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MYALEPTA® (metreleptin) Receives Positive CHMP Opinion in Patients with Generalized and Partial Lipodystrophy

Europe represents potentially the largest market for metreleptin with expected authorization in both generalized and partial lipodystrophy

VANCOUVER, British Columbia, June 01, 2018 (GLOBE NEWSWIRE) -- Novelion Therapeutics Inc. (NASDAQ:NVLN), a biopharmaceutical company dedicated to developing new standards of care for individuals living with rare diseases, today announced that 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 MYALEPTA.

The CHMP recommends granting marketing authorization for MYALEPTA, 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.

The CHMP's positive opinion will now be reviewed by the European Commission (EC), which has the authority to grant approval of this indication. The Company anticipates a decision in mid-2018. If approved by the EC, MYALEPTA, which has orphan drug designation in Europe, will be the first and only licensed treatment in the EU as an adjunct to diet as a replacement therapy to treat the complications of leptin deficiency in LD patients.

Murray Stewart, DM, FCRP, Executive Vice President, Head of Research & Development said, "Lipodystrophy is a serious, rare condition, characterized by loss of fat tissue. In some patients it is inherited, while in others it may be acquired. This loss of fat tissue causes a deficit in the hormone leptin leading to possible metabolic complications such as hypertriglyceridemia, insulin resistance and hyperglycemia. This decision by the CHMP is welcomed by the physician community who is in need of treatment options for its generalized and partial lipodystrophy patients."

Chief Operating Officer Jeffrey Hackman said, "The positive CHMP opinion is a substantial milestone for our company, and even more importantly, for generalized and partial lipodystrophy patients. Europe is a significant market with a meaningful number of generalized and partial lipodystrophy patients already being treated through the metreleptin early access program. Assuming EC approval, we look forward to the opportunity to work with physicians, payers and patient organizations to convert these patients to commercial patients and to reach even more generalized and partial lipodystrophy patients in need."

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. 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.

MYALEPT, the U.S. brand name of metreleptin, is approved in the U.S. to treat generalized lipodystrophy, but is not approved to treat partial lipodystrophy.

FOR IMPORTANT SAFETY INFORMATION, INCLUDING BOXED WARNING IN THE U.S., PLEASE VISIT WWW.MYALEPT.COM

About Novelion Therapeutics

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

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 expectations and beliefs about the EC's approval decision, including timing, the size of the market for MYALEPTA in the EU, and 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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