

OPEXA THERAPEUTICS, INC.

FORM 8-K (Current report filing)

Filed 10/09/14 for the Period Ending 10/09/14

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 9, 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))
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Item 7.01. Regulation FD Disclosure.

Opexa Therapeutics, Inc. (the “**Company**”) will host an Analyst and Investor Event in New York City, New York on Thursday, October 9, 2014, at which members of the Company’s senior management team will provide an R&D update and review of the Company’s Abili-T clinical trial for Secondary Progressive Multiple Sclerosis and the development provide for Neuromyelitis Optica.

The Analyst and Investor Event will take place from 8:00 a.m. to 9:15 a.m. Eastern Time at the New York Hilton Midtown, Madison Room (2nd floor). If you would like to attend the event, please confirm your attendance by e-mail to czuckero@opexatherapeutics.com or via telephone at 281.775.0600. A live webcast of the event will start at 8:15 a.m. Eastern Time and may be accessed on the Investor Relations section of the Company’s website (www.opexatherapeutics.com). An archived version of the webcast will be available for 90 days after the event on the Company’s website.

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) and will be used at the Analyst and Investor Event and other meetings is furnished as Exhibit 99.1 and 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	Opexa Therapeutics, Inc. Investor Presentation, October 2014.

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 9, 2014

OPEXA THERAPEUTICS, INC.

By: /s/ Neil K. Warma
Neil K. Warma
President & Chief Executive Officer

EXHIBIT INDEX

<u>Exhibit No.</u>	<u>Description</u>
99.1	Opexa Therapeutics, Inc. Investor Presentation, October 2014.



OPEXA THERAPEUTICS



Precision Immunotherapy®

Opexa Therapeutics, Inc.

NASDAQ: OPXA

October 2014
The Woodlands, TX

Forward-Looking Statements

All statements in this presentation other than those of historical fact, including statements regarding our preclinical and clinical development plans for Tcelna® and OPX-212, our research and other development programs, our ability to undertake certain activities and accomplish certain goals, projected timelines for our research and development activities and possible regulatory approvals, if any, our expectations regarding the relative benefits of our product candidates versus competitive therapies, our expectations regarding the possibility of licensing or collaborating with third parties regarding our product candidates or research, and our expectations regarding the therapeutic and commercial potential of our product candidates, research, technologies and intellectual property, are forward-looking statements. The words “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “design,” “intend,” “expect,” “potential” and similar expressions, as well as the negative version of these words and similar expressions, are intended to identify forward-looking statements. Our forward-looking statements do not constitute guarantees of future performance, and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those anticipated or implied in such statements. Our forward-looking statements are based upon our current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated as a result of various risks and uncertainties which include, without limitation, risks associated with the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics and risks inherent in the effort to build a business around such drugs. Although we believe our expectations are reasonable, we do not in any way guarantee future results, level of activity, performance or achievements. In addition, neither we nor any other person assumes responsibility for the accuracy and completeness of any forward-looking statements. Our forward-looking statements in this presentation speak only as of the date this presentation is actually delivered by us in person. We assume no obligation or undertaking to update or revise any statements to reflect any changes in our expectations or any change in events, conditions or circumstances on which any such statement is based. You should, however, review additional disclosures we make that further describe risks and uncertainties relevant to us in additional detail in our filings with the Securities and Exchange Commission. You may get these documents for free by visiting EDGAR on the SEC web site at <http://www.sec.gov>.

Opexa Key Investment Highlights

- **Personalized T-cell immunotherapy platform**
 - Autologous cell therapy
 - Potential to address multiple therapeutic areas
 - Strong Patent Estate: 98 patents issued on T-cell platform (domestic and international)
- **Lead Indication: Multiple Sclerosis**
 - Ongoing Phase 2b trial in Secondary Progressive Multiple Sclerosis (SPMS)
 - Potential SPMS market in North America alone could exceed \$7 Billion
 - Fast Track designation from the U. S. FDA for the treatment of SPMS
 - Option Agreement with Merck Serono, a strong potential commercial partner
- **Pipeline (platform) expansion: OPX-212 for Neuromyelitis Optica (NMO)**
 - No FDA approved drugs for the treatment of NMO
 - NMO is classified as an orphan disease
 - OPX-212 potential peak sales range ~\$300 - \$500 million
 - Subject to successful clinical development

Opexa: A Unique Drug Discovery Engine

ImmPath®: Opexa's Proprietary Platform

Personalized, targeted immune therapies

Potential new autoimmune indications

e.g.

- Type 1 Diabetes
- Lupus
- Myasthenia Gravis
- Several orphan diseases

Preclinical

**Neuromyelitis
optica**

Phase 2b

**Secondary Progressive
Multiple Sclerosis**

Strong IP Estate

- New provisional patent expands portfolio to cover B-cell diseases
- 98 issued patents, U.S. and international
- Covering T-cell diseases

Opexa Developing Highly Differentiated, Precision Therapies

Programs and indications	Research	Preclinical	Phase 1	Phase 2	Phase 3
Tcelna (imilecleucel-T) *					
Secondary Progressive Multiple Sclerosis (SPMS)	Phase 2b trial ongoing, data expected 2H 2016				
Relapsing Remitting Multiple Sclerosis (RRMS)	Phase 3 not initiated				
OPX-212					
Neuromyelitis Optica (NMO)	IND enabling activities ongoing in 2H 2014				

* Option & License agreement signed with Merck Serono, MS indications only, worldwide, excluding Japan

Recent and Upcoming Expected Milestones

Tcelna: Multiple Sclerosis

- ✓ 1H 2014: Completed Enrollment in Phase 2b SPMS trial
- 2H 2016: Top line results for Abili-T Phase 2b SPMS trial

OPX-212: NMO

- ✓ 1H 2014: Initiated Early Development Plan
- Mid- 2015: Submit IND
- 2H 2015: Initiation of Clinical Development (subject to IND acceptance and financing)
- 2015/16: Apply for Orphan Designation and Fast Track

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) (1)	~3.1
Stock Options (MM) (2)	~2.5
Debt	None

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

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



TCELNA[®] (*imilecleucel-T*) **MULTIPLE SCLEROSIS**

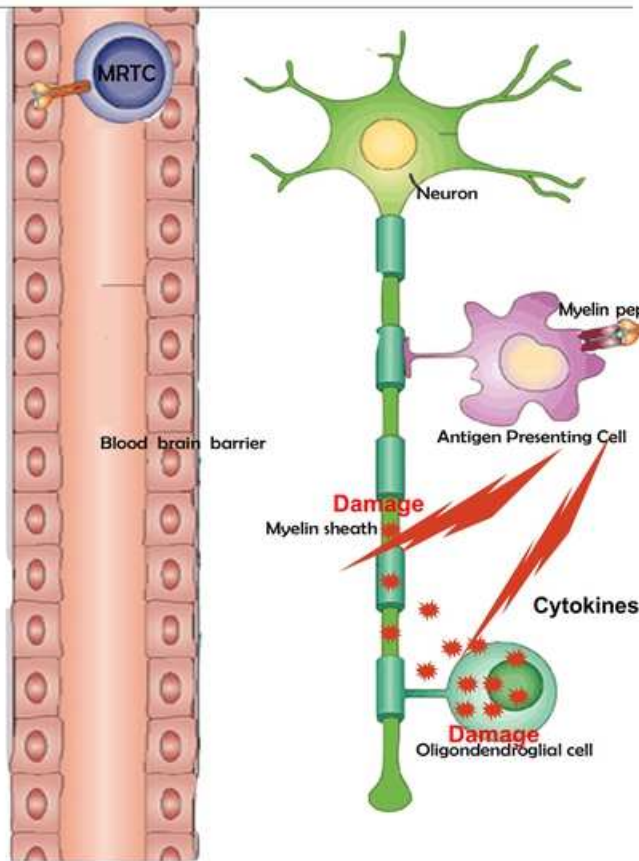
Tcelna[®]

