexa reached the 180-patient targeted enrollment in the Phase IIb "Abili-T" clinical study of Tcelna (imilecleucel-T) in patients with Secondary Progressive Multiple Sclerosis as of May 13, 2014 and subsequently closed enrollment with a total of 190 patients randomized. The Abili-T clinical trial is a randomized, double-blind, placebo-controlled study at approximately 35 leading clinical sites in the U.S. and Canada. The Company expects to report top-line results during the second half of 2016.
- **Financial**
  - Reported cash and cash equivalents of \$16,214,690 as of June 30, 2014.
  - Based on the current clinical activities for the Abili-T trial, Opexa believes it has sufficient capital to support its current clinical activities for the Abili-T trial and general operations of the Company into the fourth quarter of 2015.

"We are pleased to close the first half of 2014 in a strong position to continue developing Tcelna for the treatment of patients with Secondary Progressive Multiple Sclerosis," said Neil K. Warma, Opexa's President and Chief Executive Officer. "Completion of enrollment in the Phase IIb Abili-T trial during this past quarter represents an important milestone in the development of our personalized immunotherapy for the Secondary Progressive MS community. We remain committed to our goal in developing Tcelna for this patient population."

As of June 30, 2014, Opexa's cash and cash equivalents totaled approximately \$16,214,690 and the Company's monthly burn rate for the six months ending June 2014 was approximately \$1.2 million. Based on the current clinical activities for the Abili-T trial, Opexa believes it has sufficient liquidity to support its current clinical activities for the Abili-T trial and general operations to sustain itself into the fourth quarter of 2015.

---

## Second Quarter Financial Results

Opexa reported revenue of \$307,686 for the three months ended June 30, 2014, compared to \$348,837 for the three months ended June 30, 2013. The revenue is related to the recognized portion of the \$5 million upfront payment received from Merck Serono in conjunction with the Option and License Agreement entered into between Opexa and Merck Serono during February 2013.

Research and development expenses were \$3,409,210 for the three months ended June 30, 2014, compared with \$2,223,030 for the three months ended June 30, 2013. The increase in expenses is primarily due to the increasing enrollment of patients for the ongoing clinical trial of Tcelna in SPMS, an increase in the procurement and use of supplies for product manufacturing and development, and increases in the number of employees to support the ongoing clinical trial, employee compensation expense and stock compensation expense.

General and administrative expenses for the three months ended June 30, 2014 were \$967,367, compared with \$750,605 for the three months ended June 30, 2013. The increase in expense is due to increases in employee compensation and stock compensation expenses, and was partially offset by decreases in legal and professional fees related to financing activities.

Depreciation and amortization expenses for the three months ended June 30, 2014 were \$98,658, compared with \$88,898 for the three months ended June 30, 2013. The increase in expense is due to increases in depreciation for laboratory, manufacturing and computer equipment acquired during 2013 and 2014 and leasehold improvements during 2013 and 2014 to support increased development activities.

Opexa reported a net loss for the three months ended June 30, 2014 of \$4,163,259, or \$0.15 loss per share (basic and diluted), compared with a net loss of \$2,996,430 or \$0.37 loss per share (basic and diluted) for the three months ended June 30, 2013. The increased net loss is primarily related to a decrease in revenue and an increase in research and development expenses, higher general and administrative expenses and higher depreciation expense for the quarter ending June 30, 2014.

Cash and cash equivalents were \$16,214,690 as of June 30, 2014, compared to \$5,028,280 as of June 30, 2013.

For additional information please see Opexa's Quarterly Report on Form 10-Q filed today with the SEC.

## Conference Call and Webcast Details

Opexa will conduct a conference call and webcast to provide a corporate update and discuss the financial results at 5:00 p.m. Eastern Time today. To participate in the conference call, dial in approximately ten minutes before the scheduled 5:00 p.m. time to (253) 237-1170 or toll free at (877) 372-0867. Please reference conference ID 81938581 or the Opexa Therapeutics Earnings Call.

---

A live webcast of the call can also be accessed here or on the Investor Relations page of Opexa's website ( [www.opexatherapeutics.com](http://www.opexatherapeutics.com) ).

## **About Opexa**

Opexa's mission is to lead the field of Precision Immunotherapy™ by aligning the interests of patients, employees and shareholders. The Company's leading therapy candidate, Tcelna®, is a personalized T-cell immunotherapy that is in a Phase IIb clinical development program (the Abili-T trial) for the treatment of Secondary Progressive MS. Tcelna is derived from T-cells isolated from the patient's peripheral blood, expanded ex vivo, and reintroduced into the patients via subcutaneous injections. This process triggers a potent immune response against specific subsets of autoreactive T-cells known to attack myelin.

## **About Multiple Sclerosis (MS)**

Multiple Sclerosis is a chronic, inflammatory condition of the central nervous system and is the most common, non-traumatic, disabling neurological disease in young adults. It is estimated that approximately two million people have MS worldwide.

While symptoms can vary, the most common symptoms of MS include blurred vision, numbness or tingling in the limbs and problems with strength and coordination. The relapsing forms of MS are the most common. The Secondary Progressive form of MS represents about a third of the MS patient population.

## **About Tcelna**

Tcelna® is a potential personalized therapy that is under development to be specifically tailored to each patient's disease profile. Tcelna is manufactured using ImmPath®, Opexa's proprietary method for the production of a patient-specific T-cell immunotherapy, which encompasses the collection of blood from the MS patient, isolation of peripheral blood mononuclear cells, generation of an autologous pool of myelin-reactive T-cells (MRTCs) raised against selected peptides from myelin basic protein (MBP), myelin oligodendrocyte glycoprotein (MOG) and proteolipid protein (PLP), and the return of these expanded, irradiated T-cells back to the patient. These attenuated T-cells are reintroduced into the patient via subcutaneous injection to trigger a therapeutic immune system response.

Opexa is currently conducting a Phase IIb study of Tcelna. Named "Abili-T," the trial is a randomized, double-blind, placebo-controlled clinical study in patients who demonstrate evidence of disease progression with or without associated relapses. The trial is being conducted at approximately 35 leading clinical sites in the U.S. and Canada with each patient receiving two annual courses of Tcelna treatment consisting of five subcutaneous injections per year. The trial's primary efficacy outcome is the percentage of brain volume change (atrophy) at 24 months. Study investigators will also measure several important secondary outcomes commonly associated with MS, including disease progression as measured by the Expanded Disability Status Scale (EDSS), annualized relapse rate and changes in disability as measured by EDSS and the MS Functional Composite.

---

For more information visit the Opexa Therapeutics website at [www.opexatherapeutics.com](http://www.opexatherapeutics.com).

**Cautionary Statement Relating to Forward-Looking Information for the Purpose of "Safe Harbor" Provisions of the Private Securities Litigation Reform Act of 1995**

This earnings release 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. Statements contained in this release, other than statements of historical fact, constitute "forward-looking statements." The words "expects," "believes," "potential," "possibly," "estimates," "may," "could" and "intends," as well as similar expressions, are intended to identify forward-looking statements. These forward-looking statements do not constitute guarantees of future performance. Investors are cautioned that statements which are not strictly historical statements, including, without limitation, statements regarding plans and objectives for product development (including for Tcelna (imilecleucel T)), 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. These risks and uncertainties include, without limitation, risks associated with the following: market conditions; our capital position; our ability to compete with larger, better financed pharmaceutical and biotechnology companies; new approaches to the treatment of our targeted diseases such as MS; 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 or clinical studies related to our T-cell platform); our ability to maintain compliance with NASDAQ listing standards; the success of our clinical trials (including the Phase IIb trial for Tcelna in Secondary Progressive MS which, depending upon results, may determine whether Ares Trading SA (Merck), a wholly owned subsidiary of Merck Serono S.A., elects to exercise its option (Option) to acquire an exclusive, worldwide (excluding Japan) license of our Tcelna program for the treatment of MS); whether Merck exercises its Option and, if so, whether we receive any development or commercialization milestone payments or royalties from Merck pursuant to the Option; our dependence (if Merck exercises its Option) on the resources and abilities of Merck for the further development of Tcelna; 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 and future pipeline candidates); 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 suppliers and 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 made. We assume no obligation or undertaking to update or revise any forward-looking statements contained herein to reflect any changes in its 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 Reports on Form 10-K, Quarterly Reports on Form 10-Q and Current Reports on Form 8-K filed with the SEC.

---

OPEXA THERAPEUTICS, INC.

