Progenics Pharmaceuticals Announces FDA Approval for AZEDRA® (iobenguane I 131) to Treat Unresectable, Locally Advanced or Metastatic Pheochromocytoma or Paraganglioma

July 31, 2018

The First FDA-Approved Treatment for These Rare, Life-Threatening Neuroendocrine Cancers in Patients Aged 12 and Older

Company to Host Conference Call Tomorrow at 8:00 AM ET

NEW YORK, July 30, 2018 (GLOBE NEWSWIRE) -- Progenics Pharmaceuticals, Inc. (Nasdaq:PGNX) today announced that the U.S. Food and Drug Administration (FDA) has approved the New Drug Application (NDA) for AZEDRA (iobenguane I 131) 555 MBq/mL injection for intravenous use. AZEDRA, a radiotherapeutic, is indicated for the treatment of adult and pediatric patients 12 years and older with iobenguane scan positive, unresectable, locally advanced or metastatic pheochromocytoma or paraganglioma who require systemic anticancer therapy. AZEDRA is the first and only approved therapy for this indication.

AZEDRA can cause serious side effects including risk from radiation exposure, bone marrow problems and other cancers (myelosuppression and secondary malignancies), thyroid problems (hypothyroidism), elevations in blood pressure, kidney problems (renal toxicity), respiratory problems (pneumonitis), pregnancy warning (embryo-fetal toxicity), and fertility problems. **Please see Important Safety Information below and Full Prescribing Information.**

Pheochromocytoma and paraganglioma are neuroendocrine cancers that arise from cells in and around the adrenal glands. Pheochromocytoma and paraganglioma tumors frequently secrete high levels of hormones that can lead to life-threatening high blood pressure, heart failure, and stroke in these patients. Metastatic pheochromocytoma and paraganglioma may result in unresectable disease with a poor prognosis, including a five-year survival rate as low as 12%[1]. Before AZEDRA's approval, there were no FDA-approved anti-tumor therapeutics for these cancers.

"AZEDRA is a true breakthrough in treating pheochromocytoma and paraganglioma delivering an effective anticancer therapy to these tumors," said Dr. Daniel Pryma, Associate Professor of Radiology & Radiation Oncology and Chief, Division of Nuclear Medicine & Clinical Molecular Imaging at the Perelman School of Medicine at the University of Pennsylvania, the trial's lead investigator. "With this innovative, rationally designed treatment, we finally have a therapeutic option that helps address patients' needs."

"As the first FDA approved therapy for unresectable, locally advanced or metastatic pheochromocytoma or paraganglioma who require systemic anticancer therapy, AZEDRA provides a new treatment option for physicians and their patients," said Mark Baker, Chief Executive Officer of Progenics. "AZEDRA has been shown to decrease the need for blood pressure medication and reduce tumor size in some patients. We are extremely grateful to the patients, their families and the investigators who participated in AZEDRA's clinical development program. We also thank those who have contributed to the development of AZEDRA over many years."

Emily Collins, President of the Pheo Para Alliance stated, "The FDA's approval of AZEDRA is welcome news to patients with pheochromocytoma and paraganglioma, who have an extremely limited number of treatment options available to them. The drug's Fast-Track status and Breakthrough Therapy designation by the FDA underscores the dire need for the development and expeditious review of diagnostic and therapeutic agents for pheo/para that, generally, don't get adequate prioritization despite the growing prevalence of these and other NET cancers globally."

The FDA approval of AZEDRA was based on data from a pivotal phase 2 open-label, multi-center trial that was conducted under a Special Protocol Assessment (SPA) with the FDA. The final results showed that 17 of the 68 evaluable patients (25%) experienced a 50% or greater reduction of all antihypertensive medication for at least 6 months, achieving the primary endpoint specified in the SPA. The study also showed favorable results from a key secondary endpoint evaluating the proportion of patients with overall tumor response as measured by Response Evaluation Criteria In Solid Tumors (RECIST). Overall tumor response was achieved in 15 of the patients studied (22%). Of these 15 patients, 53% experienced durable tumor responses lasting 6 months or longer. The most common severe (Grade 3-4) adverse reactions were decrease in a specific type of white blood cell (lymphopenia, 78%), decrease in another type of white blood cell (neutropenia, 59%), decrease in platelets which are involved in blood clotting (thrombocytopenia, 50%), fatigue (26%), decrease in red blood cells (anemia, 24%), decrease in blood clotting time (increased international normalized ratio, 18%), nausea (16%), dizziness (13%), high blood pressure (hypertension, 11%), and vomiting (10%). Twelve percent of patients discontinued treatment due to adverse reactions.

