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Metreleptin and Lipodystrophy Data Presented at the International Meeting of Pediatric Endocrinology

VANCOUVER, British Columbia and CAMBRIDGE, Mass., Sept. 15, 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 two separate studies by independent researchers at the 10th International Meeting of Pediatric Endocrinology taking place in Washington, D.C., September 14-17.

The first study was a cross-section analysis examining key clinical characteristics of pediatric patients with confirmed familial partial lipodystrophy (FPL). Of the 13 pediatric patients 18 years or younger:

- All had at least one metabolic complication resulting from FPL, followed by diabetes (77 percent);
- The ages of onset were 10-18 years for NAFLD; 10-17 years for hyperlipidemia; and 11-17 years for diabetes;
- Among those with diabetes, 85 percent required diabetes medication and more than half were using insulin;
- Nearly one third of patients with hyperlipidemia were treated with lipid-lowering agents; and
- Of the 12 patients who had liver imaging performed, 11 had evidence of hepatosteatosis, or fatty liver.

The analysis was conducted in patients who were referred to the National Institutes of Health between 2003-2017 and presented by Rebecca Brown, M.D., Lasker Clinical Research Scholar, Section on Translational Diabetes and Metabolic Syndromes, Diabetes, Endocrinology, and Obesity Branch at the National Institutes of Health.

"FPL is a rare disease, and there is significant clinical variability among these patients, including differences in leptin levels, percent body fat and triglycerides, which makes accurate diagnosis of FPL challenging," said Brown. "Early diagnosis of pediatric FPL is important for management of these patients."

A second study examined the effect of metreleptin on liver volume among pediatric patients with generalized lipodystrophy (GL). Of the 13 patients assessed, all had an enlarged liver (hepatomegaly) at baseline.

For patients assessed within a year after initiating treatment with metreleptin, liver volume decreased by 25 percent. The mean duration of treatment was 9 months.
Among patients who had longer exposure to metreleptin (N=9) with a mean of 46 months, liver volume decreased by 34 percent.

The most commonly reported adverse events included gastrointestinal disorders, including abdominal pain and pancreatitis, and the two patients who experienced pancreatitis had reported episodes in their prior medical history.

This post-hoc analysis reviewed patients who participated in a prospective, open-label study conducted by the National Institutes of Health between 2000-2008.

"Fatty liver is a common complication among pediatric patients with GL," said Elif Oral, M.D., Associate Professor of Medicine, Michigan Medicine. "Treatment with metreleptin was associated with sustained reduction in liver volume during this study."

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 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. Metreleptin is approved in the U.S. to treat generalized lipodystrophy and is not approved to treat partial lipodystrophy.

About MYALEPT® (metreleptin) for injection
MYALEPT® (metreleptin) for injection is only approved in the United States and 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. 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 use in patients with metabolic disease, including diabetes mellitus and hypertriglyceridemia, without concurrent evidence of generalized lipodystrophy.

Highlights of Safety Information from U.S. Prescribing Information

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.

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 University of Michigan, Metabolism, Endocrinology and Diabetes Division

The University of Michigan, Metabolism, Endocrinology and Diabetes Division and Brehm Center for Diabetes collectively house a major referral for the study of lipodystrophy syndromes. For more information, contact Adam Neidert at (734) 615-0539.

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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