Novelion Therapeutics Announces Presentation of Positive Metreleptin Data at American Diabetes Association Scientific Sessions

June 25, 2018

Data Show Leptin Replacement Therapy Sustainably Decreased Weight Over Time in Patients with Low Baseline Leptin Levels

Company plans to initiate Phase 2 proof of concept study in hypoleptinemic metabolic disorder (HMD) by year-end

VANCOUVER, British Columbia, June 25, 2018 (GLOBE NEWSWIRE) -- **Novelion Therapeutics Inc.** (NASDAQ:NVLN), a biopharmaceutical company dedicated to developing and commercializing therapies for individuals living with rare diseases, today announced that clinical data from a metreleptin study assessing weight loss in overweight and obese adults with low leptin levels will be featured in a poster presentation at the American Diabetes Association's (ADA) 78th Scientific Sessions, being held June 22-26, 2018 in Orlando.

The study, titled "Efficacy of Metreleptin for Weight Loss in Overweight and Obese Adults With Low Leptin Levels," was conducted in adults with low baseline leptin, defined as <16 ng/mL in females and <5 ng/mL in males, and Body Mass Index (BMI) levels between 27.5 and 38.0 kg/m^2. The study assessed weight levels in these patients (n=267) following once-daily subcutaneous doses of 10 mg metreleptin, 20 mg metreleptin, or placebo over 24 weeks.

Patients with low leptin who received the 10 mg dose (n=74) or 20 mg dose (n=72) of metreleptin showed greater decreases in weight from baseline over the 24-week period compared to patients who received placebo (n=111). In addition, patients treated with the 20 mg dose displayed statistically significant weight loss by weeks 8-10, regardless of whether they had the highest or the lowest baseline levels of leptin. Notably, weight loss was maintained across dose levels for the entire six-month duration of the study, and patients with the lowest leptin levels benefitted most from metreleptin treatment.

Metreleptin was generally well-tolerated at both doses. The most common adverse events (AEs) were injection-site reactions, headache, fatigue, gastrointestinal events, and upper respiratory tract infection. Most AEs were mild to moderate in severity.

"We are very pleased with the data indicating metreleptin's activity in a population of overweight and obese adults with low baseline leptin levels, who are in need of options to help them lose weight and control the metabolic complications associated with their disease," said Dr. Murray Stewart, Executive Vice President, Head of Research and Development for Novelion. "This is encouraging because it suggests that leptin replacement shows promise in the treatment of overweight and obese adult patients suffering from hypoleptinemic metabolic disorders who are characterized by very low leptin levels. Based on this data, we look forward to initiating a Phase 2 proof of concept study in HMD before year-end."

The study was conducted by Alex DePaoli, M.D., Alison Long, M.D., Ph.D., Gregory Fine, M.S., Murray Stewart, M.D., and Sir Stephen O'Rahilly FRS FRCP.

Presentation Details

Title: Efficacy of Metreleptin for Weight Loss in Overweight and Obese Adults With Low Leptin Levels
Presenter: Dr. Alex M. DePaoli, Sansum Diabetes Research Institute
Abstract Number: 296-LB
Date: Monday, June 25, 2018 at 12:00 PM

About Hypoleptinemic Metabolic Disorder (HMD)

HMD is a spectrum of metabolic sequelae secondary to underlying leptin deficiency. These patients differ from epidemic "lifestyle" diseases such as common obesity, type 2 diabetes mellitus/insulin resistance and dyslipidemia, in that low leptin may be the driver of the metabolic dysfunction. Metreleptin is not intended to treat common obesity or adult onset diabetes; however, it may be a potential treatment option in a small segment of this population, specifically those patients with very low leptin levels.

Metreleptin is not currently approved for use in HMD. Metreleptin is only approved by the U.S. Food and Drug Administration as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy. In addition, the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) has recommended the granting of marketing authorization, under exceptional circumstances, for metreleptin as an adjunct to diet, as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy (LD) patients with confirmed congenital generalized LD or acquired generalized LD in adults and children 2 years of age and above; or with confirmed familial partial LD or acquired partial LD, in adults and children 12 years of age and above for whom standard treatments have failed to achieve adequate metabolic control.

IMPORTANT SAFETY INFORMATION

Highlights of Safety Information from U.S. Prescribing Information for MYALEPT (metreleptin U.S. brand name)

WARNING: RISK OF ANTI-METRELEPTIN ANTIBODIES WITH NEUTRALIZING ACTIVITY AND RISK OF LYMPHOMA

See full prescribing information for complete boxed warning.