Statement of Operations Data:

|                                                         | Three Months<br>Ended June 30, |                       | Six Months<br>Ended June 30, |                       |
|---------------------------------------------------------|--------------------------------|-----------------------|------------------------------|-----------------------|
|                                                         | 2014                           | 2013                  | 2014                         | 2013                  |
| Revenue:                                                |                                |                       |                              |                       |
| Option revenue                                          | \$ 307,686                     | \$ 348,837            | \$ 656,523                   | \$ 568,937            |
| Research and development                                | 3,409,210                      | 2,223,030             | 6,220,349                    | 3,844,396             |
| General and administrative                              | 967,367                        | 750,605               | 2,070,247                    | 1,853,040             |
| Depreciation and amortization                           | 98,658                         | 88,898                | 194,244                      | 167,209               |
| Operating loss                                          | (4,167,549)                    | (2,713,696)           | (7,828,317)                  | (5,295,708)           |
| Interest income                                         | 4,290                          | 3,066                 | 9,484                        | 4,940                 |
| Other income, net                                       | -                              | -                     | -                            | 37,910                |
| Interest expense                                        | -                              | (285,800)             | -                            | (1,921,054)           |
| Net loss                                                | <u>\$ (4,163,259)</u>          | <u>\$ (2,996,430)</u> | <u>\$ (7,818,833)</u>        | <u>\$ (7,173,912)</u> |
| Basic and diluted loss per share                        | \$ (0.15)                      | \$ (0.37)             | \$ (0.28)                    | \$ (0.94)             |
| Weighted average shares outstanding - Basic and diluted | 27,661,675                     | 7,991,559             | 27,623,358                   | 7,617,409             |

Selected Balance Sheet Data:

|                            | June 30,<br>2014<br>(unaudited) | December 31,<br>2013 |
|----------------------------|---------------------------------|----------------------|
| Cash and cash equivalents  | \$ 16,214,690                   | \$ 23,644,542        |
| Other current assets       | 1,481,526                       | 1,122,576            |
| Fixed assets, net          | 1,234,826                       | 1,295,024            |
| Other long term assets     | 162,045                         | 177,666              |
| Total assets               | 19,093,087                      | 26,239,808           |
| Total current liabilities  | 3,693,587                       | 3,324,493            |
| Deferred revenue           | 1,846,120                       | 2,338,041            |
| Total stockholders' equity | 13,553,380                      | 20,577,274           |

CONTACT:

**Company Contact:**

Opexa Therapeutics, Inc.  
Karthik Radhakrishnan, 281-775-0600  
Chief Financial Officer

or

**Investor Relations:**

The Trout Group  
Adam Cutler, 646-378-2936  
[opexa@troutgroup.com](mailto:opexa@troutgroup.com)



OPEXA THERAPEUTICS



*Precision Immunotherapy™*

# Opexa Therapeutics, Inc.

NASDAQ: OPXA

August 2014  
The Woodlands, TX

# Forward-Looking Statements

---

- This investor 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. Statements contained in this presentation, other than statements of historical fact, constitute “forward-looking statements.” The words “expects,” “believes,” “anticipates,” “estimates,” “may,” “could,” “intends,” and similar expressions are intended to identify forward-looking statements. Forward-looking statements do not constitute guarantees of future performance. Investors are cautioned that statements which are not strictly historical statements, including, without limitation, statements regarding the development of our product candidate, Tcelna (imilecleucel-T), 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. These risks and uncertainties include, but are not limited to, risks associated with: market conditions; our capital position; 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); our ability to satisfy various conditions required to access the financing potentially available under the purchase agreements with Lincoln Park Capital Fund, LLC (“Lincoln Park”) or sell shares of our common stock to Lincoln Park or under our at-the-market (ATM) facility; our ability to maintain compliance with NASDAQ listing standards; the success of our clinical trials (including the Phase IIb trial for Tcelna in Secondary Progressive Multiple Sclerosis which, depending upon results, may determine whether Ares Trading SA (“Merck”) elects to exercise its option for an exclusive license to Tcelna for the treatment of multiple sclerosis (the “Option”)); whether Merck exercises the Option and, if so, whether we receive any development or commercialization milestone payments or royalties from Merck pursuant to the Option; our dependence (if Merck exercises the Option) on the resources and abilities of Merck for the further development of Tcelna; the efficacy of Tcelna for any particular indication; our ability to develop and commercialize products; our ability to obtain required regulatory approvals; our compliance with all FDA regulations; our ability to obtain, maintain and protect intellectual property rights; 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 SEC.
- These forward-looking statements speak only as of the date made. We assume no obligation or undertaking to update any forward-looking statements to reflect any changes in 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 Reports on Form 10-K, Quarterly Reports on Form 10-Q, and Current Reports on Form 8-K filed with the SEC.

# Introduction to Opexa

---

- **Antigen specific T-cell immunotherapy platform**
  - Potential to address multiple therapeutic areas
  - Lower capital intensity by leveraging existing GMP manufacturing
  - Strong Patent Estate: 50 patents issued on T-cell platform (domestic and international)
- **Lead Program: Tcelna<sup>®</sup>, in development for Multiple Sclerosis**
  - Phase IIb clinical trial in Secondary Progressive MS (SPMS)
  - Limited treatment options currently available for SPMS
  - Potential SPMS market in North America alone could exceed \$7 Billion
  - Fast Track designation from the U. S. Food and Drug Administration (FDA) for the treatment of SPMS
  - Option Agreement with Merck Serono, a strong potential commercial partner

# Progress Update

---

- **Clinical**

- As of June 30, 2014, Opexa had completed enrollment and randomized 190 patients in the Abili-T study in SPMS
- Top line data expected in 2H 2016

- **Financial**

- As of June 30, 2014, Opexa had \$16.2 million in cash and cash equivalents
- Projected liquidity into Q4 2015, based on current operations and Abili-T clinical activities

# Tcelna in Secondary Progressive MS

---

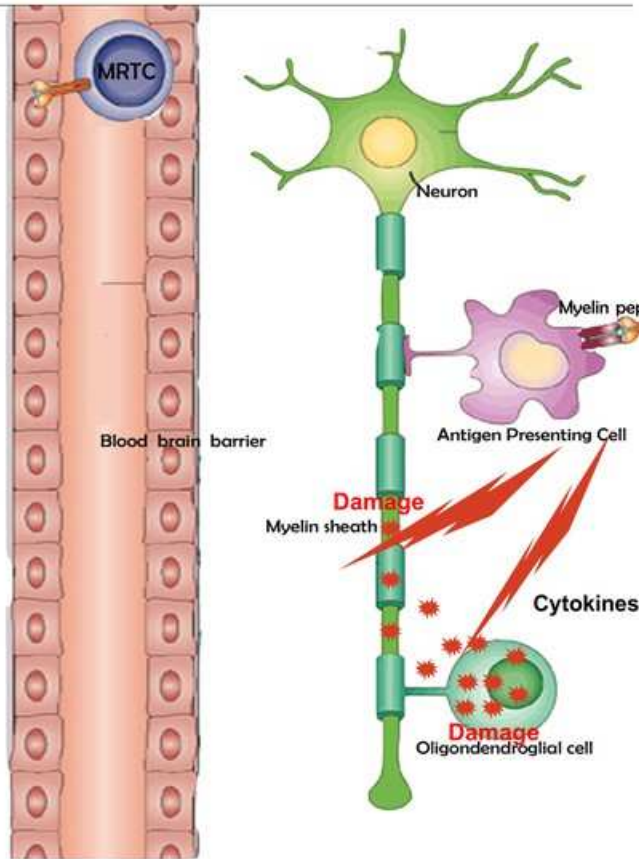
**450,000 Individuals in North America diagnosed with MS;  
30-45 % potentially have an SPMS diagnosis**



- Potential SPMS market in North America alone could exceed **\$7 billion**
- Roughly 150,000 SPMS patients in North America
- Only one drug approved for SPMS in U.S. (none in EU or Asia)
  - Drug not suitable for chronic use due to severe side effects

**Tcelna being developed to be a potential therapy of choice in SPMS**

# The Root Cause of Multiple Sclerosis: Activated T- cells Degrade Myelin and Damage Myelin Producing Cells

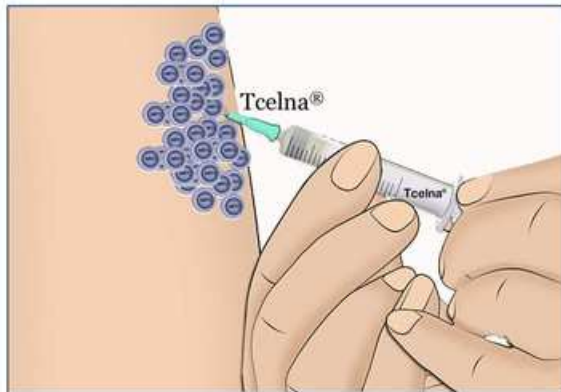


- In MS patients, the faulty immune system is not able to prevent the attack of a small sub-population of myelin reactive T-cells (MRTC)
- Myelin Reactive T-cells (MRTC) cross the blood brain barrier, enter the brain, and bind to antigen presenting cells (APC), causing a release of pro-inflammatory cytokines which lead to a two pronged attack through:
  1. Destruction of myelin sheath, the protective coating of nerve fibers
  2. Destruction of oligodendroglial cells, which are responsible for producing myelin