Lead Program Targeting Secondary Progressive MS patients

Fast Track Designation by FDA

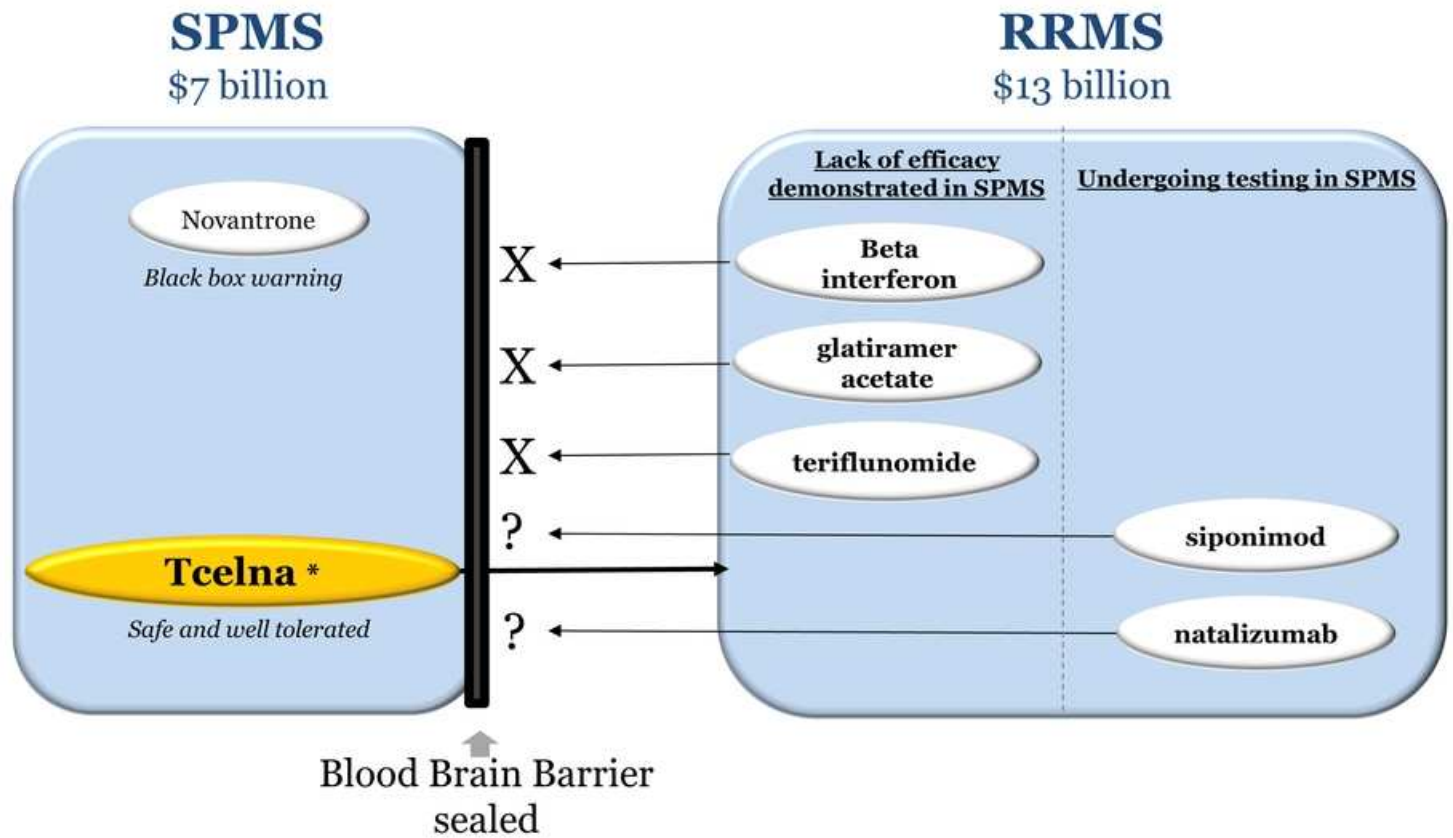
- **Phase 2b clinical trial ongoing**
- **Trial is fully enrolled: 190 patients with SPMS**
- **Top line data expected in 2H 2016**
- **Design**
 - Double-blind, 1:1 randomized, placebo-controlled
 - 35 clinical sites in USA and Canada
 - Two annual courses of personalized therapy
 - Efficacy Endpoints: Primary-Whole Brain Atrophy, Secondary-Disease Progression
- **Immune Monitoring being conducted in parallel**
 - Comprehensive biomarker analysis

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) leading to:
 1. Destruction of myelin sheath, the protective coating of nerve fibers
 2. Destruction of oligodendroglial cells, which are responsible for producing myelin
- Therapeutic dose of Tcelna (attenuated T-cell clones) is injected subcutaneously
- This triggers an immune response specifically targeting circulating MRTC
- Immune cells, including Tregs, have been primed, or sensitized to specifically target the pathogenic MRTC for elimination or regulation

Differentiation of Tcelna in SPMS



* Subject to successful clinical development and FDA approval

Merck Serono

Agreement signed 2013; strong potential partner

Option and License Agreement for worldwide rights to all Multiple Sclerosis indications, excluding Japan

- **If Merck Serono exercises option:**
 - Merck Serono to fund Phase 3, pre-commercial and commercial activities
 - Merck Serono obtains rights to develop Tcelna for all MS indications
 - Worldwide rights excluding Japan
- **Opexa received \$5 million upfront option fee at signing**
- **Opexa has potential to receive additional \$220 million in milestone payments, and**
- **Royalties ranging from 8% to 15% of annual net sales with step-ups occurring if 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