Investor Conference Call

Progenics will host a conference call tomorrow, July 31, 2018 at 8:00 AM Eastern Time to discuss the approval. The live and replayed webcast of the call will be available through the Company's website at www.progenics.com. To participate in the live call by phone, dial (877) 250-8889 (USA) or (720) 545-0001 (international) and enter the passcode 2787849. The replay of the call will be available for 90 days. This conference call will replace the quarterly call the Company usually hosts following its earnings announcement.

Approved Use:

AZEDRA® (iobenguane I 131) is indicated for the treatment of adult and pediatric patients 12 years and older with iobenguane scan positive, unresectable, locally advanced or metastatic pheochromocytoma or paraganglioma who require systemic anticancer therapy.

Important Safety Information

AZEDRA can cause serious side effects. If you experience these side effects, your health care provider may need to adjust or stop your treatment. You should always follow your health care provider's instructions. Serious side effects may include:

Radiation exposure: Treatment with AZEDRA will expose you to radiation which can contribute to your overall long-term radiation exposure. Overall radiation exposure is associated with an increased risk for cancer. Radiation risk is greater in children than in adults. You should stay well hydrated before, during, and after your treatment and urinate frequently. Your doctor will advise you on how to lessen exposure to people who may come into contact with you after AZEDRA treatment.

Bone marrow problems and other cancers: Treatment with AZEDRA may cause your blood cell counts to drop (myelosuppression). You may experience blood-related side effects such as low numbers of cells that are responsible for blood clotting (thrombocytopenia), low numbers of a type of white blood cells (neutropenia), and low red blood cells (anemia). Among the 88 patients who received a therapeutic dose of AZEDRA, 33% experienced Grade 4 thrombocytopenia, 16% experienced Grade 4 neutropenia, and 7% experienced Grade 4 anemia. Five percent of patients experienced febrile neutropenia (neutropenia with fever). People with low blood counts can develop serious infections. Your health care provider will routinely check your blood counts and tell you if they are too low. Tell your doctor if you experience any symptoms of low blood counts or infection, such as fever, chills, dizziness, shortness of breath, or increased bleeding or bruising. Your health care provider may need to adjust or stop your treatment accordingly. Other conditions that you may develop as a direct result of treatment with AZEDRA are blood and bone marrow cancers known as secondary myelodysplastic syndrome (MDS) and leukemia. MDS or acute leukemias were reported in 6.8% of the 88 patients who received a therapeutic dose of AZEDRA. The time to development of MDS or acute leukemia ranged from 12 months to 7 years. Two of the 88 patients developed other types of cancer.

Thyroid problems: Treatment with AZEDRA may increase your long-term risk of developing an underactive thyroid (hypothyroidism) or thyroid cancer. Hypothyroidism was reported in 3.4% of the 88 patients who received a therapeutic dose of AZEDRA. Take all thyroid-blocking agents as prescribed by your doctor to reduce the risk of these problems. You may need life-long monitoring for signs and symptoms of hypothyroidism.

Elevations in blood pressure: During or 24 hours following AZEDRA treatment, you may experience increases of blood pressure (hypertension) as a result of hormones released from your cancer. Eleven percent of the 88 patients who received a therapeutic dose of AZEDRA experienced a worsening of pre-existing hypertension. All changes in blood pressure occurred within the first 24 hours after treatment. No life-threatening hypertensive crises have been observed. Monitor blood pressure frequently during the first 24 hours after each therapeutic dose of AZEDRA. Tell your doctor if you experience any cardiac-related symptoms.

Kidney problems: Treatment with AZEDRA will expose your kidneys to radiation and may impair their ability to work as normal. In some cases, patients have experienced kidney failure after treatment with AZEDRA. Of the 88 patients who received a therapeutic dose of AZEDRA, 9% developed kidney failure or acute kidney injury, and 22% experienced a decrease in kidney function measured at 6 or 12 months. Your health care provider will monitor your kidneys after treatment using blood tests, particularly if you already have kidney impairment before treatment.

