Akcea Announces Its Access and Distribution Strategy for TEGSEDI™ (inotersen)

October 5, 2018

Express Scripts' Accredo® selected as specialty pharmacy for its expertise in providing timely access and support services for patients living with rare diseases

BOSTON, Oct. 05, 2018 (GLOBE NEWSWIRE) -- Akcea Therapeutics, Inc. (NASDAQ: AKCA), an affiliate of Ionis Pharmaceuticals, Inc.(NASDAQ: IONS), announced today that they are working together with Accredo® specialty pharmacy, a subsidiary of Express Scripts (NASDAQ: ESRX), to distribute TEGSEDI™ (inotersen) subcutaneous injection for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults.

Approved by the U.S. Food and Drug Administration (FDA) today, TEGSEDI is the first and only subcutaneous RNA-targeting therapeutic offering patients an effective treatment for the polyneuropathy of hATTR amyloidosis. Subcutaneous delivery represents a significant new option for the patients, caregivers, and healthcare professionals in the U.S. and making TEGSEDI widely available to patients is now a critical next step. Akcea chose Express Scripts' Accredo because of its experience supporting the unique needs of rare disease communities and its proven track record for simplifying access to therapy.

"Accredo is entirely focused on supporting the needs of patients with rare and complex conditions like hATTR amyloidosis, and we are prepared and ready to serve patients and physicians by distributing TEGSEDI," said Bill Martin, Vice President, GM Accredo and CuraScript SD. "In addition, our Rare Disease Therapeutic Resource Center (TRC)®, provides patient education and proactive outreach to help improve health outcomes while reducing time to therapy and overall healthcare costs. Working together with Akcea, we are collectively united in our commitment to simplifying the patient journey and providing timely access to therapy."

Accredo's robust team of specialty clinicians, pharmacists and over 600 field-based nurses located throughout the U.S. will augment the AKCEA CONNECT™ team of nurse case managers to provide support and address the needs of the hATTR community.

"TEGSEDI is a weekly self-administered injection, that should reduce administration costs and drug wastage. By introducing competition into the marketplace this should drive down the total cost of care," said Steve Miller, MD, and Chief Medical Officer at Express Scripts.

"Akcea and Accredo share a common goal to simplify the patient experience while improving access to therapies that can transform lives, like TEGSEDI. This strategic collaboration and partnership with Express Scripts and Accredo allows us to consider innovative access strategies such as value and outcomes-based access arrangements in addition to other contracting strategies and risk-based approaches that address access and affordability concerns of patients, providers, and payers," said Kyle Jenne, head of U.S. commercial at Akcea Therapeutics.

ABOUT TEGSEDI ™ (INOTERSEN)

TEGSEDI was approved by the U.S. Food and Drug Administration (FDA) for the treatment of the polyneuropathy of hereditary transthyretin-mediated (hATTR) amyloidosis in adults. TEGSEDI ™ (inotersen), discovered and developed by Ionis Pharmaceuticals, is the world's first and only subcutaneous RNA-targeting drug designed to reduce the production of human transthyretin (TTR) protein. TEGSEDI also received marketing authorization in the European Union and Canada for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR).

The approval is based on data from the NEURO-TTR study that was a Phase 3 randomized (2:1), double-blind, placebo-controlled, 15-month, international study in 172 patients with hATTR amyloidosis with symptoms of polyneuropathy. In NEURO-TTR, TEGSEDI demonstrated significant benefit compared to placebo in measures of neuropathy and quality of life as measured by the modified Neuropathy Impairment Score +7 (mNIS+7) and in the Norfolk Quality of Life Questionnaire-Diabetic Neuropathy (Norfolk QOL-DN) total score. Patients treated with TEGSEDI experienced similar benefit regardless of subgroups such as age, sex, race, region, Neuropathy Impairment Score (NIS), Val30Met mutation status, and disease stage.

The approval is also based on data from the NEURO-TTR Open Label Extension (OLE) that is an ongoing study for patients who completed the NEURO-TTR study, designed to evaluate the long-term efficacy and safety of TEGSEDI.

