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Can-Fite Launches First Clinical Program for Piclidenoson in the Rare Genetic Disease Lowe Syndrome

Phase 2 clinical study protocol submitted; Small Phase 2 study designed to support regulatory interactions and potential registration upon positive results

Ramat Gan, Israel, Aug. 03, 2026 (GLOBE NEWSWIRE) -- Can-Fite BioPharma Ltd. (NYSE American: CANF) (TASE:CANF), a biotechnology company advancing a pipeline of proprietary small molecule drugs that address oncological and inflammatory diseases, today announced the submission of a Phase 2 clinical study protocol to Bambino Gesù Children's Hospital in Rome, Italy, for the first clinical evaluation of Piclidenoson in patients with Lowe syndrome, a rare inherited genetic disorder with no approved disease-modifying therapies. The study will be led by Prof. Francesco Emma, an internationally recognized expert in inherited kidney diseases.

Lowe syndrome is a rare X-linked multisystem genetic disorder caused by mutations in the OCRL gene, resulting in severe renal, neurological, and ocular manifestations. The renal disease is characterized by progressive proximal tubular dysfunction leading to Fanconi syndrome, chronic kidney disease, and eventual kidney failure. Current management is supportive, and no approved therapy addresses the underlying disease mechanism.

Piclidenoson was selected for clinical evaluation based on compelling preclinical studies demonstrating restoration of OCRL-dependent cellular function found by Dr. Antonella De Matteis, Professor of Biology, Department of Molecular Medicine and Medical Biotechnology at the University of Naples Federico II, and Program Coordinator of the Cell Biology and Disease Mechanisms at the Telethon Institute of Genetics and Medicine (TIGEM) in Italy. Can-Fite and Fondazione Telethon have signed a collaboration agreement for the clinical development of Piclidenoson for the treatment of Lowe Syndrome, a high medical need with no drug available.

The Phase 2 study is an open-label, single-center clinical trial designed to evaluate the efficacy and safety of oral Piclidenoson administered twice daily for six months in 5 adult patients with genetically confirmed Lowe syndrome. The primary endpoint is improvement in renal uptake of 99mTc-DMSA as a measure of proximal tubular reabsorption capacity, with secondary endpoints evaluating urinary biomarkers of tubular function, Fanconi syndrome parameters, and safety.

"The initiation of our first clinical program in Lowe syndrome represents an important milestone for Can-Fite," said Motti Farbstein, CEO of Can-Fite BioPharma. "Supported by compelling preclinical data, this focused Phase 2 pilot study is designed to facilitate discussions with regulatory authorities regarding the clinical development and potential registration pathway for Piclidenoson in Lowe syndrome. We are pleased to collaborate with Prof. Francesco Emma and his team at Bambino Gesù Children's Hospital on this important

program."

About Piclidenoson

Piclidenoson is a robust anti-inflammatory agent, currently being evaluated in a pivotal Phase 3 psoriasis clinical study under approval of both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

Piclidenoson is a novel, first-in-class, A3 adenosine receptor agonist (A3AR) small molecule, orally bioavailable drug with an excellent safety profile demonstrating evidence of efficacy in Phase II and Phase III clinical studies. The drug's mechanism of action entails inhibition of the inflammatory cytokines interleukin 17 and 23 (IL-17 and IL-23) and the induction of apoptosis of patients' skin cell keratinocytes involved with the disease pathogenicity.

About Fondazione Telethon

Fondazione Telethon ETS is one of the main Italian biomedical charities, founded in 1990 on the initiative of a group of patients suffering from muscular dystrophy. Its mission is to achieve the cure of rare genetic diseases through scientific research of excellence, selected according to the best practices shared internationally. Through a unique method in the Italian panorama, it follows the entire "research chain" dealing with fundraising, selection and funding of projects and the research activity itself carried out in the centers and laboratories of the Foundation. Telethon also develops collaborations with public health institutions and pharmaceutical industries to translate the results of research into therapies accessible to patients. Since its foundation, Telethon has invested more than 660 million euros in research, has funded 2,960 projects with 1,720 researchers involved and 630 diseases studied. To date, thanks to Fondazione Telethon, the first gene therapy with stem cells in the world has been made available, thanks to the collaboration with the pharmaceutical industry. This therapy is intended for the treatment of ADA-SCID, a severe immunodeficiency that compromises the body's defenses from birth. In 2023, Fondazione Telethon became responsible for the production and distribution of the drug to eligible patients in the European Union