**Result → Destruction of the myelin sheath and myelin producing cells, thereby preventing remyelination**

# Tcelna<sup>®</sup> Consists of Attenuated Antigen Specific T-cell Clones

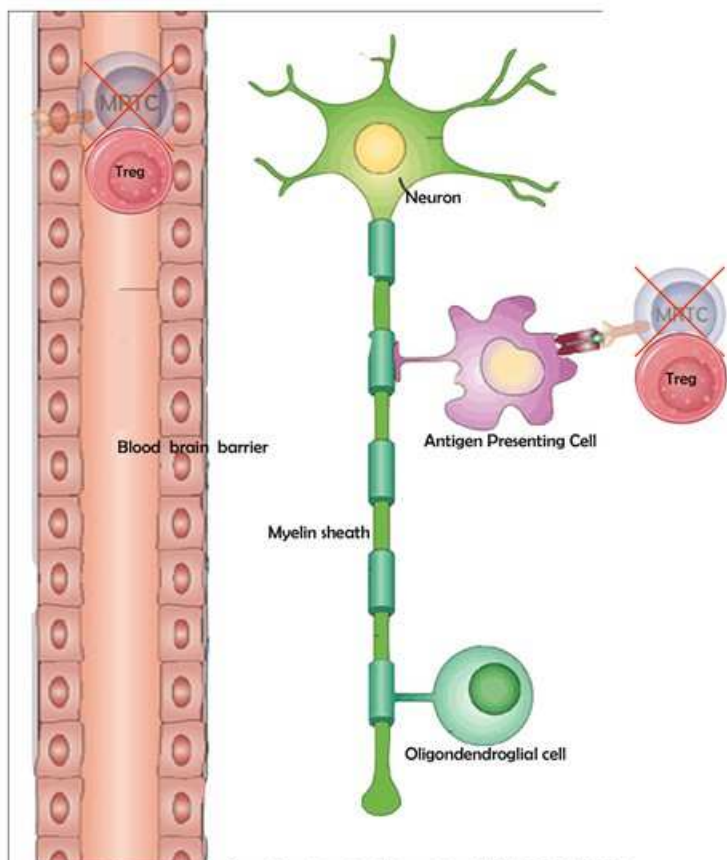
---



Tcelna: attenuated antigen specific T-cells

- Therapeutic dose of Tcelna (attenuated T-cell clones) is injected subcutaneously
- Immune system recognizes the large volume of cells (30-45 million) under the skin as potential foreign pathogens
- We believe Tcelna triggers an immune response specifically targeting circulating MRTC
- The irradiation of Tcelna prior to injection causes the injected cells to eventually die off, but only after they have primed the immune response

## Tcelna Could Address the Root Cause of Multiple Sclerosis by Preventing Demyelination and Enabling Remyelination



Adapted by permission from Macmillan Publishers Ltd: NATURE REVIEWS IMMUNOLOGY 3, 483-492 (June 2003), copyright (2003)

- Immune cells, including Tregs, have been primed, or sensitized, we believe, to specifically target the pathogenic MRTC for elimination or regulation
- Elimination of harmful MRTC may lead to:
  - Stabilization of disease by preventing further destruction of myelin
  - Improvement in condition by allowing Oligodendroglial cells to remyelinate axons (neuroprotection)

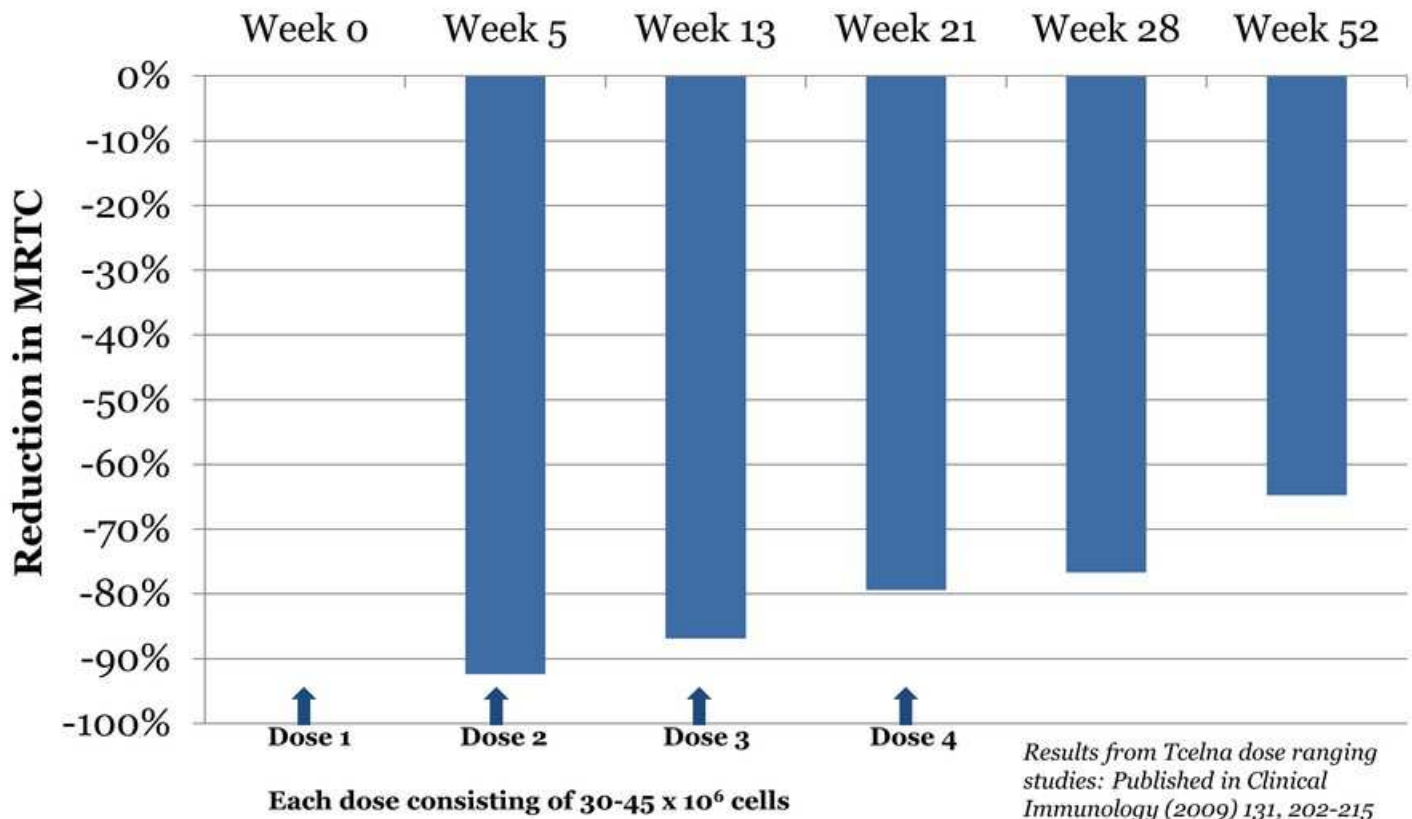
### Opexa's Strategy

Tcelna programs the immune system to **specifically** recognize MRTC as pathogenic, thereby inhibiting further destruction of the myelin sheath and potentially enabling remyelination