CLINICAL DATA: MULTIPLE SCLEROSIS

Tcelna Development Program

Broad Spectrum of Multiple Sclerosis patients enrolled in clinical trials

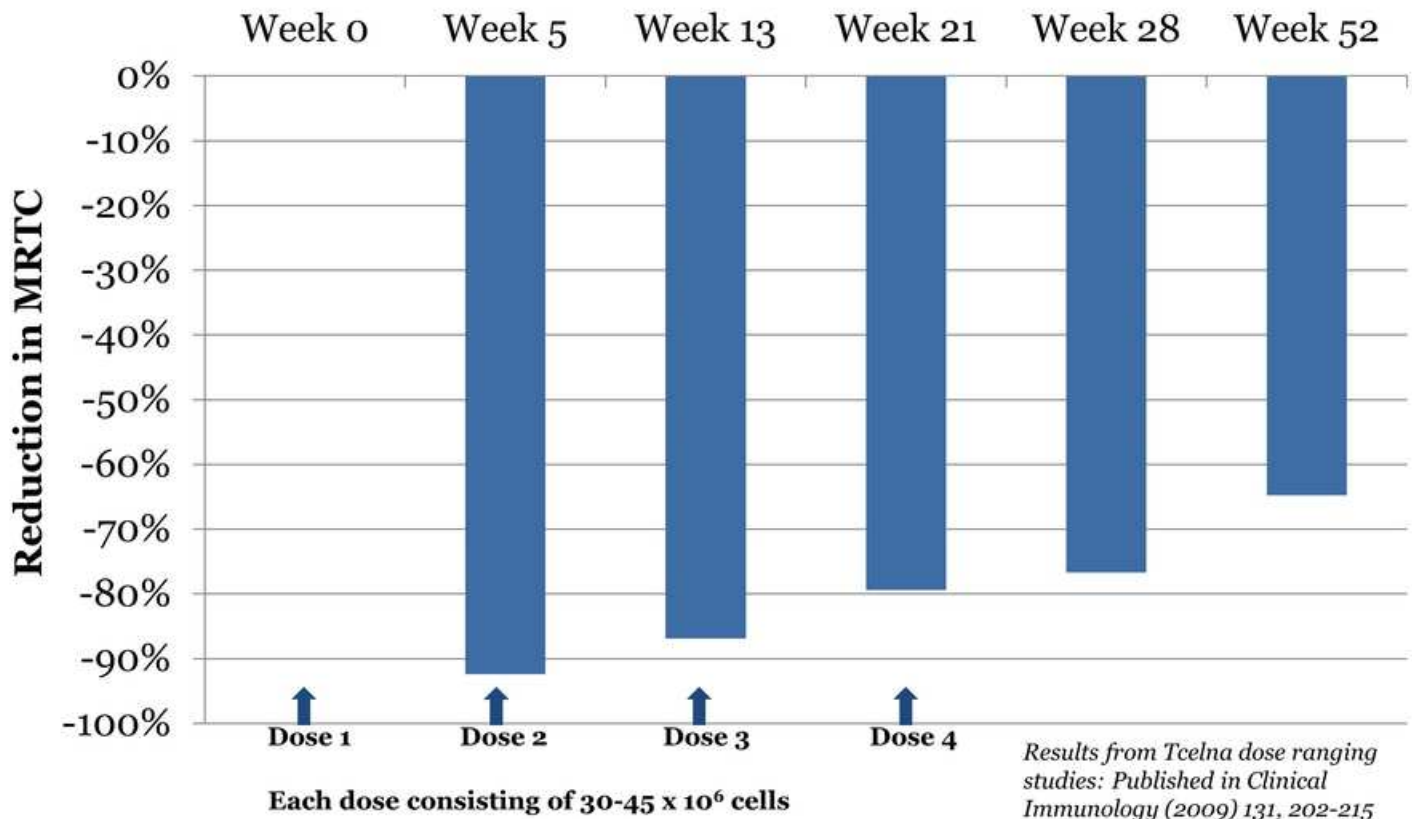
Phase	Completion Dates	Population	Total N	Treatment Duration (months)	Tcelna	Placebo
Baylor	1998	RRMS, PPMS, SPMS (26)	114	Up to 24 months	114	
Phase I dose escalation	2006	RRMS (5) SPMS (6)	16	12	16	-
Phase I/II Open label retreatment	2007	RRMS (9) SPMS (4)	13	12	13	-
Phase IIb TERMS	2008	RRMS, CIS	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	190*	24*	95*	95*

