



## Akcea Therapeutics and PTC Therapeutics Collaborate to Commercialize Two Rare Disease Drugs in Latin America

August 2, 2018

- PTC in-licenses regional rights to TEGSEDI™ and WAYLIVRA™ from Akcea -

SOUTH PLAINFIELD, N.J. and CAMBRIDGE, Mass., Aug. 2, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), and Akcea Therapeutics, Inc. (NASDAQ: AKCA), an affiliate of Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), today announced a collaboration under which PTC will commercialize two of Akcea's rare disease drugs in Latin America (LATAM): TEGSEDI™ (inotersen) and WAYLIVRA™ (volanesorsen).

TEGSEDI has received marketing authorization approval from the European Commission (EC) for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR amyloidosis). TEGSEDI is also the subject of a pending new drug application in the U.S. and Canada. TEGSEDI has a PDUFA date of October 6, 2018. WAYLIVRA is under regulatory review in the U.S., Europe and Canada for the treatment of people with familial chylomicronemia syndrome (FCS). WAYLIVRA recently received a positive vote from the FDA's Division of Metabolism and Endocrinology Products Advisory Committee and has a PDUFA date of August 30, 2018. WAYLIVRA is also in clinical development for Familial Partial Lipodystrophy, or FPL.

"This collaboration reflects our strategic initiative to leverage PTC's global infrastructure and reflects our successes in bringing innovative drugs to patients in Latin America," said Stuart W. Peltz, Ph.D., chief executive officer, PTC Therapeutics. "We are excited to work with Akcea as part of the global launch and commercialization of TEGSEDI and WAYLIVRA."

"One of the key mutations that leads to hATTR amyloidosis occurs more frequently in individuals of Portuguese descent," stated Marcio Souza, chief operating officer, PTC Therapeutics, Inc. "There are approximately 6,000 patients with polyneuropathic hATTR amyloidosis in Latin America, making it a strategically important region for TEGSEDI. We are well positioned to file for registration in key Latin American countries in the short term."

"Our decision to partner with PTC to accelerate commercial access for patients in Latin America reflects our commitment to bringing TEGSEDI and WAYLIVRA to patients as rapidly as possible," said Paula Soteropoulos, chief executive officer, Akcea Therapeutics. "PTC's established rare disease team in Latin America has a proven record of success in patient identification, in physician and patient education and support programs and in efficiently obtaining market access. PTC's patient-focused approach for rare diseases aligns with ours and will be important as we bring TEGSEDI and WAYLIVRA to patients in this region."

### Key Transaction Details

Under the agreement, PTC will gain exclusive rights to TEGSEDI and WAYLIVRA in Latin America and certain Caribbean countries. PTC will pay Akcea an upfront licensing fee of \$18 million, \$12 million which is due on signing and \$6 million which will be paid on the earlier of FDA or EMA approval of WAYLIVRA. In addition, PTC will pay Akcea regulatory milestones in the PTC territory up to a total of \$8 million. Akcea is also eligible to receive royalties from PTC in the mid-twenty percent range on net sales of each drug in the PTC territory. PTC's obligation to pay Akcea royalties begins on the earlier of 12 months after the first commercial sale of a product in Brazil or the date that PTC recognizes revenue of at least \$10 million in LATAM. The collaboration will be governed by a joint steering committee with representation from both parties. Akcea gained the global rights to TEGSEDI and WAYLIVRA through licenses from Ionis. Milestone payments and royalties that Akcea receives from PTC for TEGSEDI will be split 60% to Ionis and 40% to Akcea. All WAYLIVRA milestone payments and royalties that Akcea receives from PTC will be split 50/50 with Ionis.

### About TEGSEDI™ (inotersen)

TEGSEDI™ (inotersen), a product of Ionis' proprietary antisense technology, is an antisense oligonucleotide (ASO) inhibitor of human transthyretin (TTR) production and was developed by Ionis. TEGSEDI is the world's first RNA-targeted therapeutic to treat patients with hATTR amyloidosis. TEGSEDI is approved in the E.U. for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR) and is currently under regulatory review in the U.S. and Canada. For important safety information for TEGSEDI, including method of administration, special warnings, drug interactions and adverse drug reactions, please see the European Summary of Product Characteristics (SmPC), available from the EMA website at [www.ema.europa.eu](http://www.ema.europa.eu).

### About hereditary transthyretin (hATTR) amyloidosis

hATTR amyloidosis is a progressive, systemic and fatal inherited disease caused by the abnormal formation of the TTR protein and aggregation of TTR amyloid deposits in various tissues and organs throughout the body, including in peripheral nerves, heart,

intestinal tract, eyes, kidneys, central nervous system, thyroid and bone marrow. The progressive accumulation of TTR amyloid deposits in these tissues and organs leads to sensory, motor and autonomic dysfunction often having debilitating effects on multiple aspects of a patient's life. Patients with hATTR amyloidosis often present with a mixed phenotype and experience overlapping symptoms of polyneuropathy and cardiomyopathy.