# Mechanism of Action

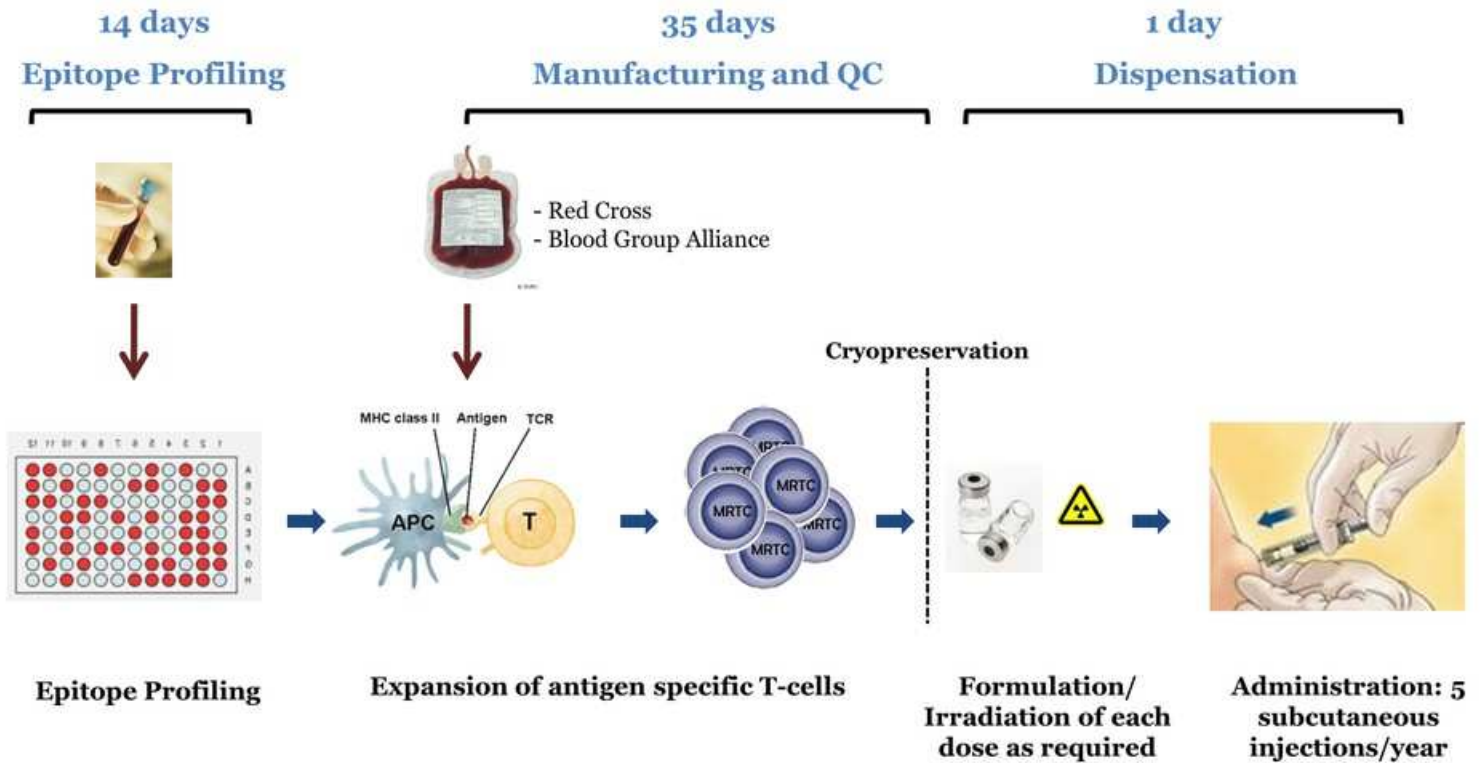
## Reduction in Myelin Reactive T-Cells

**Tcelna Demonstrated a Reduction in Myelin Reactive T-cells (MRTC)**



# Single Cycle Manufacturing Process:

## Annual course of treatment (5 doses) from one blood draw



# Merck Serono Agreement for MS indication

---

- **February 2013 option and license agreement with Merck Serono**
  - Up to \$220 million in additional payments
    - Option exercise \$25 million for starting Phase III/ or \$15 million if another Phase II
    - \$35 million FDA filing, approval and commercialized in US
    - \$30 million for EU filing, approval and commercialization in at least three countries
    - Relapsing Remitting MS development and commercialization of up to \$40 million
  - One time commercial milestones of up to \$85 million
- **Royalties ranging from 8% to 15% of annual net sales with step-ups occurring when net sales exceed \$500 million, \$1 B & \$2 B**
- **Opexa maintains key rights:**
  - Development and commercialization rights to Tcelna in Japan
  - Certain manufacturing rights
  - Co-development funding option in exchange for increased royalties
  - Rights to all other disease indications

# Tcelna Development Program

## Broad Spectrum of MS patients treated

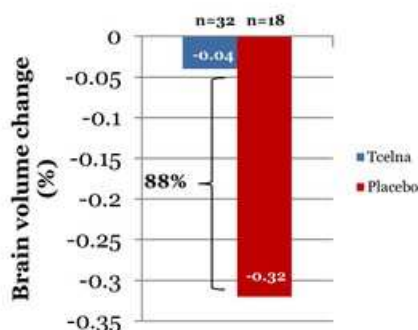
| Phase                             | Completion Dates | Population            | Total N | Treatment Duration (months)  | Tcelna              | Placebo |
|-----------------------------------|------------------|-----------------------|---------|------------------------------|---------------------|---------|
| Baylor                            | 1998             | RRMS, PPMS, SPMS (26) | 114     | Up to 24 months              | 114                 |         |
| Phase I dose escalation           | 2006             | RRMS (5)<br>SPMS (6)  | 16      | 12                           | 16                  | -       |
| Phase I/II Open label retreatment | 2007             | RRMS (9)<br>SPMS (4)  | 13      | 12                           | 13                  | -       |
| Phase IIb TERMS                   | 2008             | RRMS, CIS             | 150     | 12                           | 100                 | 50      |
| Phase IIb extension OLTERMS       | 2008             | RRMS, CIS             | 38      | At least one dose post TERMS | 15 from placebo arm |         |
| Phase IIb Abili-T                 | 2H 2016*         | SPMS                  | 180*    | 24*                          | 90*                 | 90*     |

\* Expected upon completion of ongoing SPMS Abili-T trial

# TERMS Study - Prospective Analysis in More Active or Progressive Patients

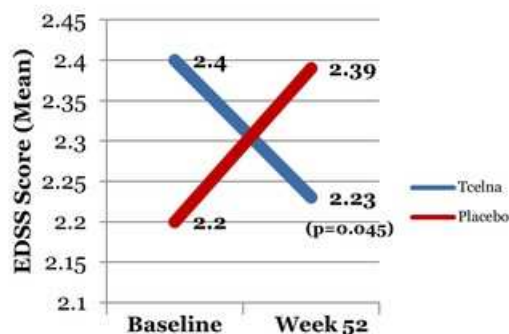
Sub-population of patients (n=50) with more progressed/active disease profile (baseline ARR >1)

Percent Brain Volume Change at Week 52



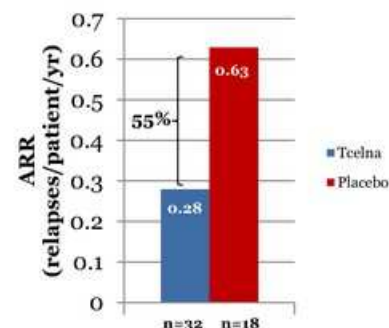
88% Reduction in Brain Atrophy

Change in Disability (EDSS) at Week 52



Statistically Significant Improvement in Disability (p=0.045)

Annualized Relapse Rate (ARR) at Week 52

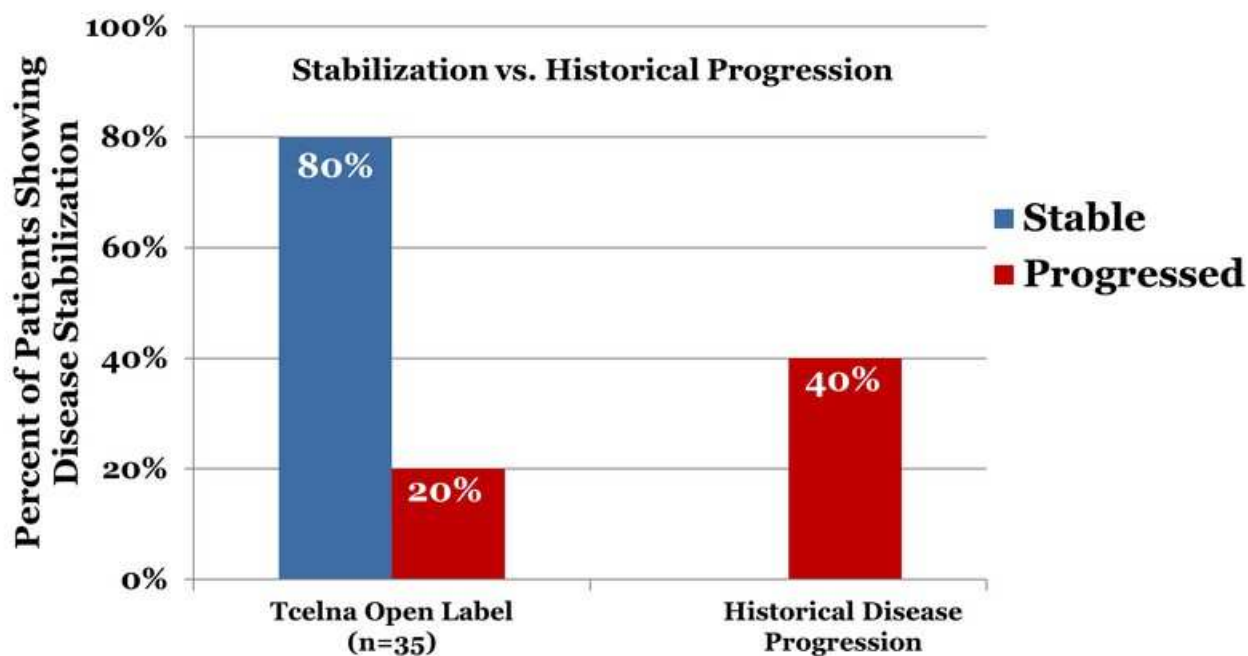


55% Reduction in ARR

These Data Support Phase IIb Program in SPMS

# Tcelna Stabilizes Disease in SPMS at 2 Years

**80% of subjects treated with Tcelna showed no further disease progression by EDSS at 2 years**



\*A small percentage of patients in pooled analysis showed an improvement (i.e. decrease in progression)