For TEGSEDI's full prescribing information, please visit www.TEGSEDI.com.

IMPORTANT SAFETY INFORMATION

TEGSEDI can cause serious side effects including:

Low platelet counts (thrombocytopenia): TEGSEDI may cause the number of platelets in your blood to be reduced. This is a common side effect of TEGSEDI. When your platelet count is too low, your body cannot form clots. You could have serious bleeding that could lead to death. **Call your healthcare provider immediately if you have:**

- Unusual bruising or a rash of tiny reddish-purple spots, often on the lower legs
- Bleeding from skin cuts that does not stop or oozes
- Bleeding from your gums or nose
- Blood in your urine or stools
- Bleeding into the whites of your eyes
- Sudden severe headaches or neck stiffness
- Vomiting or coughing up blood
- Abnormal or heavy periods (menstrual bleeding)

Kidney inflammation (glomerulonephritis): Your kidneys may stop working properly. Glomerulonephritis can lead to severe kidney damage and kidney failure that need dialysis. **Call your healthcare provider immediately if you have:**

- Puffiness or swelling in your face, feet, or hands
- New onset or worsening shortness of breath and coughing
- Blood in your urine or brown urine
- Foamy urine (proteinuria)
- Passed less urine than usual

Because of the risk of serious bleeding caused by low platelet counts and because of the risk of kidney problems, TEGSEDI is available only through a restricted program called the TEGSEDI Risk Evaluation and Mitigation Strategy (REMS) Program. Talk to your healthcare provider about how to enroll in the TEGSEDI REMS Program.

Do not use TEGSEDI if you have:

- A platelet count that is low
- Had kidney inflammation (glomerulonephritis) caused by TEGSEDI
- Had an allergic reaction to inotersen or any of the ingredients in TEGSEDI. See the end of the Medication Guide for a complete list of ingredients in TEGSEDI

Before you start TEGSEDI, tell your healthcare provider about all of your health issues, including if you:

- Have or had bleeding problems
- Have or had kidney problems
- Are pregnant or plan to become pregnant. It is not known if TEGSEDI can harm your unborn baby
- Are breastfeeding or plan to breastfeed. It is not known if TEGSEDI can pass into your breast milk or harm your baby. Talk with your healthcare provider about the best way to feed your baby while you are taking TEGSEDI

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Especially tell your healthcare provider if you take vitamin A or beta-carotene supplements, blood thinners (anticoagulants), or drugs that affect blood clotting.

Required monitoring
Your healthcare provider will test your blood and urine to check your platelet counts and kidney and liver function before you start TEGSEDI. While you are receiving TEGSEDI, you will be monitored closely for symptoms, which includes checking your platelet counts every week (or more frequently as needed), kidney function every 2 weeks, and liver function every 4 months. If your healthcare provider has you stop taking TEGSEDI, you will need to continue to get your blood and urine tested for 8 more weeks after treatment.

TEGSEDI may cause serious side effects, including:

Stroke. TEGSEDI may cause a stroke. One person taking TEGSEDI had a stroke, which occurred within 2 days after the first dose. Get emergency help immediately if you have symptoms of stroke, including sudden numbness or weakness, especially on one side of the body; severe headache or neck pain; confusion; problems with vision, speech, or balance; droopy eyelids.

Inflammatory and immune system problems. Some people taking TEGSEDI had serious inflammatory and immune system problems. Symptoms of inflammatory and immune system problems included unexpected change in walking, weakness and spasms in legs, back pain, weight loss, headache, vomiting, and problems with speech.

Liver effects. TEGSEDI may cause liver problems. Your healthcare provider should do laboratory tests to check your liver before you start TEGSEDI and while you are using it. Tell your healthcare provider if you have symptoms that your liver may not be working right, which could include unexpected nausea and vomiting, stomach pain, being not hungry, yellowing of the skin, or having dark urine.