Ultimately, hATTR amyloidosis results in death within three to fifteen years of symptom onset. Therapeutic options for the treatment of patients with hATTR amyloidosis are limited and there are currently no disease-modifying drugs approved for the disease. There are an estimated 50,000 patients with hATTR amyloidosis worldwide. Additional information on hATTR amyloidosis, including a full list of organizations supporting the hATTR amyloidosis community worldwide, is available at [www.hattrchangethecourse.com](http://www.hattrchangethecourse.com).

#### **About WAYLIVRA™ (volanesorsen)**

WAYLIVRA™, a product of Ionis' proprietary antisense technology, is under regulatory review in the U.S., E.U. and Canada as a treatment for familial chylomicronemia syndrome (FCS). The U.S. and E.U. regulatory agencies have granted Orphan Drug Designation to WAYLIVRA for the treatment of FCS. If approved, WAYLIVRA would be the first and only therapy indicated for people with FCS.

#### **About Familial Chylomicronemia Syndrome (FCS)**

FCS is an ultra-rare disease caused by impaired function of the enzyme lipoprotein lipase (LPL) and characterized by severe hypertriglyceridemia (>880mg/dL) and a risk of unpredictable and potentially fatal acute pancreatitis. Because of limited LPL function, people with FCS cannot breakdown chylomicrons, lipoprotein particles that are 90% triglycerides. In addition to pancreatitis, FCS patients are at risk of chronic complications due to permanent organ damage. They can experience daily symptoms including abdominal pain, generalized fatigue and impaired cognitions that affect their ability to work. People with FCS also report major emotional and psychosocial effects including anxiety, social withdrawal, depression and brain fog. There is no effective therapy for FCS currently available. Additional information on FCS is available at [www.fcsfocus.com](http://www.fcsfocus.com), and through the FCS Foundation at <http://www.livingwithfcs.org> and the LPLD Alliance at [www.lpldalliance.org](http://www.lpldalliance.org). For a full list of organizations supporting the FCS community worldwide, please click here.

#### **About Akcea Therapeutics, Inc.**

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<sub>RX</sub>, AKCEA-ANGPTL3-L<sub>RX</sub>, AKCEA-APOCIII-L<sub>RX</sub>, and AKCEA-TTR-L<sub>RX</sub>, 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 E.U. for the treatment of stage 1 or stage 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR) and is currently under regulatory review in the U.S. and Canada. WAYLIVRA is under regulatory review in the U.S., E.U. and Canada 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](http://www.akceatx.com).

#### **About PTC Therapeutics**

PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder products and has a global commercial footprint. This success is the foundation that drives investment in a robust pipeline of transformative medicines and PTC's mission to provide access to best-in-class treatments for patients who have an unmet medical need.

#### **PTC'S and AKCEA'S Forward-Looking Statement**

This press release includes forward-looking statements regarding the business of PTC Therapeutics, Inc. and Akcea Therapeutics, Inc. and the therapeutic and commercial potential of TEGSEDI™ (inotersen) and WAYLIVRA™ (volanesorsen) and other products in development. Any statement describing PTC's or Akcea's goals, expectations, financial or other projections, intentions or beliefs, including the commercial potential of TEGSEDI, WAYLIVRA or other of PTC's or Akcea's drugs in development 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, including receiving any necessary regulatory approval, that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. PTC's and 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 PTC's and 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 PTC and Akcea, and PTC and Akcea do not undertake or plan to update or revise any such statements to reflect actual results or changes in goals, expectations, financial or other projections, intentions or beliefs occurring after the date of this press release except as required by law. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning PTC's and Akcea's programs are described in additional detail in PTC's and Akcea's quarterly reports on Form 10-Q and annual reports on Form 10-K, which are on file with the SEC, as well as in other filings with the SEC by PTC and Akcea. Copies of these and other documents are available from each company.

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

Ionis Pharmaceuticals™ is a trademark of Ionis Pharmaceuticals, Inc. Akcea Therapeutics™, TEGSED™ and WAYLIVRA™ are trademarks of Akcea Therapeutics, Inc.

**For More Information:**

**PTC Therapeutics, Inc.**

**Investors:**

Emily Hill

+ 1 (908) 912-9327

[ehill@ptcbio.com](mailto:ehill@ptcbio.com)

**Media:**

Jane Baj

+1 (908) 912-9167

[jbaj@ptcbio.com](mailto:jbaj@ptcbio.com)

**Akcea Therapeutics, Inc.**

**Investors:**

Kathleen Gallagher

Vice President of Communications and Investor Relations

617-207-8509

[kgallagher@akceatx.com](mailto:kgallagher@akceatx.com)

**Media:**

Lynn Granito

Berry & Company

T: 212 253-8881

[lgranito@berrypr.com](mailto:lgranito@berrypr.com)

 View original content: <http://www.prnewswire.com/news-releases/akcea-therapeutics-and-ptc-therapeutics-collaborate-to-commercialize-two-rare-disease-drugs-in-latin-america-300690950.html>

SOURCE PTC Therapeutics, Inc.; Akcea Therapeutics, Inc.