\*\*Historical control: ESIMS Study, published *Hommes Lancet* 2004

# Abili-T : Phase IIb Clinical Trial

---

- **Enrollment completed Q2 2014**
  - 190 patients randomized
- **FDA Fast Track Designation**
- **Secondary Progressive MS patient population**
  - EDSS range 3-6; 18-60 years old
- **Design**
  - Double-blind, 1:1 randomized, placebo-controlled
  - Approximately 35 clinical sites in USA and Canada
  - Two annual courses of personalized therapy
- **Top-line data expected second half of 2016**

# 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



# Immune Monitoring Program

Trial initiation      40 patients at dose 5      90 patients at dose 5      180 patients at 10 doses      End of Study

**2012****2013****2014****2015****2016**

## Immuno vigilance

Whole blood analysis:  
Absolute frequency of T-cells, B-cells, Dendritic cells and Monocytes

- Goal is to show no systemic impact on immune response, i.e. Tcelna is a Precision Immunotherapy
- Tcelna differentiates by not depleting a broad spectrum of T-cells

## Phenotypical Analysis (CD4 and CD8)

Pro-inflammatory      Anti-inflammatory

|                |             |
|----------------|-------------|
| TH1            | Treg        |
| TH17           | Tr1         |
|                | TH2         |
| IL-2           | IL-4        |
| IL-6           | IL-5        |
| IL-12          | IL-10       |
| IL-17E, IL-17F |             |
| IL-23          | IL-27       |
| TNF $\alpha$   | TGF $\beta$ |
| IFN $\gamma$   | BDNF        |

## Plausible indicators of Tcelna efficacy

- Reduction in Th1/TH17
- Increase in Treg/Tr1 cells
- Reduction in proinflammatory cytokines (IL-12, IL-23, IFN $\gamma$ , TNF $\alpha$ )
- Increase in anti-inflammatory cytokines (IL-10, IL-27)
- Loss of proinflammatory monocyte markers (CD16/HLA-DR, CCR5)
- Gain in anti-inflammatory monocyte markers (PDL-1, HLA-G, ICOS)

# Financials

---

|                                                    |        |
|----------------------------------------------------|--------|
| Cash and Cash Equivalents (MM) as of June 30, 2014 | \$16.2 |
| Shares outstanding (MM) as of June 30, 2014        | ~27.7  |
| Warrants (MM) <sup>(1)</sup>                       | ~3.1   |
| Stock Options (MM) <sup>(2)</sup>                  | ~2.5   |

(1) Weighted average exercise price = \$4.12 as of June 30, 2014

(2) Weighted average exercise price = \$2.96 as of June 30, 2014

# Experienced Management Team and Board of Directors

## Neil Warma, President & CEO, Director

- › 19+ years international healthcare experience with large Pharma and emerging biotechnology companies
- › Former Senior Management, Novartis Pharmaceuticals, Basel, Switzerland
- › Former CEO, Viron Therapeutics, Inc.
- › Co-founder and President of MedExact Inc., a company subsequently acquired

## Karthik Radhakrishnan, Chief Financial Officer

- › 10+ years of health care capital markets experience
- › Formerly, Vice President at ING Investment Management
- › MBA, MS in Engineering, CFA charter holder

## Don Healey, Ph.D., Chief Scientific Officer

- › 25+ years of experience in cellular immunology and immune regulation
- › Former Director of Immunology, Argos Therapeutics

## Donna Rill, Chief Development Officer

- › 30 years in cell and gene therapy research and clinical application
- › Designed and validated cGMP Cell & Gene Therapy Laboratories, Vector Production facilities, and Translational Research Labs

## Kenny Frazier, VP of Clinical Dev. and Regulatory Affairs

- › 24 years of extensive clinical and regulatory experience
- › Formerly, Head of Clinical Operations, Lexicon Pharmaceuticals and Tanox, Inc.

## Board of Directors

### Timothy Barabe

Board member of Arqule, Inc.; Former CFO of Affymetrix, Human Genome Sciences, Inc., Regent Medical UK and Sandoz GmbH

### Dr. Hans-Peter Hartung

Chair of Neurology at Heinrich-Heine University, Germany; Executive Board member of ECTRIMS

### Gail J. Maderis

CEO, BayBio, Former CEO of Five Prime Therapeutics, Founder of Genzyme Molecular Oncology

### Michael S. Richman

CEO, Amplimmune

### Scott B. Seaman

Executive Director, Alkek Foundation

### Neil K. Warma

President & CEO, Opexa

# SPMS Scientific Advisory Board

---

- **Dawn McGuire, M.D., FAAN (Chair)**
  - Advisory Council of the Gill Heart Institute
  - Former Vice President of Clinical Research at Elan Pharmaceuticals
- **Hans-Peter Hartung, M.D**
  - Chair of Neurology at Heinrich-Heine University, Düsseldorf
  - Executive Board member ofECTRIMS, World Health Organization Advisory Board on MS
- **Mark S. Freedman, M.D.**
  - Director of the Multiple Sclerosis Research Unit at Ottawa Hospital
  - Multiple Sclerosis Society of Canada, National MS Society (USA)
  - ACTRIMS committee member
- **Clyde Markowitz, M.D.**
  - Director of MS Center at the University of Pennsylvania
- **Doug Arnold, M.D.**
  - James McGill Professor Neurology and Neurosurgery at the Montreal Neurological Institute
- **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

# Investment Thesis

---

- T-cell platform company with Fast Track designation in SPMS
- Strong Intellectual property with 50 issued patents (domestic and international)
- Esteemed Scientific Advisory Board
- Precision Immunotherapy potentially optimizes benefit-risk profile
- Targeting an unmet medical need in a potentially substantial market
- Option Agreement with Merck Serono, a strong potential commercial partner
- Replacement value of company is multiples of present market cap
- Attractive potential risk-reward profile for long term/value investors
- Goal-oriented management team focused on value creation



# APPENDIX

---

# Tcelna® Highlights

---

- Clinical studies conducted in Relapsing Remitting and Secondary Progressive MS
- Progress made on manufacturing with focus on commercially viable process
  - Over 850 Tcelna preparations have been successfully manufactured, reproducible and consistent
- Safety demonstrated and trends in clinical efficacy across broad spectrum of patients for Relapsing Remitting and Secondary Progressive MS
- Dose and regimen for ongoing clinical development has been selected based on dose ranging studies
- Commercial opportunities in MS:
  - Secondary Progressive MS
    - Abili-T Phase II SPMS trial ongoing with 180 patients expected in U.S. and Canada
    - Fast track designation
  - Relapsing Remitting MS
    - Formal *End of Phase II* meetings have been conducted
    - FDA feedback obtained for potential Phase III studies

# **Tcelna Manufacturing: Personalization followed by Expansion Step**

---

## **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

# Tcelna Manufacturing: Precision Medicine

## Proprietary Assay Enables Annual Personalized Treatments

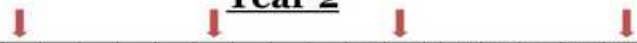
### Year 1

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 shift
- Develop newly personalized formulation annually based on evolved epitope profile

### Year 3



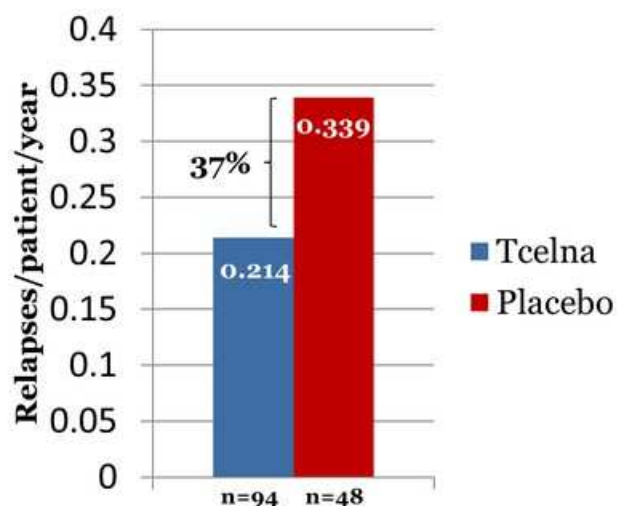
| Patient XX | Peptide 1 | Peptide 2 | Peptide 3 | Peptide 4 | Peptide 5 | Peptide 6 | Peptide 7 | Peptide 8 | Peptide 9 | Peptide 10 | Peptide 11 | Peptide 104 | Peptide 105 | Peptide 106 | Peptide 107 | Peptide 108 | Peptide 109 |
|------------|-----------|-----------|-----------|-----------|-----------|-----------|-----------|-----------|-----------|---------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

