Eton Pharmaceuticals Announces U.S. FDA Approval for KHINDIVI™ (hydrocortisone) Oral Solution

- KHINDIVI is the first and only FDA-approved hydrocortisone oral solution
- Commercial launch expected the week of June 2nd
- Eton expects combined peak sales of KHINDIVI and ALKINDI SPRINKLE® (hydrocortisone) oral granules to exceed $50 million

DEER PARK, Ill., MAY 28, 2025 (GLOBE NEWSWIRE) -- Eton Pharmaceuticals, Inc ("Eton" or "the Company") (Nasdaq: ETON), an innovative pharmaceutical company focused on developing and commercializing treatments for rare diseases, today announced the U.S. Food and Drug Administration (FDA) approval of a New Drug Application (NDA) for KHINDIVI™ (hydrocortisone) Oral Solution as a replacement therapy in pediatric patients five years of age and older with adrenocortical insufficiency.

KHINDIVI is the only FDA-approved oral solution formulation of hydrocortisone. It comes in a 1mg/ml strength designed to eliminate the need to split or crush tablets, and to offer simple and accurate dosing specifically tailored to each patient's needs. It does not require refrigeration, mixing, or shaking – it is a ready-to-use oral liquid solution. KHINDIVI is designed to offer administration simplicity and dosing accuracy, and to provide a therapy option for patients who have difficulty swallowing tablets or with special administration needs, such as patients with a gastric tube.

"The FDA approval of KHINDIVI is a tremendous achievement for Eton and more importantly, a pivotal step forward for pediatric patients with adrenal insufficiency. As a home-grown program, our team expertly navigated the development, clinical and regulatory pathway. In addition, being in a position to commercialize KHINDIVI within days of this approval is a further testament to the executional excellence from our entire company," said Sean Brynjelsen, CEO of Eton Pharmaceuticals.

"For decades, patients have been seeking an FDA-approved hydrocortisone liquid that allows incremental, accurate dosing in the preferred dosage form for children. We are excited to now make it available to patients. Our commercial team is fully mobilized and ready to hit the ground running within the first week of approval. We're committed to ensuring that pediatric endocrinologists across the country are aware of this important new treatment option," continued Brynjelsen.

"Managing adrenal insufficiency in pediatric patients requires precise and consistent hydrocortisone dosing that can be carefully titrated to small increments that address the individualized pharmacokinetic needs of each child," said Dr. Kyriakie Sarafoglou, Professor, Division of Pediatric Endocrinology & Division of Pediatric Genetics & Metabolism, University of Minnesota. "The availability of an FDA-approved oral hydrocortisone liquid solution offers physicians a new tool to dose patients accurately, which is important to clinical outcomes during this dynamic period of growth and development."

"For families facing the daily challenges of pediatric congenital adrenal hyperplasia (CAH), timely access to the right treatments is critical," said Dina Matos, Executive Director of the CARES Foundation—the only U.S. organization solely focused on the CAH community. "The introduction of KHINDIVI is a

significant advancement, particularly because accurately splitting pills to achieve proper dosing for children has long been a struggle. The ability to dose patients more accurately is critical for treatment outcomes. We commend Eton for working to make this therapy accessible through specialty channels. This marks meaningful progress for our community and a vital step toward easing the daily burden on parents and caregivers."

KHINDIVI will be promoted by Eton's existing team of pediatric endocrinology rare disease specialists. Eton currently commercializes ALKINDI SPRINKLE (hydrocortisone) Oral Granules, which is FDA-approved for pediatric patients with adrenocortical insufficiency. The addition of KHINDIVI will provide adrenal insufficiency patients and caregivers with an additional option when choosing the treatment that best meets their individual needs.

Adrenocortical insufficiency is a rare, but serious condition in which the adrenal glands do not produce sufficient cortisol. Eton estimates that there are more than 5,000 adrenal insufficiency patients in the U.S. between the ages of 5 and 17, and expects peak sales of KHINDIVI, combined with ALKINDI SPRINKLE, will exceed $50 million per year.

