Novelion Therapeutics Announces Marketing Authorization for MYALEPTA® (metreleptin) in the European Union to Treat Generalized and Partial Lipodystrophy

July 31, 2018

MYALEPTA is the first approved therapy in Europe indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in lipodystrophy patients

VANCOUVER, British Columbia, July 31, 2018 (GLOBE NEWSWIRE) -- Novelion Therapeutics Inc. (NASDAQ:NVLN), a biopharmaceutical company dedicated to delivering new standards of care for people living with rare and underserved metabolic diseases, today announced that the European Commission (EC) has granted marketing authorization for MYALEPTA® (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 (Berardinelli-Seip syndrome) or acquired generalized LD (Lawrence syndrome) in adults and children 2 years of age and above; or with confirmed familial partial LD or acquired partial LD (Barraquer-Simons syndrome), in adults and children 12 years of age and above for whom standard treatments have failed to achieve adequate metabolic control.

MYALEPTA, a recombinant analog of human leptin, is the first and only licensed treatment in Europe indicated as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in LD patients. MYALEPTA was granted orphan drug designation in Europe in 2012. The EC's authorization follows the positive Committee for Medicinal Products for Human Use (CHMP) opinion on May 31, 2018.

Pricing and reimbursement negotiations with healthcare authorities have commenced and will be pursued on a country-by-country basis.

"The approval of MYALEPTA in the EU is a significant advancement for patients with generalized and partial lipodystrophy and a significant milestone for our company," said Jeff Hackman, interim chief executive officer and chief operating officer. "In preparation for launch, we have worked closely with regulatory authorities, patient organizations, and healthcare professionals across Europe to ensure that generalized LD and partial LD patients in need have access to MYALEPTA as quickly as possible, subject to securing pricing and reimbursement approvals where required. With the approval to treat both generalized and partial lipodystrophy patients, and a meaningful number of these patients identified or being treated via early access programs, we believe the EU represents a significant market opportunity for metreleptin."

Professor Stephen O'Rahilly , Professor of Clinical Biochemistry and Medicine at Addenbrooke's Hospital, Cambridge, said, "Lipodystrophy is a rare condition which, due to its complex nature, requires highly specialized treatment. Patients with lipodystrophy have previously relied on lifestyle changes and medications, like insulin injections to manage the condition's associated complications. Today's milestone marks a significant change in the way lipodystrophy is treated. For the first time, we will be able to offer patients in Europe a treatment option that addresses one of the fundamental aspects of the condition itself."

The clinical data supporting the authorization resulted from an open-label, single-arm study evaluating MYALEPT treatment in 48 patients. In accordance with the receipt of a marketing authorization under exceptional circumstances, certain risk minimization measures and post-authorization obligations will be required, including proposed studies which will further the understanding of MYALEPTA's impact on patients with generalized and partial lipodystrophy.

About Lipodystrophy

LD syndromes are ultra-rare disorders characterized by the irreversible loss of adipose tissue. Lipodystrophy can be genetic or acquired after an immune condition and occurs in childhood or adolescence. In patients with lipodystrophy syndromes, levels of leptin are often very low. Leptin is a naturally occurring hormone produced in adipose tissue and is an important regulator of energy homoeostasis, fat and glucose metabolism, reproductive capacity, and other diverse physiological functions.

With generalized lipodystrophy, the loss of fat affects the whole body. With partial lipodystrophy, the loss of fat typically occurs in the arms, legs, head, and trunk regions, while accumulation of fat may occur in other areas of the body, including the neck, face, and intra-abdominal regions. Generalized lipodystrophy affects approximately one person out of every one million, whereas partial lipodystrophy affects approximately three people in every one million.

MYALEPT, the U.S. brand name of metreleptin, received approval from the U.S Food and Drug Administration in February 2014, as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired GL. MYALEPT is not approved in the U.S. for partial lipodystrophy.

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 MYALEPT 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 global biopharmaceutical company dedicated to developing and commercializing therapies that deliver new standards of care for people living with rare and underserved metabolic diseases. Our goal is to develop and bring to market transformational therapies that have the potential to significantly change the treatment paradigm for patients affected by a variety of rare and metabolic diseases, including diseases associated with low leptin, such as low-leptin associated obesity. With a global footprint and an established commercial portfolio, including MYALEPT® (metreleptin) and JUXTAPID® (lomitapide), our business is supported by differentiated treatments that treat severe and rare diseases.

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 expectations for pricing and reimbursement approvals in the EU, access to MYALEPTA in the EU, the size of the market for MYALEPTA in the EU, the potential market size for MYALEPTA, converting early access patients to commercial patients and adding new MYALEPTA patients in the EU, 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, 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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