# TERMS Study

## A Completed Phase IIb Clinical Trial in RRMS

---

- Completed Phase IIb clinical trial in 150 RRMS patients; 33 sites in U.S.
- mITT population (n=142)
  - 37% reduction in ARR vs. placebo
  - ARR 0.214 vs. 0.339
- Superior safety and promising efficacy trend demonstrated
- Two *End-of-Phase II* meetings with FDA successfully completed

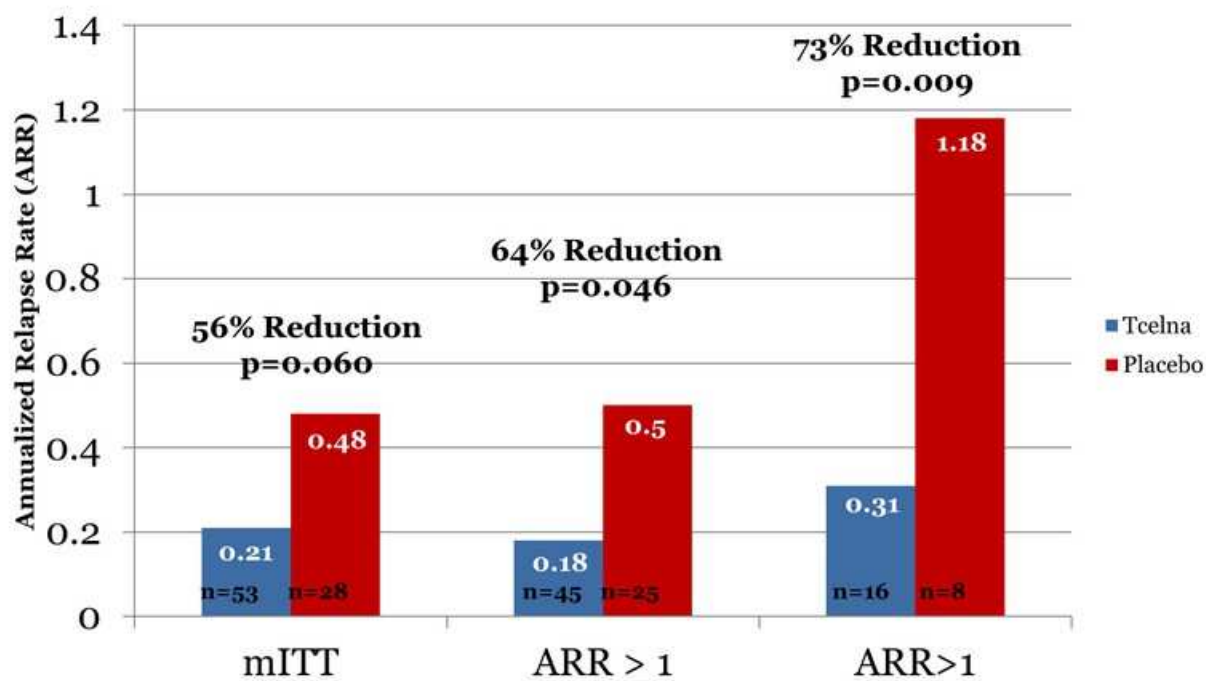


# TERMS Study: Safety Overview

---

| Safety Summary                |                                                                                            |
|-------------------------------|--------------------------------------------------------------------------------------------|
| Deaths                        | <ul style="list-style-type: none"><li>• No deaths occurred</li></ul>                       |
| Serious Adverse Events (SAEs) | <ul style="list-style-type: none"><li>• No treatment-related SAEs</li></ul>                |
| Most common adverse events    | <ul style="list-style-type: none"><li>• Mild to moderate injection site reaction</li></ul> |

# TERMS Study - Subgroup Analysis in Patients Naïve to DMT



Annualized relapse rate in DMT naïve populations. ARR in placebo subjects without previous disease modifying treatment (DMT) experience reflects relapse rates commonly seen in other placebo controlled trials in MS. In this subpopulation, treatment with Teelna resulted in a 56–73% reduction in ARR compared with placebo.