* Expected upon completion of ongoing SPMS Abili-T trial

Mechanism of Action

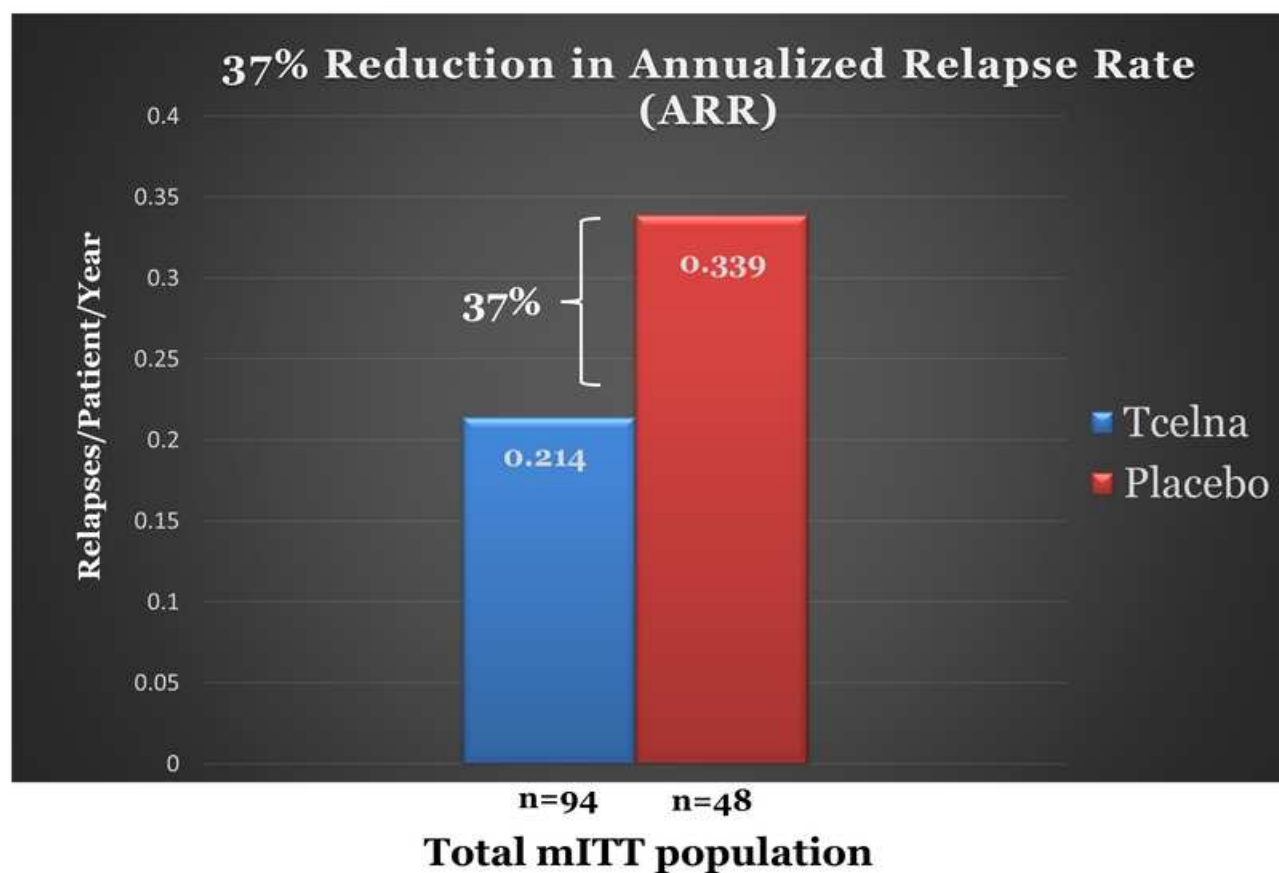
Reduction in Myelin Reactive T-Cells

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



Phase 2b RRMS clinical trial

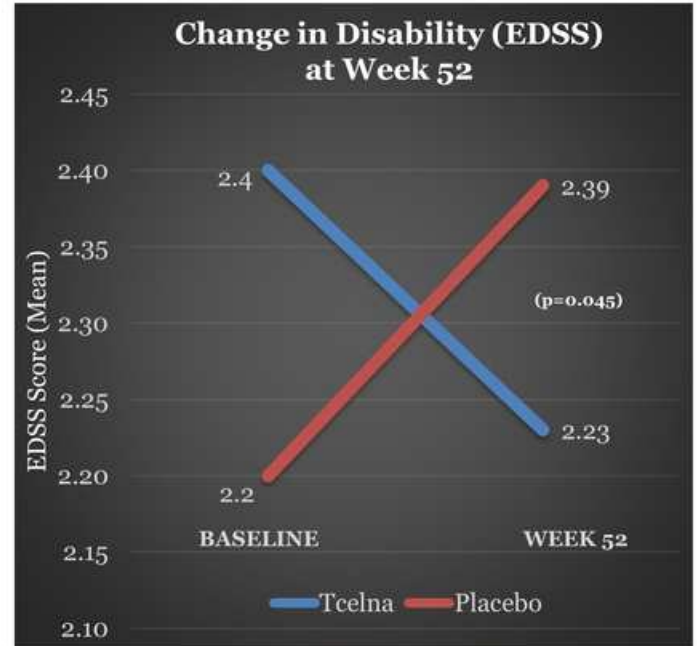
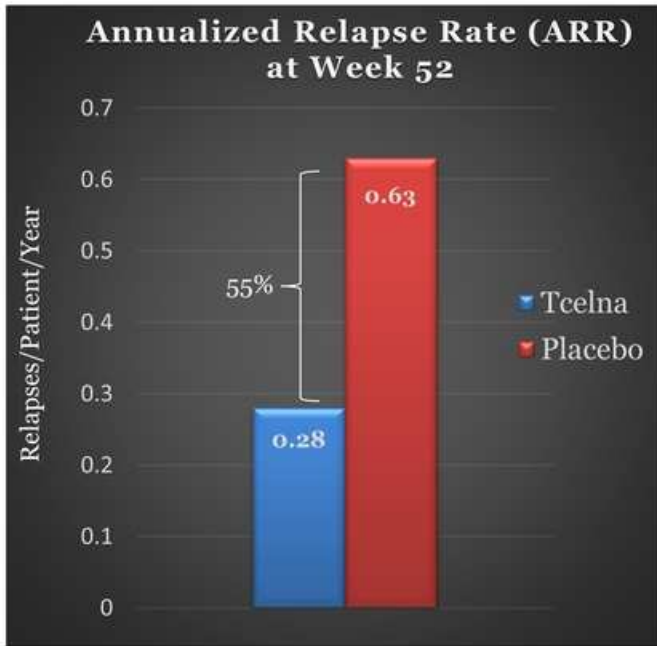
Tcelna showed 37% improvement over placebo (mITT)



Phase 2b RRMS Clinical Trial

Reversal of Disability in Prospective Analysis in More Active Patients

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

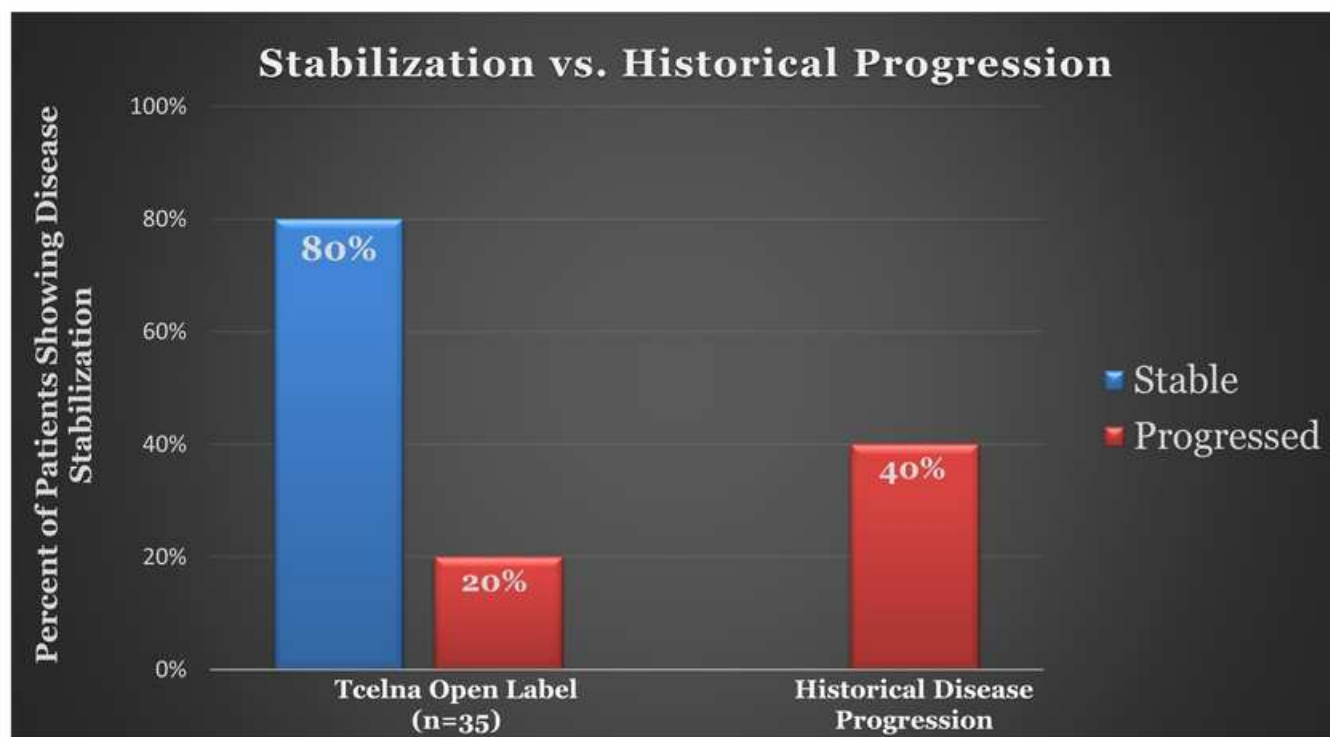


Statistically Significant Improvement in Disability (p=0.045)

Phase I/II SPMS Clinical Trials

Tcelna Stabilizes Disease in SPMS at 2 Years

80% of patients 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 *Honmes Lancet* 2004

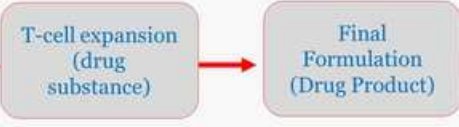


IMMPATH®: **PROPRIETARY DRUG DEVELOPMENT PLATFORM**

Proprietary Platform

Leverage existing platform to generate new drug candidates

ImmPath® Manufacturing Process

Predecessors to Manufacturing			Manufacturing		Dispensation
					
• Epitope Profiling performed to define patient specific peptide reactivity necessary for manufacturing • Shipment of blood to Opexa • Specimen logged and assigned a unique identifier number			• Clonal expansion of antigen-specific T-cells • Cryopreservation of cell lines • Cells thawed and diluted in excipient • Attenuated • Release testing		• Product released from QA • Shipped to physician's office