Allergic reactions. TEGSEDI may cause serious allergic reactions. These allergic reactions often occur within 2 hours after injecting TEGSEDI. Get emergency help immediately if you have any symptoms of a serious allergic reaction, including joint pain, chills, redness on palms of hands, muscle pain, chest pain, flushing, tremor or jerking movements, flu-like symptoms, high blood pressure, or difficulty swallowing.

Eye problems (low vitamin A levels). Treatment with TEGSEDI will lower the vitamin A levels in your blood. Your healthcare provider will tell you how much supplemental vitamin A to take every day; only take the amount they tell you to take. Call your healthcare provider if you get eye problems, such as having difficulty seeing at night or in low-lit areas (night blindness).

The most common side effects of TEGSEDI include injection site reactions (such as redness or pain at the injection site), nausea, headache, tiredness, low platelet counts (thrombocytopenia), and fever. These are not all of the possible side effects of TEGSEDI. Talk to your healthcare provider about any side affects you may be experiencing.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

Please see Medication Guide and full Prescribing Information, including boxed WARNING.

ABOUT AKCEA CONNECT™
Akcea Therapeutics is committed to ensuring that patients have access to TEGSEDI. Our patient assistance program called AKCEA CONNECT offers assistance to qualified patients at no cost. AKCEA CONNECT offers personalized and dedicated support to patients and their care teams through best-in-class services. The program focuses on sharing knowledge, enabling access and empowering patients in order to optimize care in rare diseases, improve patient outcomes, and enhance patients' overall experiences. For more information please visit www.AkceaConnect.com or call 1-866-AKCEATX (1-866-252-3289).

ABOUT AKCEA THERAPEUTICS
Akcea Therapeutics, Inc., an affiliate of Ionis Pharmaceuticals, Inc., is a biopharmaceutical company focused on developing and commercializing drugs to treat patients with serious and rare diseases. Akcea is advancing a mature pipeline of six novel drugs, including TEGSEDI ™ (inotersen), WAYLIVRA ™ (volanesorsen), AKCEA-APO(a)-L_{Rx}, AKCEA-ANGPTL3-L_{Rx}, AKCEA-APOCIII-L_{Rx}, and AKCEA-TTR-L_{Rx}, all with the potential to treat multiple diseases. All six drugs were discovered by and are being co-developed with Ionis, a leader in antisense therapeutics, and are based on Ionis' proprietary antisense technology. TEGSEDI is approved in the U.S., E.U. and Canada. WAYLIVRA is under regulatory review for the treatment of familial chylomicronemia syndrome, or FCS, and is currently in Phase 3 clinical development for the treatment of people with familial partial lipodystrophy, or FPL. Akcea is building the infrastructure to commercialize its drugs globally. Akcea is a global company headquartered in Cambridge, Massachusetts. Additional information about Akcea is available at www.akceatx.com.

ABOUT EXPRESS SCRIPTS
Express Scripts puts medicine within reach of tens of millions of people by aligning with plan sponsors, taking bold action and delivering patient-centered care to make better health more affordable and accessible.

Headquartered in St. Louis, Express Scripts provides integrated pharmacy benefit management services, including network-pharmacy claims processing home delivery pharmacy care, specialty pharmacy care, specialty benefit management, benefit-design consultation, drug utilization review, formulary management, and medical and drug data analysis services. Express Scripts also distributes a full range of biopharmaceutical products and provides extensive cost-management and patient-care services.

FORWARD-LOOKING STATEMENT
This press release includes forward-looking statements regarding the business of Akcea Therapeutics, Inc. and the therapeutic and commercial potential of TEGSEDI™ (inotersen). Any statement describing Akcea's goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties, particularly those inherent in the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. Akcea's forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements. Although Akcea's forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Akcea. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Akcea's programs are described in additional detail in Akcea's annual report on Form 10-K for the year ended December 31, 2017, and most recent Form 10-Q quarterly filing, which are on file with the SEC. Copies of this and other documents are available from the Company.

In this press release, unless the context requires otherwise, "Ionis," "Akcea," "Company," "Companies," "we," "our," and "us" refers to Ionis Pharmaceuticals and/or Akcea Therapeutics.

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