Respiratory problems: Treatment with AZEDRA may cause noninfectious lung inflammation (pneumonitis). Tell your doctor if you experience shortness of breath, difficulty breathing, or cough.

Pregnancy warning: Before treatment with AZEDRA, tell your doctor if you are pregnant or plan to become pregnant. Exposure to radiation from treatment with AZEDRA can harm your unborn baby. Use an effective method of birth control during treatment with AZEDRA and for 7 months (for females) and 4 months (for males) after your final dose. Do not breastfeed during treatment with AZEDRA and for 80 days after your final dose.

Fertility problems: Treatment with AZEDRA may cause infertility due to radiation absorbed by your testes or ovaries over the treatment period that is within the range of exposure where temporary or permanent infertility may be expected.

The most common and most serious side effects of AZEDRA include decreased blood cell counts, nausea, vomiting and fatigue. These are not all the possible side effects of AZEDRA. For more information, ask your health care provider.

Drugs that reduce catecholamine uptake or that deplete catecholamine stores may interact with AZEDRA and may affect how well it works. These drugs were not permitted in the clinical trials. Tell your doctor before starting any medication, including over the counter medications, herbal or dietary supplements.

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 full Prescribing Information for AZEDRA.
Distributed by: Progenics Pharmaceuticals, Inc., NY 10007

Reference:
AZEDRA® prescribing information. New York, NY: Progenics Pharmaceuticals, Inc.; 07 2018.

About PROGENICS

Progenics develops innovative medicines and other technologies to target and treat cancer, including: 1) therapeutic agents designed to treat cancer (AZEDRA, PSMA TTC and 1095), 2) PSMA-targeted imaging agents for prostate cancer (1404 and PyL™), and 3) imaging analysis technology. Progenics has two commercial products, RELISTOR® (methylnaltrexone bromide) for opioid-induced constipation, which is partnered with Valeant Pharmaceuticals International, Inc.; and AZEDRA, for the treatment of unresectable, locally advanced or metastatic pheochromocytoma or paraganglioma (rare neuroendocrine tumors of neural crest origin) who require systemic anticancer therapy.

This press release contains "forward-looking statements" regarding future events. Statements contained in this communication that refer to Progenics' estimated or anticipated future results or other non-historical facts are forward-looking statements that reflect Progenics' current perspective of existing trends and information as of the date of this communication. Forward looking statements are generally accompanied by words such as "anticipate," "believe," "plan," "could," "should," "estimate," "expect," "forecast," "outlook," "guidance," "intend," "may," "might," "will," "possible," "potential," "predict," "project," or other similar words, phrases or expressions. Such statements are predictions only, and are subject to risks and uncertainties that could cause actual events or results to differ materially. These risks and uncertainties include, among others, market acceptance for approved products; the risk that the planned commercial launch of AZEDRA may not meet revenue and income expectations; the cost, timing and unpredictability of results of clinical trials and other development activities and collaborations, such as the Phase 3 clinical program for 1404; our ability to successfully develop and commercialize the products of EXINI Diagnostics AB; the effectiveness of the efforts of our partners to market and sell products on which we collaborate and the royalty revenue generated thereby; generic and other competition; the possible impairment of, inability to

obtain and costs of obtaining intellectual property rights; possible product safety or efficacy concerns, general business, financial, regulatory and accounting matters, litigation and other risks. More information concerning Progenics and such risks and uncertainties is available on its website, and in its press releases and reports it files with the U.S. Securities and Exchange Commission, including those risk factors included in its Annual Report on Form 10-K for the fiscal year ended December 31, 2017, as updated in its subsequent Quarterly Reports on Form 10-Q. Progenics is providing the information in this press release as of its date and, except as expressly required by law, Progenics disclaims any intent or obligation to update or revise any forward-looking statements, whether as a result of new information, future events or circumstances or otherwise.

Additional information concerning Progenics and its business may be available in press releases or other public announcements and public filings made after this release. For more information, please visit www.progenics.com. Information on or accessed through our website or social media sites is not included in the company's SEC filings.

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[1]*Park, J., Korean J Urology 2011;52:241-246.*
[2]*AZEDRA Prescribing Information. New York, NY: Progenics Pharmaceuticals, Inc.; July 30, 2018*

Primary Logo

Source: Progenics Pharmaceuticals Inc.