Strengths and Advantages of Opexa Platform

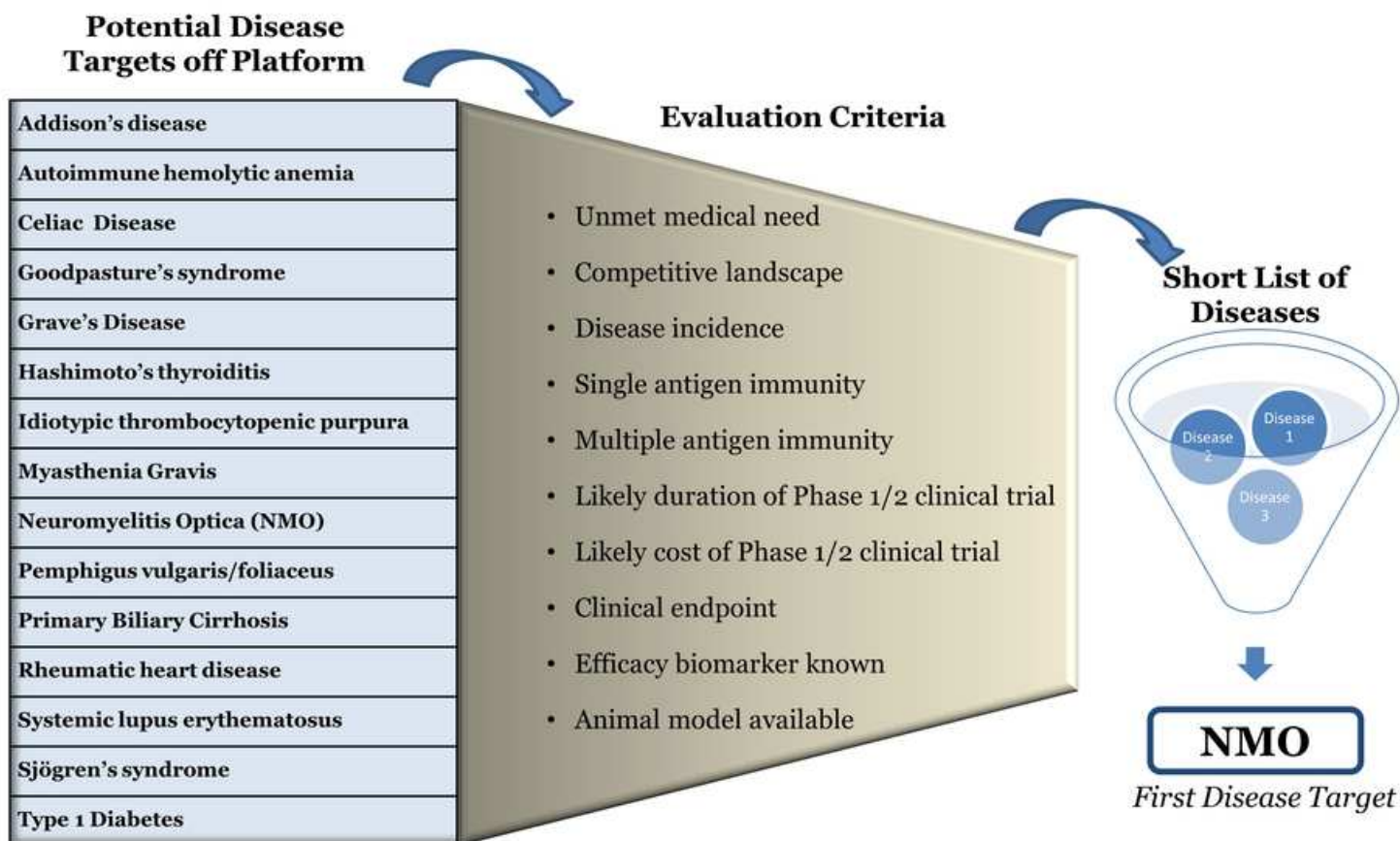
Potential Reduced Time and Cost to First in Man Study

- ImmPath, Opexa proprietary platform
 - Advantages:
 - Possibility exists to generate multiple drug candidates
 - Potential reduction in time to market
 - Potential reduction in development cost
- Preclinical timeline and cost:
 - From initiation of development program to IND
 - Opexa goal: < 1 year; <\$1 million
 - Others: 3-4 years; \$3-5 million



OPX-212: NEUROMYELITIS OPTICA (NMO)

Comprehensive Process to Determine Optimal Additional Indications for Opexa



NMO: A rare disease of the Central Nervous System



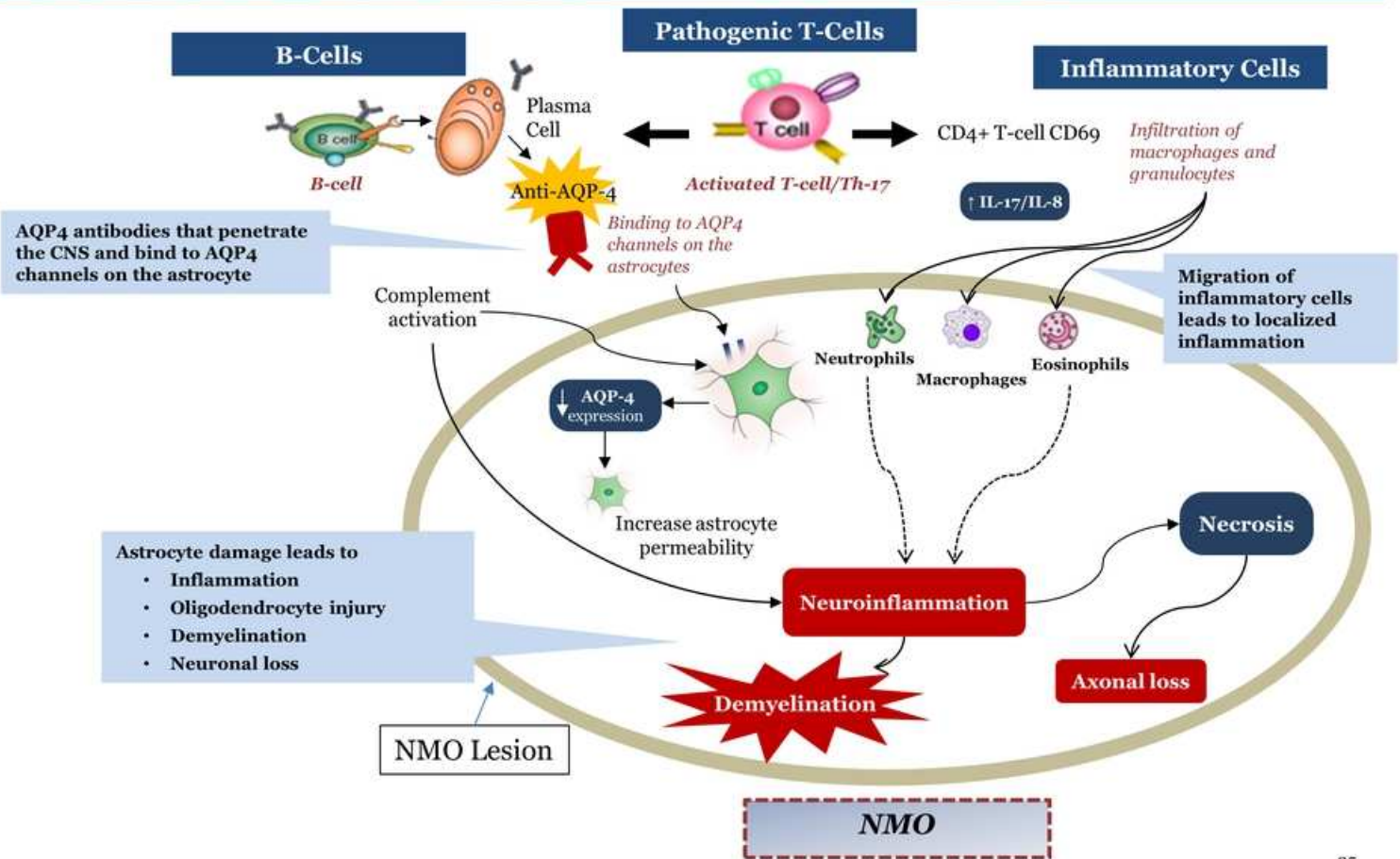
Image reprinted with permission from MultiView, Inc.