Another gene therapy resulting from Telethon research made available is the one for a serious neurodegenerative disease, metachromatic leukodystrophy. This therapeutic approach is in an advanced stage of development for another immunodeficiency, Wiskott-Aldrich syndrome. Other diseases on which the gene therapy developed by Telethon researchers has been evaluated in patients are beta thalassemia and two metabolic diseases of childhood, mucopolysaccharidosis type 6 and type 1. In addition, within the Telethon institutes a targeted therapeutic strategy is being studied or developed for other genetic diseases, such as hemophilia or various hereditary vision defects. In parallel, the study of basic mechanisms and potential therapeutic approaches for diseases still unanswered continues in all laboratories funded by Telethon.

About Can-Fite BioPharma Ltd.

Can-Fite BioPharma Ltd. (NYSE American: CANF) (TASE: CANF) is an advanced clinical stage drug development Company with a platform technology that is designed to address multi-billion dollar markets in the treatment of cancer, liver, and inflammatory disease. The Company's lead drug candidate, Piclidenoson recently reported topline results in a Phase 3

trial for psoriasis and commenced a pivotal Phase 3 trial. Can-Fite's liver drug, Namodenoson, is being evaluated in a Phase III trial for hepatocellular carcinoma (HCC), a Phase 2b trial for the treatment of MASH, and in a Phase 2a study in pancreatic cancer. Namodenoson has been granted Orphan Drug Designation in the U.S. and Europe and Fast Track Designation as a second line treatment for HCC by the U.S. Food and Drug Administration. Namodenoson has also shown proof of concept to potentially treat other cancers including colon, prostate, and melanoma. CF602, the Company's third drug candidate, has shown efficacy in the treatment of erectile dysfunction. These drugs have an excellent safety profile with experience in over 1,600 patients in clinical studies to date. For more information please visit: www.canfite.com.

Forward-Looking Statements

This press release may contain forward-looking statements, about Can-Fite's expectations, beliefs or intentions regarding, among other things, its product development efforts of Piclidenoson for the treatment of Lowe syndrome. All statements in this communication, other than those relating to historical facts, are "forward looking statements". Forward-looking statements can be identified by the use of forward-looking words such as "believe," "expect," "intend," "plan," "may," "should" or "anticipate" or their negatives or other variations of these words or other comparable words or by the fact that these statements do not relate strictly to historical or current matters. Forward-looking statements relate to anticipated or expected events, activities, trends or results as of the date they are made. Because forward-looking statements relate to matters that have not yet occurred, these statements are inherently subject to known and unknown risks, uncertainties and other factors that may cause Can-Fite's actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. Important factors that could cause actual results, performance or achievements to differ materially from those anticipated in these forward-looking statements include, among other things, our market and other conditions, history of losses and needs for additional capital to fund our operations and our inability to obtain additional capital on acceptable terms, or at all; uncertainties of cash flows and inability to meet working capital needs; the initiation, timing, progress and results of our preclinical studies, clinical trials and other product candidate development efforts; our ability to advance our product candidates into clinical trials or to successfully complete our preclinical studies or clinical trials; our receipt of regulatory approvals for our product candidates, and the timing of other regulatory filings and approvals; the clinical development, commercialization and market acceptance of our product candidates; our ability to establish and maintain strategic partnerships and other corporate collaborations; the implementation of our business model and strategic plans for our business and product candidates; the scope of protection we are able to establish and maintain for intellectual property rights covering our product candidates and our ability to operate our business without infringing the intellectual property rights of others; competitive companies, technologies and our industry; risks related to not satisfying the continued listing requirements of NYSE American; and statements as to the impact of the political and security situation in Israel on our business. More information on these risks, uncertainties and other factors is included from time to time in the "Risk Factors" section of Can-Fite's Annual Report on Form 20-F filed with the SEC on March 26, 2026 and other public reports filed with the SEC and in its periodic filings with the TASE. Existing and prospective investors are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. Can-Fite undertakes no obligation to publicly update or review

any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by any applicable securities laws.

Contact

Can-Fite BioPharma Motti Farbstein info@canfite.com

+972-3-9241114

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