KHINDIVI will be available in the coming days in the United States exclusively through Anovo, a specialty pharmacy dedicated to serving patients with rare and chronic conditions. Anovo will administer the Eton Cares Program in partnership with Eton Pharmaceuticals. The program provides prescription fulfillment, insurance benefits investigation, educational support, financial assistance for qualified patients, and other services designed to help patients access treatment. Eton Cares will offer co-pay assistance to allow for $0 co-pays for qualifying patients.

Clinicians seeking to prescribe KHINDIVI can e-prescribe by selecting Anovo #5 or fax in a patient referral form to 855-813-2039. Additional product details can be found on the product website, https://www.khindivi.com.

INDICATION

KHINDIVI is a corticosteroid indicated as replacement therapy in pediatric patients 5 years of age and older with adrenocortical insufficiency.

Limitation of Use

KHINDIVI is not approved for increased dosing during periods of stress or acute events. Use a different hydrocortisone-containing drug product for stress dosing.

IMPORTANT SAFETY INFORMATION

Contraindication

Hypersensitivity to hydrocortisone or any of the other ingredients in KHINDIVI oral solution.

Warnings and Precautions

Adrenal Crisis: Undertreatment or sudden discontinuation of therapy with KHINDIVI may lead to symptoms of adrenal insufficiency, adrenal crisis, and death. Adrenal crisis may also be induced by stress events, such as infections or surgery when patients require higher doses of corticosteroids. During periods

of stress (e.g., infections, surgery), switch to another oral hydrocortisone product and increase the dose if oral medications are tolerated. Monitor patients when switching to KHINDIVI to ensure KHINDIVI is providing the same level of hydrocortisone exposure as the previously used oral hydrocortisone formulation. If symptoms of adrenal insufficiency occur, increase the total daily dosage of KHINDIVI.

Systemic Adverse Reactions Due to Inactive Ingredients:

Hyperosmolarity

KHINDIVI is not approved in pediatric patients less than 5 years of age. The inactive ingredients polyethylene glycol 400, propylene glycol, and glycerin undergo substantial systemic absorption, individually or in combination, resulting in increased plasma osmolarity in all pediatric patients, especially in pediatric patients less than 5 years of age. Monitor pediatric patients using KHINDIVI for signs and symptoms consistent with hyperosmolarity.

Metabolic Acidosis and Other Adverse Reactions

The inactive ingredient polyethylene glycol 400 and propylene glycol that may result in metabolic acidosis, hypoglycemia, hepato-renal injury, and central nervous system toxicity (e.g., seizure and coma), may increase the risk of adrenal crisis. Monitor laboratory values and for physical signs and symptoms of these adverse reactions.

Laxative Effects Due to Inactive Ingredients

The inactive ingredients polyethylene glycol 400 and glycerin, whether alone or in combination, may cause gastrointestinal irritation resulting in vomiting and/or diarrhea. These gastrointestinal reactions may increase the risk of adrenal crisis. Monitor for signs or symptoms of gastrointestinal irritation and associated fluid and electrolyte abnormalities.

Immunosuppression and Increased Risk of infection with Use of a Dosage Greater Than Replacement: The use of a greater than replacement dosage can suppress the immune system and increase the risk of infection with any pathogen, including viral, bacterial, fungal, protozoan, or helminthic pathogens. Monitor for the development of infection and consider dosage reduction as needed.

Growth Retardation: Long-term use in excessive doses may cause growth retardation. Use the minimum dosage of KHINDIVI to achieve desired clinical response and monitor the patient's growth.

Cushing's Syndrome Due to Use of Excessive Doses of Corticosteroids: Prolonged use with supraphysiologic doses may cause Cushing's syndrome. Monitor patients for signs and symptoms of Cushing's syndrome every 6 months.

