



Progenics Announces Updated Survival Data for AZEDRA® (iobenguane I 131) in Advanced Pheochromocytoma and Paraganglioma

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*- Long-Term Follow Up Data from Pivotal Study Presented at ASCO -
- Patients Treated with AZEDRA Show Overall Survival of 73.1% at Two Years and 44.2% at Four Years -*

NEW YORK, June 03, 2019 (GLOBE NEWSWIRE) -- Progenics Pharmaceuticals, Inc. (NASDAQ:PGNX), an oncology company developing innovative targeted medicines and artificial intelligence to find, fight and follow cancer, announced today that long-term follow up data from its pivotal Phase 2 study of AZEDRA (iobenguane I 131) were presented at the 2019 American Society of Clinical Oncology (ASCO) in Chicago.

The data were presented in a poster presentation titled, "Long-term survival and safety from a multi-center, open-label, pivotal phase 2 study of iobenguane I 131 in patients (Pts) with unresectable, locally advanced or metastatic pheochromocytoma or paraganglioma (PPGL)."

"The maturing data set further confirms the long-term efficacy and potential benefits of AZEDRA in patients with iobenguane scan positive, unresectable, locally advanced or metastatic PPGL, and highlights the potential of this treatment option for patients with these rare neuroendocrine tumors," stated Richard B. Noto, Director of the Division of Nuclear Medicine at Rhode Island Hospital. "The 5-year long-term survival rate of 38.3% is particularly impressive and encouraging given that the rate observed in the literature can be as low as 12%.¹ The data help establish AZEDRA as an efficacious and well-tolerated treatment option in this patient population."

The following data were included as part of the presentation:

- As of Jan 25, 2019, median overall survival time (OS) for all patients was 41.1 months (95% CI 31.1, 91.2).
- Median OS was 17.5 months (95% CI 4.0, 31.5) and 48.7 months (95% CI 33.2, 91.2) in patients receiving one and two doses, respectively.
- The 12-month OS rate was 91%.
- A tail of survival was observed, with OS of 73.1% at two years and 44.2% at four years.
- The most common treatment-emergent adverse events were consistent with expected radiation-related risks of hematologic toxicities, nausea/vomiting, fatigue and dizziness.

"This pivotal study, the largest and most rigorous ever conducted in advanced PPGL, has continued to deliver compelling results for AZEDRA," stated Asha Das, M.D., Chief Medical Officer of Progenics. "We are proud to offer AZEDRA as the first approved treatment option for patients with advanced or metastatic pheochromocytoma and paraganglioma, and we look forward to exploring the potential for AZEDRA in other MIBG-avid tumor types."

Indication

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

Warnings and Precautions:

- **Risk from Radiation Exposure:** AZEDRA contributes to a patient's overall long-term radiation exposure. Long-term cumulative radiation exposure is associated with an increased risk for cancer. These risks of radiation associated with the use of AZEDRA are greater in pediatric patients than in adults. Minimize radiation exposure to patients, medical personnel, and household contacts during and after treatment with AZEDRA consistent with institutional good radiation safety practices and patient management procedures.
- **Myelosuppression:** 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. Monitor blood cell counts weekly for up to 12 weeks or until levels return to baseline or the normal range. Withhold and dose reduce AZEDRA as recommended in the prescribing information based on severity of the cytopenia.
- **Secondary myelodysplastic syndrome, leukemia, and other malignancies:** Myelodysplastic syndrome (MDS) and 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 a non-hematological malignancy.
- **Hypothyroidism:** Hypothyroidism was reported in 3.4% of the 88 patients who received a therapeutic dose of AZEDRA. Initiate thyroid-blocking medications starting at least 1 day before and continuing for 10 days after each AZEDRA dose to reduce the risk of hypothyroidism or thyroid neoplasia. Evaluate for clinical evidence of hypothyroidism and measure thyroid-stimulating hormone (TSH) levels prior to initiating AZEDRA and annually thereafter.
- **Elevations in blood pressure:** Eleven percent of the 88 patients who received a therapeutic dose of AZEDRA

experienced a worsening of pre-existing hypertension defined as an increase in systolic blood pressure to ≥ 160 mmHg with an increase of 20 mmHg or an increase in diastolic blood pressure to ≥ 100 mmHg with an increase of 10 mmHg. All changes in blood pressure occurred within the first 24 hours post infusion. Monitor blood pressure frequently during the first 24 hours after each therapeutic dose of AZEDRA.