Anti-metreleptin antibodies with neutralizing activity have been identified in patients treated with MYALEPT. The consequences are not well characterized but could include inhibition of endogenous leptin action and/or loss of MYALEPT efficacy. Worsening metabolic control and/or severe infection have been reported. Test for anti-metreleptin antibodies with neutralizing activity in patients with severe infections or loss of efficacy during MYALEPT treatment.

T-cell lymphoma has been reported in patients with acquired generalized lipodystrophy, both treated and not treated with MYALEPT. Carefully consider the benefits and risks of MYALEPT treatment in patients with significant hematologic abnormalities and/or acquired generalized lipodystrophy.

MYALEPT is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the MYALEPT REMS PROGRAM.

CONTRAINDICATIONS

MYALEPT is contraindicated in general obesity not associated with congenital leptin deficiency and in patients with hypersensitivity to metreleptin.

WARNINGS AND PRECAUTIONS

Anti-metreleptin antibodies with neutralizing activity: Could inhibit endogenous leptin action and/or result in loss of MYALEPT efficacy. Test for neutralizing antibodies in patients with severe infections or loss of efficacy during MYALEPT treatment.

T-cell lymphoma: Carefully consider benefits and risks of treatment with MYALEPT in patients with significant hematologic abnormalities and/or acquired generalized lipodystrophy.

Hypoglycemia: A dose adjustment, including possible large reductions, of insulin or insulin secretagogue may be necessary. Closely monitor blood glucose in patients on concomitant insulin, or insulin secretagogue.

Autoimmunity: Autoimmune disorder progression has been observed in patients treated with MYALEPT. Carefully consider benefits and risks of MYLEPT treatment in patients with autoimmune disease.

Hypersensitivity reactions (e.g., anaphylaxis, urticaria or generalized rash) have been reported. Patients should promptly seek medical advice about discontinuation of MYALEPT if a hypersensitivity reaction occurs.

Benzyl Alcohol Toxicity: Preservative-free Water for Injection is recommended for use in neonates and infants.

ADVERSE REACTIONS

Most common adverse reactions (≥10%) in clinical trials were headache, hypoglycemia, decreased weight, and abdominal pain.

USE IN SPECIAL POPULATIONS

MYALEPT should be used during pregnancy only if the potential benefit justifies the potential risk to the fetus. No adequate and well-controlled studies have been conducted with metreleptin in pregnant women. Nursing Mothers should discontinue drug or nursing.

For additional information, please see the U.S. Prescribing Information including Box Warning for MYALEPT.

About Novelion Therapeutics

Novelion Therapeutics is a biopharmaceutical company dedicated to developing new standards of care for individuals living with rare diseases. Novelion has a rare disease product portfolio through its subsidiary, Aegerion Pharmaceuticals, Inc. The Company seeks to advance its portfolio of rare disease therapies by investing in science and clinical development.

Forward Looking Statements

Certain statements in this press release constitute "forward-looking statements" of Novelion within the meaning of applicable laws and regulations and constitute "forward-looking information" within the meaning of applicable securities laws. Any statements contained herein which do not describe historical facts, including statements regarding our plans to initiate a Phase 2 proof of concept study in HMD and our expectations and beliefs about leptin replacement showing promise in the treatment of overweight and obese adult patients suffering from hypoleptinemic metabolic disorders who are characterized by very low leptin levels, are forward-looking statements which involve risks and uncertainties that could cause actual results to differ materially from those discussed in such forward-looking statements. Such risks and uncertainties include, among others, that clinical data, such as the data included in the poster presentation, does not mean that metreleptin will have success in the planned Phase 2 proof of concept study in HMD or subsequent, later stage clinical trials and those risks identified in our filings with the U.S. Securities and Exchange Commission (the "SEC"), including under the heading "Risk Factors" in our Annual Report on Form 10-K filed on March 16, 2018, and subsequent filings, with the SEC, available on the SEC's website at www.sec.gov. Any such risks and uncertainties could materially and adversely affect our results of operations and cash flows, which would, in turn, have a significant and adverse impact on our stock price. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. Except as required by law, we undertake no obligation to update or revise the information contained in this press release, whether as a result of new information, future events or circumstances or otherwise.

Investors and others should note that we communicate with our investors and the public using our company website, www.novelion.com, including, but not limited to, company disclosures, investor presentations and FAQs, SEC filings, press releases, public conference call transcripts and webcast transcripts. The information that we post on this website could be deemed to be material information. As a result, we encourage investors, the media and others interested to review the information that we post there on a regular basis. The contents of our website shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

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