- Neuromyelitis optica (NMO) is a rare or orphan autoimmune disease
- Immune system cells and antibodies mistakenly attack and destroy myelin cells in the optic nerves and the spinal cord
- Individuals with NMO develop optic neuritis, which causes pain in the eye and vision loss, and transverse myelitis, which causes weakness, numbness, and sometimes paralysis of the arms and legs
- There are no FDA-approved therapies for NMO
- Affects any age varying from 3 to 90 years, the average age of onset ~41 years

An orphan disease with no FDA-approved therapy

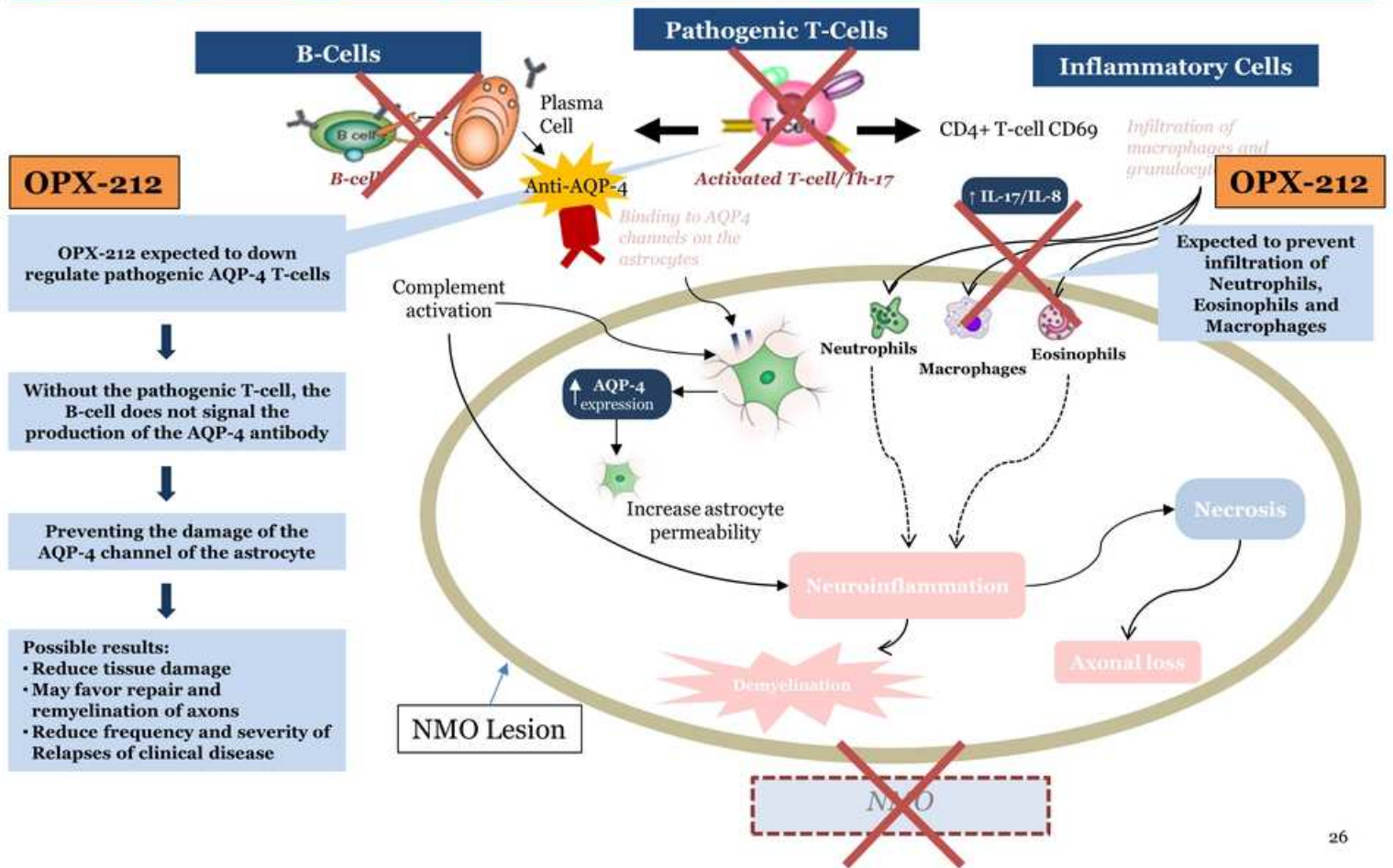
Disease Pathogenesis

An astrocytopathic disease of the CNS characterized by optic neuritis, transverse myelitis; it is a B-cell-dependent disease associated with anti-aquaporin-4 antibodies



Potential Intervention Points for T-cell Therapy

T-cell therapy believed to inhibit AQP-4 autoantibody, pro-inflammatory cytokines and prevent infiltration of macrophages and granulocytes



OPX-212 represents a unique T-cell immunotherapeutic approach in NMO

OPX-212

- Potential to avoid systemic immunosuppression
- Potential to selectively decrease AQP-4 reactive T-cells, and subsequently impact anti-AQP-4 antibody titer
- Stabilization of disease by preventing further destruction of AQP-4 expressing astrocytes, thereby limiting secondary demyelination of nerve fibers



NMO

Current therapeutic approach

- Systemic immunosuppression can lead to infections and cancers. Long term consequences not understood
- Fails to re-educate the immune response for immune 'tolerance' to AQP-4

