



### COMPANY PROFILE

**Opexa Therapeutics (NASDAQ:OPXA)** is a biotechnology company dedicated to the development of patient-specific cellular therapies for the treatment of autoimmune diseases. The company's leading product candidate has the potential to address the significant unmet medical needs of the large multiple sclerosis (MS) patient population.

**Opexa's lead therapy, Tovaxin®**, is a cellular immunotherapy currently in Phase IIb clinical development for the treatment of MS that is specifically tailored to each patient's disease profile. Opexa believes the potential combination of efficacy, superior safety, improved tolerability and administration may position Tovaxin as the MS treatment of choice as compared to existing therapeutics.

**Positive results from the Phase IIb** Tovaxin for Early Relapsing Multiple Sclerosis (TERMS) study demonstrated key indications of therapeutic activity, as well as an excellent safety profile.

**Planning is underway for** the next clinical study with Tovaxin in patients with MS.

### TOVAXIN®

Tovaxin possesses a unique mechanism of action that combats the demyelination of the nerve fibers in the central nervous system, the underlying cause of MS. Opexa believes Tovaxin may possess a number of advantages compared to other MS therapies currently available or in development, including:

**Efficacy** - Clinical trials conducted to date demonstrate that Tovaxin may result in a reduction in the Annualized Relapse Rate (ARR) for patients with Clinically Isolated Syndrome (CIS), Relapsing-Remitting Multiple Sclerosis (RRMS) and Secondary-Progressive Multiple Sclerosis (SPMS). Tovaxin has also been shown to improve disability in a number of MS patients and may have a neuroprotective benefit.

**Safety and Tolerability** - It is believed that Tovaxin treatment selectively targets and depletes the pathogenic T-cell population. It is not a general immune suppressant and, accordingly, is not associated with the serious side effects seen by those MS treatments that function by systemically suppressing the immune system. In clinical trials conducted to date, there have been no serious adverse events associated with Tovaxin treatment.

**Improved Compliance** - Currently, available therapies are administered monthly and, in some cases, daily. Tovaxin's treatment regimen of only five subcutaneous injections per year may provide significant compliance benefits to both patients and physicians.

**Customized Therapy** - Using Opexa's proprietary Epitope Analysis Assay (EAA), the company customizes Tovaxin treatments to specifically target an individual's disease progression and/or modification.

### INVESTMENT HIGHLIGHTS

- Public biotechnology company developing novel therapies for the treatment of autoimmune diseases including multiple sclerosis.
- Tovaxin - A 12-month analysis of the Phase IIb TERMS study demonstrated additional positive findings. In patients having at least one relapse in the 12 months prior to treatment (Annualized Relapse Rate or ARR  $\geq 1$ ), the data showed:
  - More than 83% of Tovaxin-treated patients remained relapse free at one year
  - 42% decrease in ARR following treatment compared to placebo, yielding an ARR=0.20
  - 73% of Tovaxin-treated patients showed stabilization or improvement in MS disability including 16.5% with a sustained improvement in the Expanded Disability Status Scale (EDSS) of at least one full point
- Tovaxin is being developed as first line treatment; may address significant market with unmet needs
  - Current MS therapies generate approximately \$9B sales annually
  - 50% of patients are non-compliant due to side effects and treatment regimens of current therapies
- Potential to generate significant revenue by capturing:
  - Non-compliant market (currently 50% of MS patients)
  - Newly diagnosed patients
  - Patients dissatisfied with currently marketed drugs
- August 2009: completed stem cell agreement with Novartis potentially worth \$50M
  - \$3M upfront payment and possible technology transfer milestones, as well as development and commercialization milestones and royalties
- Novel T-Cell technology platform supporting current development programs has applicability to other large disease opportunities
- Broad intellectual property portfolio
- cGMP and GTP compliant in-house manufacturing

## TOVAXIN PHASE 2b RESULTS

Data from the 12-month analysis of the Phase 2b TERMS study demonstrated a number of positive outcomes. Recently announced data in patients with at least one relapse in the 12 months prior to study entry (ARR  $\geq$  1; representing 86% of total study population) showed:

- More than 83% of Tovaxin-treated patients remained relapse free at one year
- 42% decrease in ARR following treatment compared to placebo, yielding an ARR=0.20
- 73% of Tovaxin-treated patients showed stabilization or improvement in MS disability including 16.5% with a sustained improvement in the Expanded Disability Status Scale (EDSS) of at least one full point.

