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Novelion Therapeutics Announces Presentation of Metreleptin Data at American Diabetes Association

VANCOUVER, British Columbia and CAMBRIDGE, Mass., June 12, 2017 (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 the presentation of data by researchers at the American Diabetes Association Scientific Sessions in San Diego.

The study, titled "Rate of Diabetes Remission in Generalized Lipodystrophy Patients Treated with Metreleptin," was a post-hoc analysis of a prospective, open-label study of 53 patients conducted at the National Institutes of Health. The study assessed the percent of generalized lipodystrophy (GL) patients with diabetes mellitus who achieved diabetes remission after investigational treatment with leptin replacement therapy (metreleptin).

In this post-hoc analysis, treatment with metreleptin allowed one-third (N=18) of patients to stop treatment with all antidiabetic medications, including insulin, for one year or more. The study also suggests that 17 percent of patients (N=9) achieved remission of diabetes.

Fifty-three patients with diabetes mellitus were assessed after one year of leptin replacement therapy for complete, partial or prolonged diabetes remission, defined as:

- Complete diabetes remission: A1c < 5.7% in the absence of anti-diabetic medications for one year or more; (baseline A1c mean 6.5%),
- Partial diabetes remission: A1c $\leq$ 6.4% in the absence antidiabetic medications for one year or more; (baseline A1c mean 9.3%),
- Prolonged remission: A1c < 5.7% in the absence of antidiabetic medications for five years or more; (baseline A1c mean 8.5%).

Among the 9 patients who achieved diabetes remission, 6 patients were on insulin and received an average dose of 440 units per day prior to metreleptin treatment.

Treatment-emergent adverse events occurring in greater than 10 percent of patients were: weight loss, abdominal pain, hypoglycemia, decreased appetite, and headache. Treatment-related serious adverse events occurred in 4 percent of patients.

"Severe insulin resistance adds significant complexity to controlling blood glucose among generalized lipodystrophy patients who have developed diabetes," said Rebecca Brown, M.D., Lasker Clinical Research Scholar in the NIH's National Institute of Diabetes and Digestive and Kidney Diseases. "We were pleased to observe that more than 15% of patients in the study were able come off all of their diabetes treatment medications for one year or more while maintaining normal blood glucose levels. Long-term follow up of these patients will help determine the impact of this therapy on complications of diabetes."

The post-hoc analysis was completed by Brown and Elif Oral, M.D., Associate Professor of Medicine, Michigan Medicine. Study drug was provided by Novelion Therapeutics' subsidiary.

GL is characterized by irreversible widespread loss of adipose tissue leading to low leptin levels. The absence of leptin results in the deposition of fat in the liver and muscle tissue and can lead to severe insulin resistance.

About Lipodystrophy

Lipodystrophy syndromes (LD) 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 GL, 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. Metreleptin is only approved in the U.S. to treat GL and is not approved to treat partial lipodystrophy.

U.S. INDICATION 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.

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

For more detailed information, please see full Prescribing Information, including boxed warning, for MYALEPT.

NIDDK DISCLAIMER
The research described here is conducted in part by the Intramural Research Program of the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) of the National Institutes of Health. The content in this release is the sole responsibility of the authors and does not necessarily represent the official views or imply endorsement of the National Institutes of Health.

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.

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