- **Renal toxicity:** Of the 88 patients who received a therapeutic dose of AZEDRA, 7% developed renal failure or acute kidney injury and 22% demonstrated a clinically significant decrease in glomerular filtration rate (GFR) measured at 6 or 12 months. Monitor renal function during and after treatment with AZEDRA. Patients with baseline renal impairment may be at greater risk of toxicity; perform more frequent assessments of renal function in patients with mild or moderate impairment. AZEDRA has not been studied in patients with severe renal impairment.
- **Pneumonitis:** Fatal pneumonitis occurred 9 weeks after a single dose in one patient in the expanded access program. Monitor patients for signs and symptoms of pneumonitis and treat appropriately.
- **Embryo-fetal toxicity:** Based on its mechanism of action, AZEDRA can cause fetal harm. Verify pregnancy status in females of reproductive potential prior to initiating AZEDRA. Advise females and males of reproductive potential of the potential risk to a fetus and to use effective contraception during treatment with AZEDRA and for 7 months after the final dose. Advise males with female partners of reproductive potential to use effective contraception during treatment and for 4 months after the final dose.
- **Risk of infertility:** Radiation exposure associated with AZEDRA may cause infertility in males and females. Radiation absorbed by testes and ovaries from the recommended cumulative dose of AZEDRA is within the range where temporary or permanent infertility can be expected following external beam radiotherapy.

Adverse Reactions:

The most common severe (Grade 3–4) adverse reactions observed in AZEDRA clinical trials ($\geq 10\%$) were lymphopenia (78%), neutropenia (59%), thrombocytopenia (50%), fatigue (26%), anemia (24%), increased international normalized ratio (18%), nausea (16%), dizziness (13%), hypertension (11%), and vomiting (10%). Twelve percent of patients discontinued treatment due to adverse reactions (thrombocytopenia, anemia, lymphopenia, nausea and vomiting, multiple hematologic adverse reactions).

Drug Interactions:

Based on the mechanism of action of iobenguane, drugs that reduce catecholamine uptake or that deplete catecholamine stores may interfere with iobenguane uptake into cells and therefore interfere with dosimetry calculations or the efficacy of AZEDRA. These drugs were not permitted in clinical trials that assessed the safety and efficacy of AZEDRA. Discontinue the drugs listed in the prescribing information for at least 5 half-lives before administration of either the dosimetry dose or a therapeutic dose of AZEDRA. Do not administer these drugs until at least 7 days after each AZEDRA dose.

For important risk and use information about AZEDRA, please see Full Prescribing Information.

To report suspected adverse reactions, contact Progenics Pharmaceuticals, Inc. at 844-668-3950 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

About PROGENICS

Progenics is an oncology company focused on the development and commercialization of innovative targeted medicines and artificial intelligence to find, fight and follow cancer, including: therapeutic agents designed to treat cancer (AZEDRA[®], 1095, and PSMA TTC); prostate-specific membrane antigen ("PSMA") targeted imaging agent for prostate cancer (PyL[™]); and imaging analysis technology (aBSI and PSMA AI). Progenics has two commercial products, AZEDRA, for the treatment of patients with unresectable, locally advanced or metastatic pheochromocytoma or paraganglioma (rare neuroendocrine tumors of neural crest origin) who require systemic anticancer therapy; and RELISTOR[®] (methylnaltrexone bromide) for the treatment of opioid-induced constipation, which is partnered with Bausch Health Companies Inc.

This press release contains projections and other "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 generally will be 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 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; the unpredictability of the duration and results of regulatory review of New Drug Applications (NDA) and Investigational NDAs; the inherent uncertainty of outcomes in the intellectual property disputes such as the dispute with the University of Heidelberg regarding PSMA-617; our ability to successfully develop and commercialize products that incorporate licensed intellectual property; 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 year ended December 31, 2018, 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.

¹Park, J., Korean J Urology 2011;52:241-246.

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