Decrease in Bone Mineral Density: Corticosteroids decrease bone formation and increase bone resorption which may lead to the development of osteoporosis. Use the minimum dosage of KHINDIVI to achieve desired clinical response.

Psychiatric Adverse Reactions: Use may be associated with severe psychiatric adverse reactions, such as euphoria, mania, psychosis with hallucinations and delirium, or depression. Symptoms typically emerge within a few days or weeks of starting the treatment. Most reactions resolve after either dose reduction or withdrawal, although specific treatment may be necessary. Monitor patients for behavioral and mood disturbances during treatment. Instruct caregivers and/or patients to seek medical advice if psychiatric symptoms develop.

Ophthalmic Adverse Reactions: Cataracts, glaucoma, and central serous chorioretinopathy have been reported with prolonged use of high doses. Monitor patients for blurred vision or other visual disturbances, and if they occur, refer them to an ophthalmologist.

Gastrointestinal Adverse Reactions: There is an increased risk of gastrointestinal perforation in patients with certain gastrointestinal disorders. Signs of gastrointestinal perforation, such as peritoneal irritation, may be masked in patients receiving corticosteroids. Corticosteroids should be used with caution if there is a probability of impending perforation, abscess, or other pyogenic infections; diverticulitis, fresh intestinal anastomoses, and active or latent peptic ulcer.

Concurrent administration of corticosteroids with nonsteroidal anti-inflammatory drugs (NSAIDs) may increase the risk of gastrointestinal adverse reactions. Monitor patients receiving corticosteroids and concomitant NSAIDs for gastrointestinal adverse reactions.

Risk of Kaposi's Sarcoma with Use of a Dosage Greater Than Replacement: Kaposi's sarcoma has been reported to occur in patients receiving corticosteroid therapy, most often for chronic conditions at a dosage greater than replacement (supraphysiologic dosage). If patients take a supraphysiologic chronic dosage of KHINDIVI, they are at increased risk of developing Kaposi's sarcoma.

Vaccination: Administration of live vaccines may be acceptable in KHINDIVI-treated pediatric patients with adrenocortical insufficiency who receive replacement corticosteroids.

Adverse Reactions

The serious adverse reactions associated with KHINDIVI are adrenal crisis, systemic adverse reactions due to inactive ingredients, immunosuppression and increased risk of infection with dosage greater than replacement, Cushing's Syndrome, growth retardation, Kaposi's Sarcoma risk, psychiatric, ophthalmic and gastrointestinal adverse reactions.

To report a suspected adverse event related to KHINDIVI, contact Eton Pharmaceuticals, Inc. at 1-855-224-0233 or the U.S. Food and Drug Administration (FDA) at http://www.fda.gov/MedWatch or call 1-800-FDA-1088.

Please see full Prescribing Information for more information.

INDICATION AND IMPORTANT SAFETY INFORMATION

Contraindication

Hypersensitivity to hydrocortisone or any of the ingredients in ALKINDI SPRINKLE.

Warnings and Precautions

Adrenal Crisis: Undertreatment or sudden discontinuation of therapy may lead to symptoms of adrenal insufficiency, adrenal crisis, and death. Adrenal crisis may also be induced by stressor events, such as infections or surgery. Monitor patients closely when switching from other forms of hydrocortisone to ALKINDI SPRINKLE. Instruct patients and/or caregivers to contact their healthcare provider if the full dose of ALKINDI SPRINKLE is not administered, as a repeat dose may be required. Increase the dose

during periods of stress. Switch patients who are vomiting, severely ill, or unable to take oral medications to parenteral corticosteroid formulations.

Immunosuppression and Increased Risk of Infection with Use of a Dosage Greater Than Replacement: Use of a greater than replacement dosage can suppress the immune system and increase the risks of new infections or exacerbation of latent infections with any pathogen, including viral, bacterial, fungal, protozoan, or helminthic infections. Monitor patients for signs and symptoms of infections.

