# Progenics Pharmaceuticals Announces Presentation of AZEDRA® (iobenguane I 131) Biochemical Tumor Marker Data at the 2018 Endocrine Society (ENDO) Annual Meeting

March 19, 2018

**Significant correlation between overall tumor biomarker response and achievement of the primary and secondary endpoints in pivotal study of AZEDRA**

NEW YORK, March 19, 2018 (GLOBE NEWSWIRE) -- Progenics Pharmaceuticals, Inc. (Nasdaq:PGNX), an oncology company developing innovative medicines and other products for targeting and treating cancer, announced that biochemical tumor marker data from the Company's pivotal Phase 2 trial of its targeted, high-specific-activity radiotherapeutic candidate, AZEDRA® (iobenguane I 131), in patients with malignant, recurrent, and/or unresectable pheochromocytoma and paraganglioma (pheo/para) were presented at the Endocrine Society (ENDO) Annual Meeting in Chicago, Illinois.

"In this pivotal study of AZEDRA in pheo and para patients, the overall tumor biomarker response correlated significantly with responder status with both the primary and secondary endpoints," said Dr. Camilo Jimenez, Associate Professor, Department of Endocrine Neoplasia and Hormonal Disorders at the University of Texas M. D. Anderson Cancer Center. "AZEDRA has already been shown to have a significant positive impact on the cardiovascular symptoms associated with pheochromocytoma and paraganglioma. It has also demonstrated objective antitumor effects as measured by Response Evaluation Criteria In Solid Tumors (RECIST) criteria. The biochemical tumor marker data presented today provides further evidence of AZEDRA's potential to offer a meaningful treatment option for patients with these life-threatening tumors."

Dr. Jimenez reviewed the data in an oral presentation titled, "AZEDRA® (iobenguane I 131) in Patients with Metastatic and/or Recurrent and/or Unresectable Pheochromocytoma or Paraganglioma: Biochemical Tumor Marker Results of a Multicenter, Open-Label Pivotal Phase 2 Study."

Tumor biomarkers were analyzed in patients who had individual tumor biomarkers above 1.5x the upper limit of normal (ULN) at baseline. The overall tumor biomarker response correlated with responder status for those who met the study's primary endpoint ($r = 0.31$, $p = 0.011$) and objective tumor response ($r=0.35$, $p=0.006$). The biomarkers evaluated were Chromogranin A (CgA, serum), Normetanephrine (NM, serum and urine) and Norepinephrine (NE, serum and urine). At 12 months following the first therapeutic dose of AZEDRA, urine and serum NE showed best response (CR/PR) rates of 42.1% and 31.0%, respectively, and urine and serum NM responder rates of 36.0% and 44.0%, respectively. In addition, serum CgA showed best response rate of 67.9% at 12 months.

"The compelling results from this pivotal trial formed the basis of our New Drug Application for AZEDRA, which was accepted for review by the FDA at the end of 2017," said Mark Baker, Chief Executive Officer of Progenics. "We are eagerly awaiting the FDA's action date at the end of April for our innovative radiopharmaceutical treatment. AZEDRA has the potential to be the first FDA-approved therapy to address the high unmet need of patients with malignant pheo and para."

The pivotal phase 2 open-label, multi-center trial was conducted under a Special Protocol Assessment (SPA) with the U.S. Food and Drug Administration (FDA). The trial met the primary endpoint evaluating the proportion of pheochromocytoma and paraganglioma patients who achieved a 50% or greater reduction of all antihypertensive medication for at least six months, and showed favorable results from a key secondary endpoint evaluating the proportion of patients with overall tumor response as measured by RECIST. AZEDRA was also shown to be safe and generally well tolerated.

**About AZEDRA®**

AZEDRA (iobenguane I 131) is a high-specific-activity radiotherapeutic product candidate in development as a treatment for malignant, recurrent, or unresectable pheochromocytoma and paraganglioma, which are rare neuroendocrine tumors of neural crest origin. AZEDRA is a substrate for norepinephrine reuptake transporter which is highly expressed on the cell surface of neuroendocrine tumors. AZEDRA has been granted Orphan Drug designation, Fast Track status, and Breakthrough Therapy designation in the U.S. Under a SPA agreement with the FDA, a Phase 2 pivotal study has been completed in patients with malignant, recurrent, or unresectable pheochromocytoma and paraganglioma. The FDA granted Priority Review of Progenics' New Drug Application and has set an action date of April 30, 2018 under the Prescription Drug User Fee Act. There are currently no FDA-approved therapies for the treatment of these ultra-rare diseases.

**About Pheochromocytoma and Paraganglioma**

Pheochromocytoma and paraganglioma are rare neuroendocrine tumors that arise from cells of the autonomic nervous system. Pheochromocytoma forms in the adrenal medulla, whereas paragangliomas form outside the adrenal gland. Standard treatment options for these tumors include surgery, palliative therapy and symptom management. Pheochromocytoma and paraganglioma tumors frequently secrete high levels of hormones that can lead to life-threatening hypertension, heart failure, and stroke in these patients. Malignant and recurrent pheochromocytoma and paraganglioma may result in unresectable disease with a poor prognosis, representing a significant management challenge with very limited treatment options and no approved anti-tumor therapies.

**About Progenics**

Progenics develops innovative medicines and other technologies to target and treat cancer. Progenics' pipeline includes: 1) therapeutic agents designed to precisely target cancer (AZEDRA®, 1095, and PSMA TTC), 2) PSMA-targeted imaging agents for prostate cancer (1404 and PyL™), and 3) imaging analysis technology. Progenics' first commercial product, RELISTOR® (methylnaltrexone bromide) for opioid-induced constipation, is partnered with Valeant Pharmaceuticals International, Inc.

*This press release may contain 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, 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 unpredictability of the duration and results of regulatory review of New Drug Applications (NDA) and Investigational NDAs, including our NDA for AZEDRA and related inspections of Progenics' and its contract manufacturing organizations' facilities and other sites and other requirements that will need to be met before any approval is obtained; market acceptance for approved products; 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 annual period ended December 31, 2017. 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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