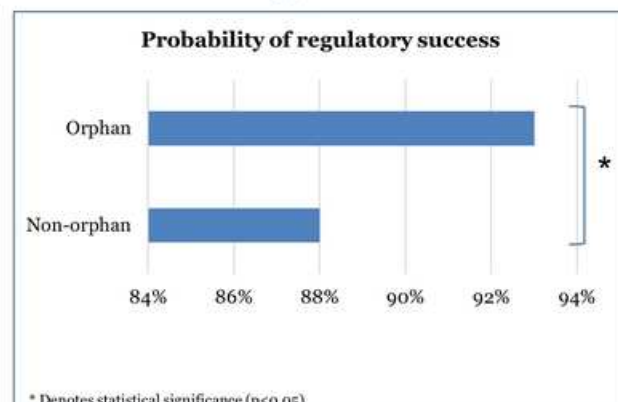
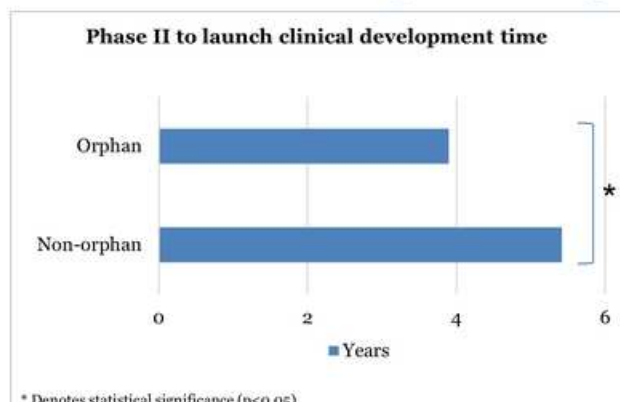


Orphan Drug Model:

Substantial Regulatory Advantages

- Rare diseases, also known as orphan diseases, individually affect small groups of patients but are estimated to affect over 25 million people alone in the U.S.
- Orphan disease defined as affecting less than 200,000 patients in the U.S.
- Orphan drug market estimated at > \$50 billion in 2011

Orphan Drug Development Advantages



Decreased development times (from Phase 2 to launch)

Reprinted from Drug Discovery Today, 17/ 13-14, Meekings, Williams & Arrowsmith, Orphan Drug Development: An economically viable strategy for biopharma R & D, 660-664, 2012, with permission from Elsevier

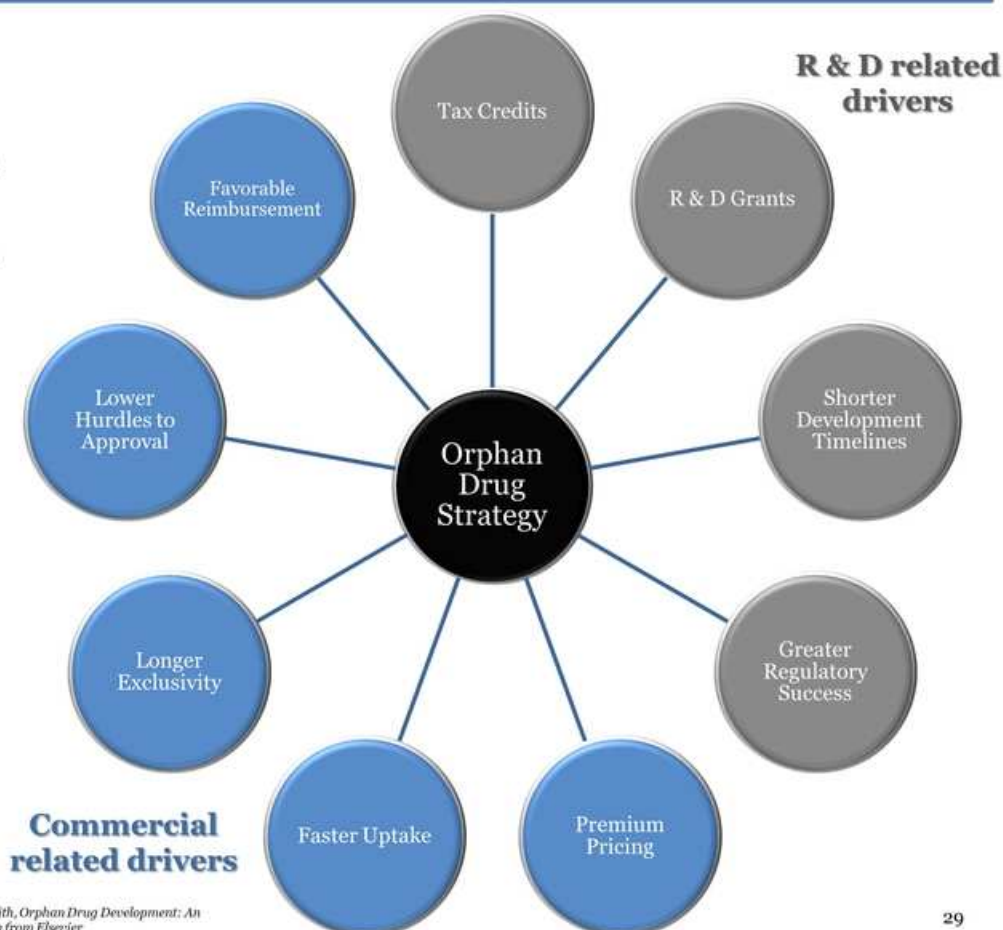
Greater chance of regulatory success

Orphan Drug Model:

Significant Economic and Commercial Potential

- Market exclusivity for orphan designated drugs:
 - U.S. 7 years; EU 10 years

Opexa has potential to pursue orphan drug strategy, possibly for several diseases



Reprinted from Drug Discovery Today, 17/ 13-14, Meekings, Williams & Arrouesmith, Orphan Drug Development: An economically viable strategy for biopharma R & D, 660-664, 2012, with permission from Elsevier

NMO Development Program

OPX-212 Early Development Plan was initiated in Q1 2014

Completed Activities		
✓	Q1/2 2014	Regulatory/Clinical - Pre-IND meeting conducted with FDA - Engaged Thought Leaders - Engaged Patient Advocacy Groups
✓	Q1/2 2014	R&D, Process Development, Manufacturing - Scientific justification of Mechanism of Action - Detection of EPA response in multiple NMO patient samples - Successful preclinical manufacturing runs
Planned Development Activities		
	2014	Preclinical Development
	Mid-2015	IND submission (subject to successful preclinical development)
	2H 2015	Clinical Development (subject to FDA review and acceptance of IND)

