Yaupon Announces Positive Results from Pivotal Study of Its Proprietary Topical Mechlorethamine Gel; A Potential Treatment for Early-Stage Cutaneous T-cell Lymphoma

- Data on this largest study ever in CTCL to be presented at First World Congress of Cutaneous Lymphomas -

- Yaupon Plans to File NDA in 2010 -

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RADNOR, Pa.--(BUSINESS WIRE)--Yaupon Therapeutics, a privately held specialty pharmaceutical company, today announced positive top-line results from the pivotal multi-center study of its lead product candidate, a proprietary topical mechlorethamine gel for the treatment of early-stage cutaneous T-cell lymphoma (CTCL – stages I-IIa). The top-line data show that the study, which was conducted under a Special Protocol Assessment (SPA), achieved its primary and secondary endpoints. The top-line data are being presented for the first time today at the First World Congress of Cutaneous Lymphomas in Chicago, IL, by Stuart R. Lessin, MD, lead investigator of the study and director of dermatology at Fox Chase Cancer Center. Yaupon intends to file a New Drug Application (NDA) with the FDA later this year.

> "We worked hard to create a topical mechlorethamine formulation that is stable, standardized and cosmetically elegant. We believe our product candidate has the potential to significantly improve the quality of life for patients."

"This is an important achievement for patients living with CTCL. Mechlorethamine has been used for decades in a variety of non-controlled, pharmacy-based formulations that are not subject to consistent manufacturing practices. This is the first time we have a manufactured product that can be reviewed by the FDA and, hopefully, made widely available to patients," said Dr. Lessin. "My mentors published the original research in the early 1970s, so it is especially gratifying to me to have led this collaborative clinical study."

The pivotal trial was a randomized, observer-blinded clinical study designed to determine if Yaupon's proprietary topical mechlorethamine gel was non-inferior to a pharmacy-compounded formulation of mechlorethamine in Aquaphor® in patients with stage I-IIa CTCL. The study, conducted in 260 patients at 13 U.S. cancer centers, is the largest prospective study ever conducted in patients with CTCL.

To meet the criteria for non-inferiority, the lower limit of 95 percent confidence interval around the ratio of response rates had to be greater than 0.75. The primary endpoint for response was a 50 percent or greater improvement from baseline in the Composite Assessment of Index Lesion Severity (CAILS) scoring system. In the Intent to Treat population, 59 percent of the patients randomized to the Yaupon arm compared to 48 percent of the patients on the Aquaphor® arm achieved a response, resulting in a ratio of 1.226 and 95 percent confidence limit of 0.974 to 1.552, thus meeting the protocol defined endpoint. The most common adverse events were related to skin toxicity (local skin irritation, pruritis, contact dermatitis, erythema) and the withdrawal rates due to skin toxicity were similar in both arms.

"Achieving a positive outcome in this pivotal study is a major milestone for Yaupon and we believe that we are now well-positioned to file our first NDA later this year," said Robert Alonso, president and CEO of Yaupon. "We worked hard to create a topical mechlorethamine formulation that is stable, standardized and cosmetically elegant. We believe our product candidate has the potential to significantly improve the quality of life for patients."

Dating back to the 1950s, mechlorethamine has been used in a non-standardized way in both aqueous and paraffin (petroleum jelly) based formulations, either prepared by the patient or by special compounding pharmacies. The paraffin-based formulations are extremely greasy, which often leads to patient dissatisfaction. Yaupon's investigational mechlorethamine is a novel, quick-drying gel formulation made under Good Manufacturing Practices (GMPs). Yaupon expects to file an NDA later this year to seek FDA approval. The product candidate has been granted Fast Track Status and Orphan Drug Status by the FDA.

"Gaining formal FDA acceptance of a standardized topical mechlorethamine product has been one of the highest priorities for the Cutaneous Lymphoma Foundation since 1998," said Judy Jones, president and co-founder of the Cutaneous Lymphoma Foundation. "We are delighted with these study results and are hopeful that the product will soon be available for the 30,000 Americans living with CTCL."

About CTCL

According to the Cutaneous Lymphoma Foundation, cutaneous T-cell lymphoma or CTCL is a rare form of non-Hodgkin's lymphoma that is difficult to diagnose, as mild cases are often confused with other skin conditions. Unlike most non-Hodgkin's lymphomas, CTCL is caused by a mutation of T-cells. The malignant T-cells in the body initially migrate to the skin, causing various lesions to appear. These lesions change shape as the disease progresses, typically beginning as what appears to be a rash, which can be very itchy, and eventually forming into plaques and tumors.

CTCL affects approximately 30,000 patients in the United States, with approximately 3,500 people newly diagnosed each year. Most cases of CTCL are early-stage and are diagnosed in patients over the age of 50. The cause of CTCL remains unknown and there is no known cure. For more information, please visit www.clfoundation.org.

About Yaupon Therapeutics

Yaupon Therapeutics is a privately held, specialty pharmaceutical company that develops small molecule pharmaceuticals licensed from under-served academic institutions. The Company's lead product candidate is a proprietary topical mechlorethamine gel that recently completed a pivotal trial under a Special Protocol Assessment (SPA) with the Food and Drug Administration (FDA) for the treatment of early-stage cutaneous T-cell lymphoma. In addition, Yaupon has a pipeline of earlier-stage products. For more information, please visit www.yauponthерapeutics.com.

Aquaphor® is a registered trademark Beiersdorf AG.

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