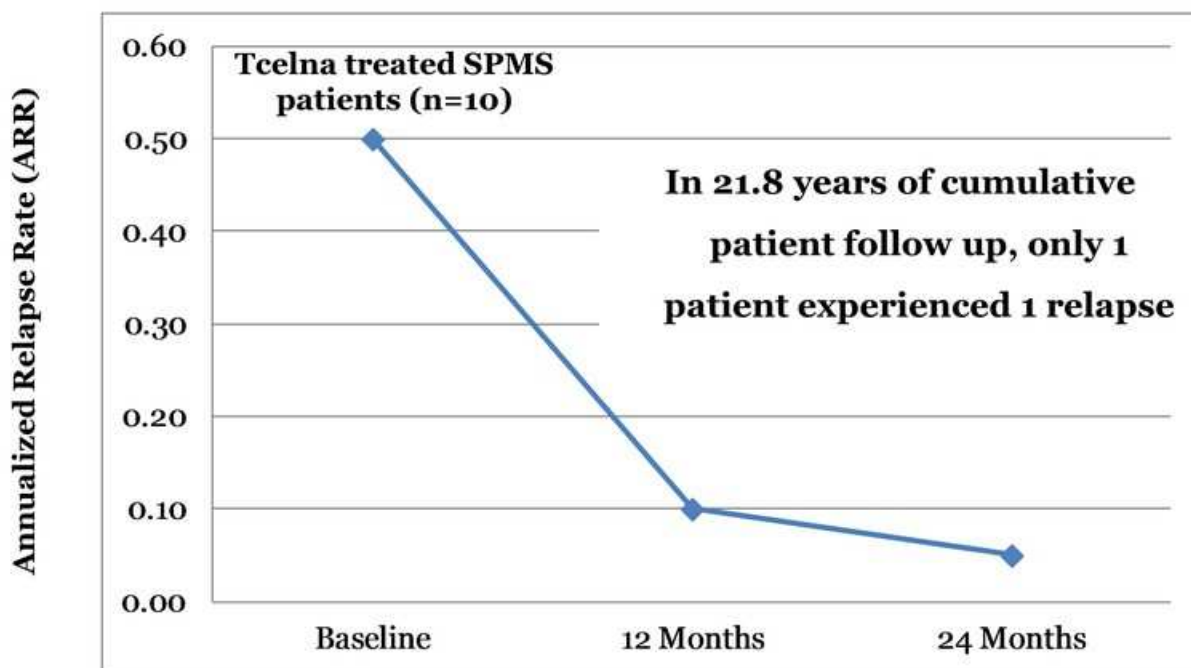
# **Secondary Progressive MS:**

## **Overview of Phase I/II Patients treated with Tcelna**

---

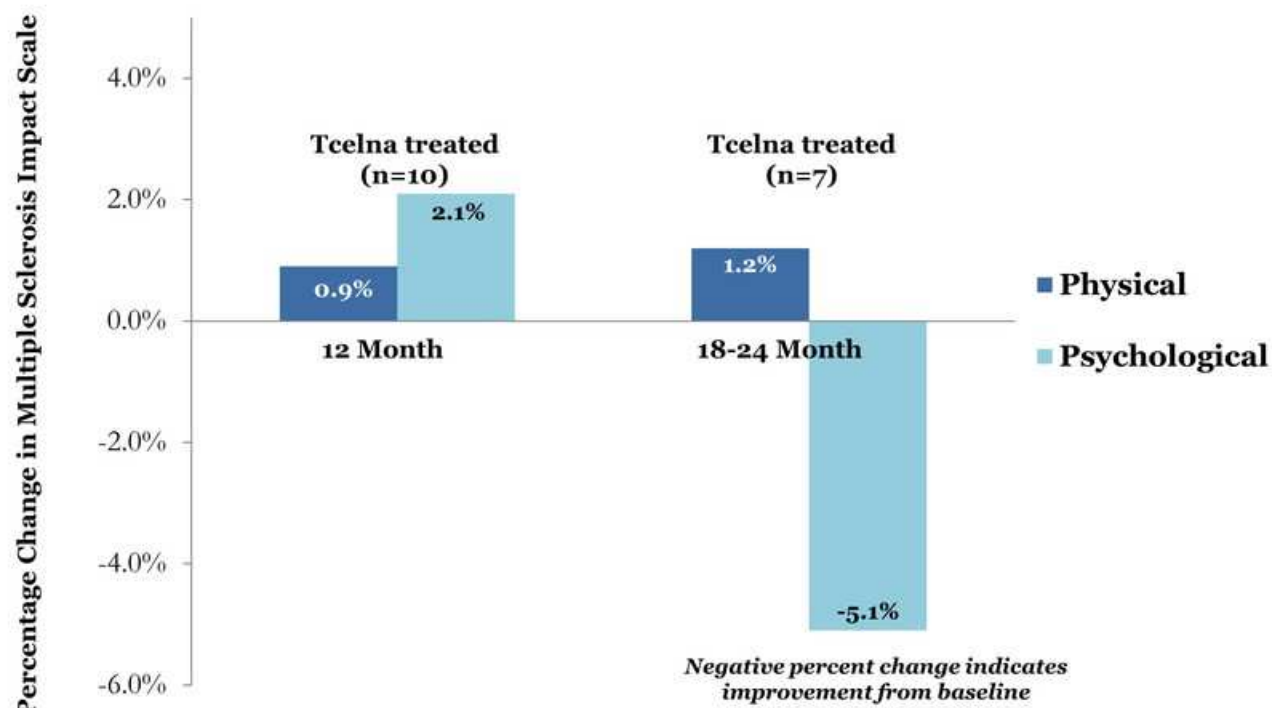
- **36 SPMS patients treated in three clinical studies**
  - 35 of these patients were treated with Tcelna for two years
  - 26 patients treated at Baylor; 10 patients treated by Opexa
- **Promising efficacy observed**
  - Disease stabilization as measured by EDSS was observed in 80% of patients at two years (compared to a historical control data that shows progression rate of 40%)
  - 10 patients in Opexa sponsored study showed significant reduction in relapse rates from a baseline ARR of 0.5 to an ARR of 0.1 at two years
  - No worsening in key Quality of Life Indicators (physical and psychological condition) at two years
- **Well-tolerated with a favorable safety profile**
  - No Serious Adverse Events

# Tcelna Reduces Annualized Relapse Rate (ARR) in SPMS at 2 years



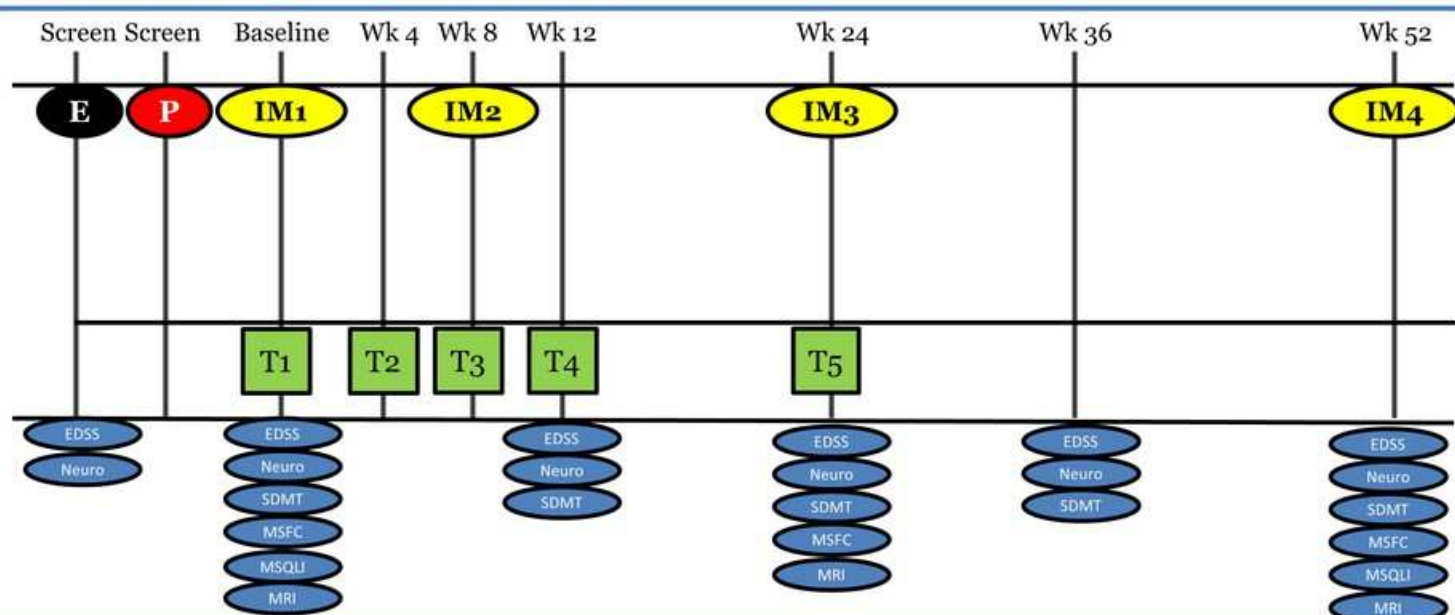
*One relapse observed in 21 years of cumulative patient follow up*

# Tcelna Stabilizes Quality of Life Scores in SPMS at 2 years



***Following two years of treatment, no worsening of physical condition and improvement in psychological condition***

# Abili-T Annual Treatment and Efficacy Assessment Schedule



## Efficacy Assessments

EDSS: Expanded Disability Status Scale  
 NEURO: Neurological Examination  
 SDMT: Symbol Digit Modalities Test  
 MSFC: MS Functional Composite  
 MSQI: MS Quality of Life Indicator  
 MRI: Magnetic Resonance Imaging



Tcelna dose



Epitope  
Profile Assay



Immune  
Monitoring



500 mL  
Procurement

# Mechanism of Action

---

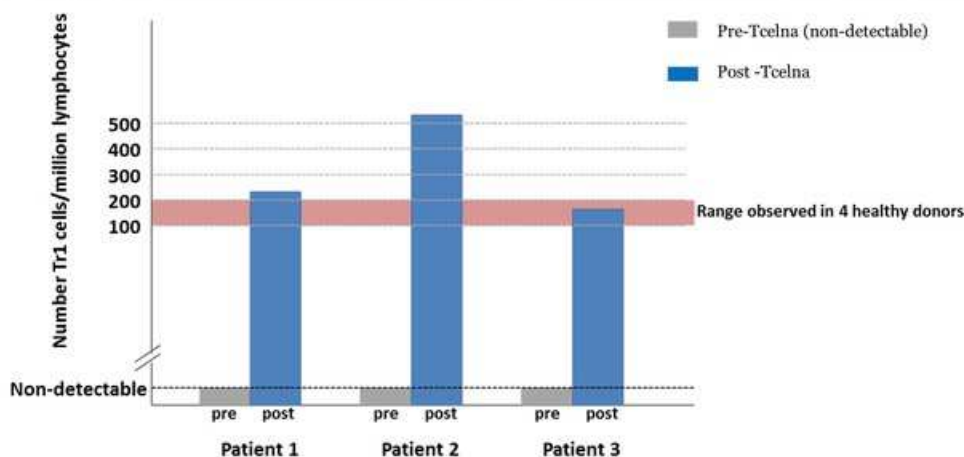
- **Attenuated, patient-specific (autologous) myelin reactive T-cells (MRTC)**
  - MRTC expanded *ex vivo* in response to immunodominant peptides of MBP, MOG and PLP
- **Therapeutic sc dosing ( $30-45 \times 10^6$  cells) stimulates host reactivity to the ‘over-represented’ MRTC inducing a dominant negative ‘regulatory T-cell’ response leading to:**
  - Down-regulation of similar endogenous disease-causing myelin reactive T-cells
  - Potential to induce up-regulation of regulatory cells (Foxp3+ and Tr1 cells) to reduce inflammation and provide possible neuroprotection, should these gain entry to the CNS
- **Phase I/II studies conducted with Tcelna in RRMS and SPMS showed a substantial reduction in MRTC, and improved clinical outcomes**
  - lower relapse rates and stabilization of disease as defined by EDSS

# Mechanism of Action

## Tcelna increases Tr1 cells in SPMS Patients

### Observations

- Increase in Tr1 cells from non-detectable to detectable levels in Tcelna treated patients (n=3)
- Increase in Tr1 cells to a level similar to those observed in healthy controls (n=4)
- $p=0.971$  (i.e. no statistical difference between healthy donor and post-treatment TR1 dose levels)