Growth Retardation: Long-term use in excessive doses may cause growth retardation. Use the minimum dosage of ALKINDI SPRINKLE to achieve desired clinical response and monitor the patient's growth.

Cushing's Syndrome Due to Use of Excessive Doses of Corticosteroids: Prolonged use with supraphysiologic doses may cause Cushing's syndrome. Monitor patients for signs and symptoms of Cushing's syndrome every 6 months; pediatric patients under one year of age may require more frequent monitoring.

Decrease in Bone Mineral Density: Corticosteroids decrease bone formation and increase bone resorption, which may lead to inhibition of bone growth and development of osteoporosis. Use the minimum dosage of ALKINDI SPRINKLE to achieve desired clinical response.

Psychiatric Adverse Reactions: Use may be associated with severe psychiatric adverse reactions, such as euphoria, mania, psychosis with hallucinations and delirium, or depression. Symptoms typically emerge within a few days or weeks of starting the treatment. Most reactions resolve after either dose reduction or withdrawal, although specific treatment may be necessary. Monitor patients for behavioral and mood disturbances during treatment. Instruct caregivers and/or patients to seek medical advice if psychiatric symptoms develop.

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Gastrointestinal Adverse Reactions: There is an increased risk of gastrointestinal perforation in patients with certain gastrointestinal disorders. Signs of gastrointestinal perforation, such as peritoneal irritation, may be masked in patients receiving corticosteroids. Corticosteroids should be used with caution if there is a probability of impending perforation, abscess, or other pyogenic infections; diverticulitis; fresh intestinal anastomoses; and active or latent peptic ulcer.

Concurrent administration of corticosteroids with nonsteroidal anti-inflammatory drugs (NSAIDs) may increase the risk of gastrointestinal adverse reactions. Monitor patients receiving corticosteroids and concomitant NSAIDs for gastrointestinal adverse reactions.

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Vaccination: Administration of live vaccines may be acceptable in ALKINDI SPRINKLE-treated pediatric patients with adrenocortical insufficiency who receive replacement corticosteroids.

Adverse Reactions

Common adverse reactions for corticosteroids include fluid retention, alteration in glucose tolerance, elevation in blood pressure, behavioral and mood changes, increased appetite, and weight gain.

To report a suspected adverse event related to ALKINDI SPRINKLE, contact Eton Pharmaceuticals, Inc. at 1-855-224-0233 or the U.S. Food and Drug Administration (FDA) at http://www.fda.gov/MedWatch or call 1-800-FDA-1088.

INDICATION

ALKINDI SPRINKLE is a corticosteroid indicated for replacement therapy in pediatric patients with adrenocortical insufficiency.

Please see full Prescribing Information **for more information.**

About Eton Pharmaceuticals

Eton is an innovative pharmaceutical company focused on developing and commercializing treatments for rare diseases. The Company currently has eight commercial rare disease products: KHINDIVI®, INCRELEX®, ALKINDI SPRINKLE®, GALZIN®, PKU GOLIKE®, Carglumic Acid, Betaine Anhydrous, and Nitisinone. The Company has five additional product candidates in late-stage development: ET-600, Amglidia®, ET-700, ET-800 and ZENEO® hydrocortisone autoinjector. For more information, please visit our website at www.etonpharma.com.

Forward-Looking Statements

Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements associated with the expected ability of Eton to undertake certain activities and accomplish certain goals and objectives. These statements include but are not limited to statements regarding Eton's business strategy, Eton's plans to develop and commercialize its product candidates, the safety and efficacy of Eton's product candidates, Eton's plans and expected timing with respect to regulatory filings and approvals, and the size and growth potential of the markets for Eton's product candidates. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "intends," "will," "goal," "potential" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon Eton's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks associated with the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. These and other risks concerning Eton's development programs and financial position are described in additional detail in Eton's filings with the Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made. Eton undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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