AADi Launches Its Phase 2 Registration Trial for ABI-009, a Targeted mTOR Inhibitor, for Patients with Advanced PEComa, a Rare Form of Sarcoma

Study to be conducted at major cancer centers across the US

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PACIFIC PALISADES, Calif.--(BUSINESS WIRE)--AADi, LLC, a clinical stage biopharmaceutical company focused on treating diseases driven by mTOR activation, today announced the initiation of its registration trial for advanced Perivascular Epithelioid Cell tumors (PEComa) with ABI-009, its targeted albumin-bound mTOR inhibitor. ABI-009 is the nanoparticle albumin-bound (nab®) version of the mTOR inhibitor sirolimus or rapamycin and leverages the same technology of the nab® platform that is behind the success of ABRAXANE® (paclitaxel protein-bound particles for injectable suspension) (albumin-bound). ABI-009 was licensed to AADi in 2014 by Celgene Corporation (NASDAQ:CELG). AADi plans to develop ABI-009 initially in oncology and cardiovascular indications.

“We are extremely excited to launch our phase 2 registration trial in this very rare disease where activation of the mTOR pathway is known to play an important role. ABI-009 has a unique pharmacology amongst the mTOR inhibitors which we believe will translate into better outcomes for the patients,” said Neil Desai, Ph.D., Founder and Chief Executive Officer of AADi.

In August 2015, AADi received agreement from the FDA on the design of its phase 2 registration study for the treatment of advanced (locally advanced or metastatic) PEComa with ABI-009. The study will enroll approximately 35 patients and the primary endpoint for the study is the overall response rate.

“This is the first clinical trial focused on this rare form of sarcoma where the biology driving the disease matches the pharmacology and mechanism of action of the drug,” said Dr. Andrew Wagner, Principal Investigator of the study, Senior Physician, Center for Sarcoma and Bone Oncology and Assistant Professor of Medicine, Dana Farber Cancer Institute and Harvard Medical School. Dr. Wagner was the first to describe the relevance of mTOR inhibitors in the treatment of malignant PEComa due to the pathogenic activation of the mTOR pathway in these tumors. “It is important that we continue to search for new treatments for rare diseases and to scientifically test their safety and activity.”

A phase 1 trial for ABI-009 was completed in patients with advanced non-hematologic malignancies in which the drug was well tolerated with evidence of activity in heavily pretreated patients. AADi also has an ongoing phase 1/2 trial of ABI-009 for the treatment of non-muscle invasive bladder cancer that is being conducted as part of a Fast-Track STTR grant awarded to AADi from the National Cancer Institute (NCI) of the National Institutes of Health (NIH). In addition to these studies, AADi plans to initiate a phase 2 trial in patients with various solid tumors that have been selected for mTOR pathway activations.

As part of its focus in cardiovascular disease, AADi plans to initiate clinical studies with ABI-009 in the treatment of pulmonary arterial hypertension, a rare, progressive and debilitating disease that is highly dependent on mTOR activation, and which occurs due to abnormal constriction of the arteries in the lungs resulting in shortness of breath and increasing stress on the heart or heart failure.

AADi is currently raising capital to fund its research programs through the end of phase 2 clinical studies.

About PEComa

Perivascular epithelioid cell tumors (PEComa) are a rare subset of soft tissue sarcomas recently recognized as a distinct entity by the World Health Organization in 2002 and are composed of histologically and immunohistochemically distinctive epithelioid cells. PEComas appear to arise most commonly at visceral (especially gastrointestinal and uterine), retroperitoneal, and abdominopelvic sites. Most PEComas are benign, but there is a subset of PEComas, i.e., advanced malignant PEComas [1], for which there are currently no approved therapies and for which there are only a few case reports in the literature. The prognosis for this patient subset is poor, with a median survival estimated to be 12-17 months following diagnosis of advanced disease [2]. Overactivation of the mTOR pathway has been reported in malignant PEComa, and mTOR inhibitors have shown anecdotal efficacy in this indication in case reports or retrospective studies [1].

About AADi and ABI-009

AADi, LLC is a clinical stage biopharmaceutical company led by Dr. Neil Desai, an inventor of ABRAXANE ®, ABI-009 and the nab® technology platform. AADi's lead product is ABI-009, a nanoparticle albumin-bound mTOR inhibitor based on sirolimus or rapamycin, also known as nab-rapamycin. AADi aims to develop the full potential of the albumin-bound mTOR inhibitor in diseases that are driven by mTOR activation and where the mTOR inhibitors have not or cannot be effectively exploited due to problems of effective drug delivery, safety or effective targeting to the disease site. In a phase 1 study ABI-009 was well tolerated at intravenous doses significantly higher than other mTOR inhibitors and its pharmacokinetic profile, with high Cmax and AUC, is very different from the available mTOR inhibitors. In animal models, ABI-009 has shown greater antitumor efficacy than the oral mTOR inhibitors at the same dose. Similar to Abraxane, the albumin-bound rapamycin is expected to have high tumor penetration by taking advantage of mechanisms of albumin uptake in tumors and other areas with tissue remodeling or inflammation.

1. Wagner AJ *et al.* (2010). *J Clin Oncol* **28**, 835-840.
2. Bleeker JS et al(2012). *Sarcoma* 2012, 541626

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