### Clinical findings in Tcelna treated patients

- All three patients (who had experienced relapse in preceding 12-24 month) remained relapse free during the 52-week assessment
- Showed stabilization of disease progression
- Showed a 57%-67% reduction in myelin reactive T-cell counts from baseline

# Mechanism of Action

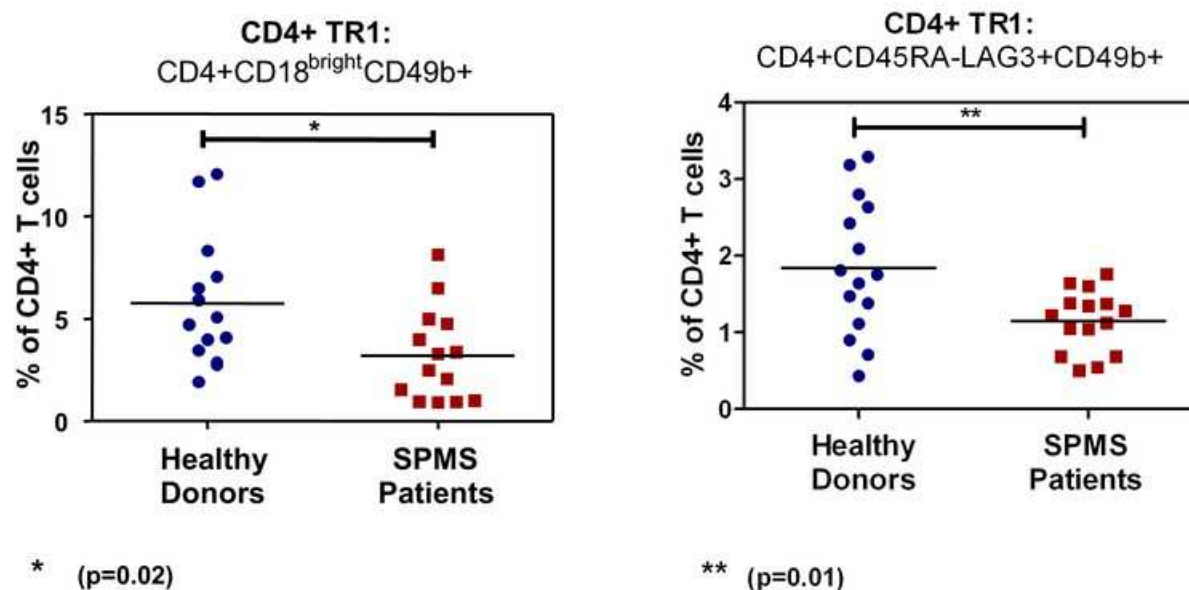
## The Immunopathology of SPMS

---

- **SPMS is associated with compartmentalized CNS inflammatory cells**
  - Microglia activation suggests ongoing chronic innate immune responses
- **SPMS is associated with inflammatory processes localized within the central nervous system**
  - Mechanisms responsible for continued neurodegeneration in SPMS are distinct from those of RRMS
  - In SPMS, it is believed that the Blood Brain Barrier (BBB) remains closed enabling only those products that are able to cross the BBB to be potentially effective
  - SPMS is characterized by a chronic inflammatory process (vs. acute episodes for RRMS)
- **Immunological defects associated with progression to SPMS**
  - Levels of the anti-inflammatory cytokine IL-10 are reduced in patients with SPMS
  - A therapeutic that has the potential to restore local production of IL-10 would be expected to alleviate chronic inflammation, and thereby reduce neurodegeneration

# Baseline Abili-T patients versus Healthy Individuals

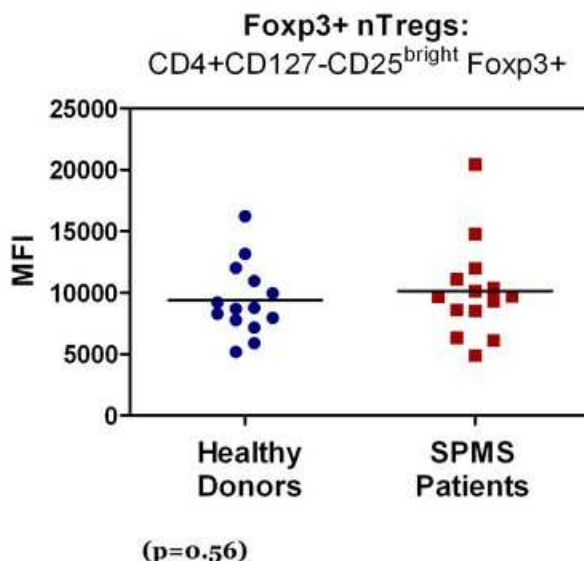
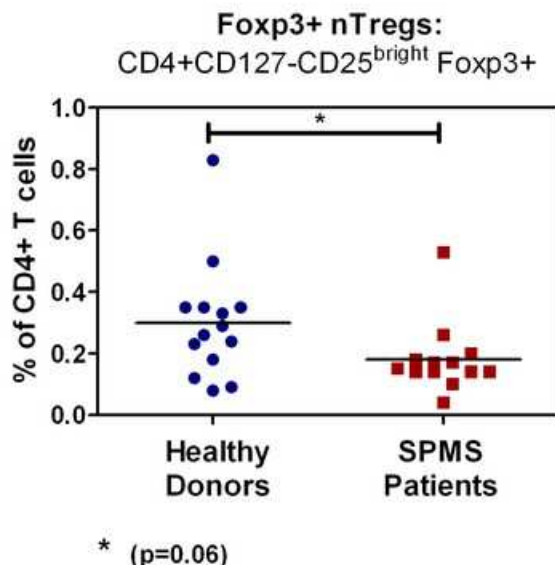
SPMS subjects have a reduced frequency of IL-10 secreting TR1 cells



ACTRIMS 2013 Poster: Profiling of Secondary Progressive MS by Multicolor Flow Cytometry  
 Lauren W Collison, Ph.D; Chris L Ayers, Ph.D., Jordan L Harrell, MBE; Don Healey, Ph.D.

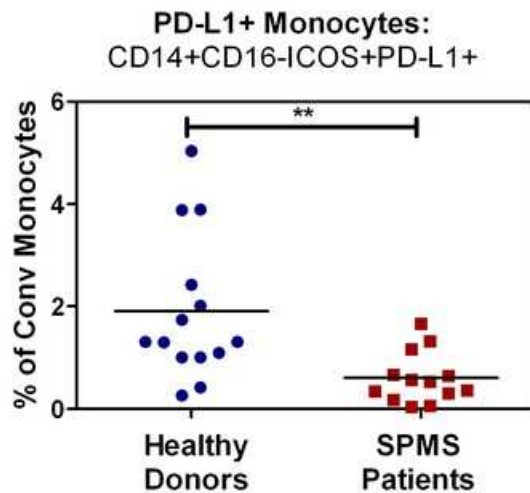
# SPMS subjects have reduced nTReg cells (another type of CD4 cells)

Frequency of nTregs may be reduced in SPMS, although the level of Foxp3 expression is equivalent to that of healthy donors. Therefore, function of Tregs cells is equivalent between the populations, unlike in RRMS where reduction in Tregs may play a greater role in the persistence of autoimmunity.

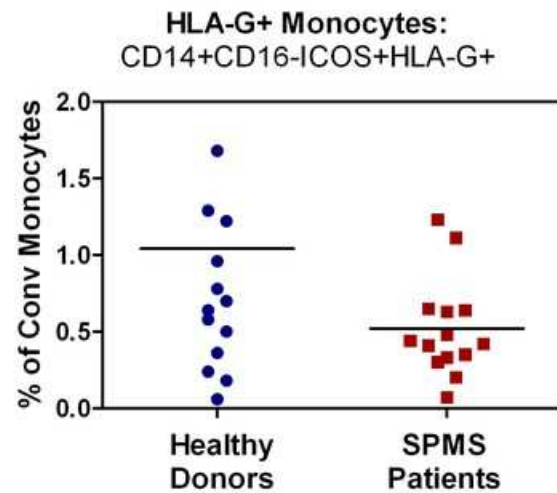


ACTRIMS 2013 Poster: Profiling of Secondary Progressive MS by Multicolor Flow Cytometry  
Lauren W Collison, Ph.D; Chris L Ayers, Ph.D., Jordan L Harrell, MBE; Don Healey, Ph.D.

# SPMS patients have fewer Anti-inflammatory monocytes (PDL1 and HLA-G)



\*\* (p=0.004)



(p=0.164)

ACTRIMS 2013 Poster: Profiling of Secondary Progressive MS by Multicolor Flow Cytometry  
Lauren W Collison, Ph.D; Chris L Ayers, Ph.D., Jordan L Harrell, MBE; Don Healey, Ph.D.