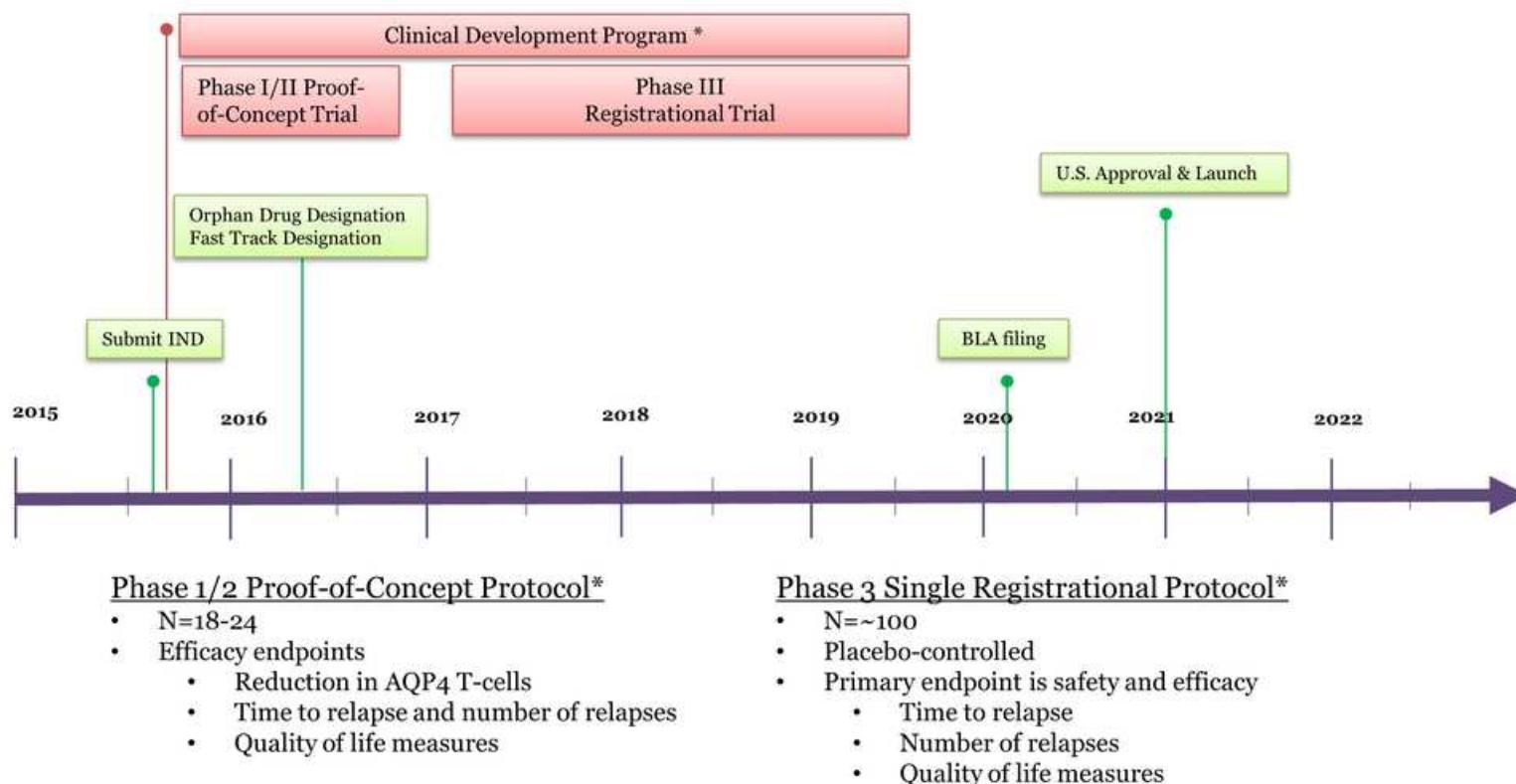
Expect minimal impact on cash burn to IND submission

NMO Early Development Program

Activities to support IND submission; Process and Product Development

- NMO patient apheresis samples are being utilized to help develop the Epitope Profile Assay to define T-cell reactivity to AQP4, as well as to refine the manufacturing process for OPX-212, and executing on CMC runs
- NMO patient blood draw samples are being tested for inter-patient epitope diversity in T-cell reactivity to AQP4
- NMO patient blood draws are being collected on a monthly basis for 6 months in order to test for intra-patient variability over time
- Pre-clinical 'bio-activity' study in mice to demonstrate the potential of a prototype murine attenuated anti-AQP4 product to modulate immunity to AQP4

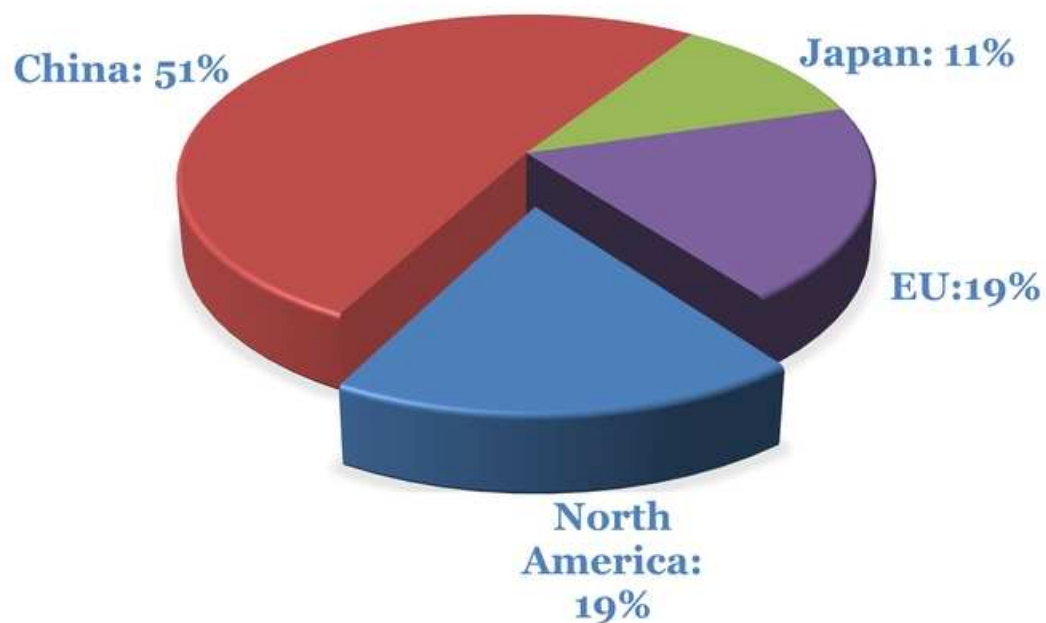
OPX-212: Potential US product launch projected as early as 2021



* Subject to FDA review and approval, resource funding

Global Patient Breakdown in 2021

Global patient numbers estimated at 70,000 – 140,000



NMO Investment Thesis

- Any therapy that could achieve tolerization of the AQP-4 reactive T-cells may offer a cure for NMO
- OPX-212 has the potential to address the root cause of NMO
- OPX-212 leverages Opexa's T-cell platform to B-cell mediated orphan diseases
- Opexa retains world wide economics from OPX-212. Product has potential to generate \$300-\$500 million in peak sales, subject to successful clinical development
- Higher prevalence of NMO in Asia could complement Opexa's MS rights in Japan
- IND filing expected in Mid 2015
- Open label Phase I/II clinical trials expected to start in 2H 2015 (subject to IND acceptance and financing)

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

- › 25+ 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

Opexa Investment Thesis

TECHNOLOGY

- Proprietary Platform: potential to yield multiple drug candidates
- Differentiated, personalized approach
- Strong patent estate (98 domestic and international)

PIPELINE

- **Tcelna**: Secondary Progressive MS, limited competition, \$7BN potential market
- **OPX-212**: Recently launched development program in NMO, no approved therapies, orphan indication
- Precision Immunotherapy potentially optimizes benefit-risk profile

VALIDATION

- Option agreement secured with Merck Serono – strong potential partner
- FDA Fast track designation for Tcelna in SPMS
- Esteemed Scientific Advisory Board