All analyses of the TERMS study data have shown Tovaxin to be safe and well tolerated with no serious adverse events related to Tovaxin treatment. Mild injection site reaction was the most common adverse event.

Patients with more active disease (ARR  $\geq$  1) demonstrated especially promising responses to Tovaxin treatment in the TERMS trial, consistent with the data from the earlier Phase I/II clinical trials. This patient population, which represents significant market potential, is potentially the target population for Tovaxin's initial market entry. The FDA reviewing body for this program is the Office of Cellular and Gene Therapy within the Center for Biologics Evaluation and Research (CBER). Partnering discussions are taking place in parallel with further clinical planning and preparation.

## TECHNOLOGY PLATFORM

Opexa's initial disease targets are autoimmune diseases amenable to T-cell based therapies, such as MS. Opexa has developed a proprietary T-cell technology platform that allows for the production of patient-specific T-cell therapies that induce therapeutic immune responses to combat a variety of autoimmune diseases. Tovaxin, Opexa's lead therapeutic candidate for the treatment of MS is based on this platform.

## MANUFACTURING

Tovaxin is manufactured in-house at Opexa's cGMP (Good Manufacturing Practice) facility. For the procurement and shipping stages of the manufacturing process, Opexa leverages several of the well established worldwide logistics and distribution channels that have become standardized and regulated over the past 10-15 years by the FDA and practiced on a large scale across the industry by agencies such as the American Association of Blood Banks and the International Red Cross.

The manufacturing process has been shown to be very consistent and reproducible with attractive Cost of Goods at this stage of development.

## ABOUT MULTIPLE SCLEROSIS

- Multiple Sclerosis (MS) is a chronic, progressive inflammatory disease that attacks the myelin sheath that surrounds and insulates nerve fibers and facilitates the conduction of nerve impulse transmissions in the central nervous system (brain and spinal cord).
- In patients with MS, it is believed that activated T-cells (myelin reactive T-cells) infiltrate the central nervous system and trigger the attack on the myelin sheath leading to demyelination, while also inducing a damaging general pro-inflammatory immune response.
- Ultimately, the nerve fiber axons become damaged and cause impulse transmissions to diffuse into the tissue, resulting in the symptoms and disability associated with the disease.
- According to the National Multiple Sclerosis Society, MS affects approximately 2.5 million individuals worldwide. In 2008, industry experts estimated the global sales of MS therapeutics at approximately \$9 billion. Patient compliance remains a concern among many and new treatments with novel mechanisms of action could bring significant benefit to patients.

## 2009 OPEXA MILESTONES - ACHIEVED

- Announced positive results from 12-month analysis of Phase IIb TERMS study
- Signed exclusive stem cell agreement with Novartis potentially worth \$50M
- Published Tovaxin clinical data in *Journal of Clinical Immunology* and presented Tovaxin clinical data at American Academy of Neurology annual meeting
- Closed \$5.1M registered direct offering

## CORPORATE INFORMATION (As of 04/09/2010)

- Stock Symbol NASDAQ Capital Markets.....OPXA
- Stock Price.....\$2.41
- 52 Week Range.....\$0.31 - \$6.93
- Market Capitalization.....\$37 M
- Shares Outstanding..... 15.5 M
- Fiscal Year End.....12/31

## MANAGEMENT

- Neil Warma, President and Chief Executive Officer
- Jaye Thompson, Ph.D., SVP, Clinical Development
- Donna Rill, SVP, Operations
- Donald Healey, VP, Scientific Development

## CONTACT

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