March 30, 2017

Novelion Therapeutics' Subsidiary Announces New Data to be Presented at the Annual Meeting of the Endocrine Society

VANCOUVER, British Columbia and CAMBRIDGE, Mass., March 30, 2017 (GLOBE NEWSWIRE) -- Novelion Therapeutics Inc. (NASDAQ:NVLN) (TSX:NVLN), a biopharmaceutical company dedicated to developing new standards of care for individuals living with rare diseases, and its subsidiary today announced the presentation of data by academic researchers at ENDO 2017, the 99th annual meeting of the Endocrine Society, taking place in Orlando, April 1-4.

Among the data being presented are: research findings on MYALEPT® (metreleptin), a recombinant synthetic analog of the hormone leptin; clinical screening in Dunnigan Type 2 lipodystrophy; the association of T-cell lymphoma in acquired generalized lipodystrophy; genetic variants affecting the diagnosis of generalized lipodystrophy; metabolic disturbances in patients with lipodystrophy syndromes, and generalized lipodystrophy in high-prevalence areas. Data will also be presented on clinical screening to detect differences in diagnosis of metabolic syndrome vs. partial lipodystrophy.

There will also be an oral presentation at the congress highlighting an open-label study regarding lipodystrophy-associated NASH (nonalcoholic steatohepatitis, or fatty liver) treated with metreleptin.

"We continue to learn more about the role of low leptin in disease pathology. Scientific forums such as ENDO are important for enhancing our collective understanding of low-leptin mediated diseases such as lipodystrophy syndromes," said John Orloff, M.D., executive vice president, head of research and development for Novelion.

Leptin is a naturally occurring hormone and an important regulator of energy homeostasis, fat and glucose metabolism. Metreleptin is an analog of leptin made through recombinant DNA technology and metreleptin is approved in the United States as a replacement therapy, in addition to diet, to treat the complications of leptin deficiency in patients with generalized lipodystrophy.

In December 2016, the company submitted a marketing authorization application (MAA) to the European Medicines Agency (EMA) seeking approval for metreleptin as replacement therapy to treat complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy (GL) and in a subset of patients with partial lipodystrophy (PL).

The following abstracts will be presented at ENDO as an exchange of scientific and clinical information (all times, EDT):

Presentations/Abstracts at a Glance

Poster SAT 630; Saturday, April 1; 1 p.m.
Diagnosis of acquired generalized lipodystrophy in a patient with T-cell lymphoma: understanding the association. Presenter Nazanene H. Esfandiari, M.D.
Location: OCCC - West Hall B (EXPO Hall)

Oral Presentation: OR22-4; Saturday, April 1; 11:30 a.m.
Long-term effects of recombinant human leptin (metreleptin) on nocturnal LH secretion in lipodystrophy patients. Presenter Brent S. Abel, B.S.
Location: OCCC - W315

Poster MON 033; Monday, April 3; 1 p.m.
Total generalized congenital lipodystrophy (LCG) in a patient from high prevalence area. Presenter Gonzalo Francisco Miranda Sr.
Location: OCCC - West Hall B (EXPO Hall)

Poster MON 034; Monday, April 3; 1 p.m.
Clinical screening in Dunnigan Type 2 lipodystrophy kindred. Presenter Irla Andrade Dantas.
Location: OCCC - West Hall B (EXPO Hall)

Poster MON 035; Monday, April 3; 1 p.m.
When traditional measures are not sufficient to manage the severe metabolic disturbances in patients with Lipodystrophy

Syndromes. What's next? Presenter Marla E Sevilla Alsina, M.D.
Location: OCCC - West Hall B (EXPO Hall)

Poster MON 036; Monday, April 3; 1 p.m.
First described Chilean case of Mdpl syndrome with POLD1 mutation. Presenter Francisco Cordero, M.D.
Location: OCCC - West Hall B (EXPO Hall)

Poster MON 037; Monday, April 3; 1 p.m.
Pushing the envelope again: Setting the record straight on acquired generalized lipodystrophy. Presenter Shafaq Khairi, M.D.
Location: OCCC - West Hall B (EXPO Hall)

Oral Presentation: OR09-4; Tuesday, April 4; 9:45 a.m.
Efficacy of metreleptin therapy in the treatment of fatty liver disease associated with partial lipodystrophy. Open label study of 23 patients. Presenter Nevin Ajluni, M.D.
Location: OCCC - W308

U.S. INDICATIONS AND IMPORTANT SAFETY INFORMATION

MYALEPT® (metreleptin) for injection is a leptin analog indicated as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy. LIMITATIONS OF USE: The safety and effectiveness of MYALEPT for the treatment of complications of partial lipodystrophy or for the treatment of liver disease, including nonalcoholic steatohepatitis (NASH), have not been established. MYALEPT is not indicated for use in patients with HIV-related lipodystrophy. MYALEPT is not indicated for patients with metabolic disease, including diabetes mellitus and hypertriglyceridemia, without concurrent evidence of generalized lipodystrophy.

Anti-metreleptin antibodies with neutralizing activity have been identified in patients treated with MYALEPT. T-cell lymphoma has been reported in patients with acquired generalized lipodystrophy, both treated and not treated with MYALEPT. For more detailed information, please see additional Important Safety Information and the Prescribing Information, including boxed warning, for MYALEPT.

MYALEPT is available only through a restricted program called the MYALEPT REMS PROGRAM.

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 diversified commercial portfolio through its indirect subsidiary, Aegerion Pharmaceuticals, Inc. (Aegerion), which includes MYALEPT® and JUXTAPID® (lomitapide), and is also developing zuretinol acetate for the potential treatment of inherited retinal disease caused by underlying mutations in RPE65 or LRAT genes.

Forward-Looking Statements

Certain statements in this press release constitute "forward-looking statements" of Novelion and constitute "forward-looking information" within the meaning of applicable Canadian securities laws, including statements regarding the potential terms of exclusivity, the strength of our intellectual property portfolio and future sales of our products. Forward-looking statements are based on estimates and assumptions made by Novelion in light of current conditions and expected future developments, as well as other factors that Novelion believes are appropriate in the circumstances. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond our control, which could cause actual results to differ materially from those contemplated in these forward-looking statements. Many such risks, uncertainties and other factors are taken into account as part of our assumptions underlying these forward-looking statements and include, among others, the following: the risk that market acceptance of JUXTAPID® (lomitapide) and MYALEPT® (metreleptin), in the U.S. may not continue at the levels we expect, and may be lower outside the U.S., including in Brazil and Japan, than we expect; the risk that the conversion of prescriptions for JUXTAPID or MYALEPT into patients on therapy may be lower than we expect or the drop-out rate may be higher than we expect; the risk that private or government payers may refuse to reimburse Aegerion's or our products, or may impose onerous restrictions that hinder reimbursement or significantly limit or cap the price Aegerion or we charge or the number of reimbursed patients who receive products and the risk that Aegerion's and our patent portfolios and marketing and data exclusivity may not be as strong as we anticipate.

For additional disclosure regarding these and other risks we face, see the disclosure contained in the "Risk Factors" section of Aegerion's Quarterly Report on Form 10-Q filed on November 4, 2016, Novelion's Annual Report on Form 10-K filed on February 25, 2016 (and amended on April 29, 2016) and Quarterly Report on Form 10-Q filed on November 1, 2016 and

each company's other public filings with the U.S. Securities and Exchange Commission (SEC), available on the SEC's website at www.sec.gov. 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 calls transcripts and webcast transcripts. The information that we post on these websites 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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Primary Logo

Source: Novelion Therapeutics, Inc.