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Caelum Biosciences Announces Positive Phase 1a/1b Data Demonstrating CAEL-101 (mAb 11-1F4) Improves Organ Function in AL Amyloidosis

Eight of 12 patients (67 percent) with cardiac involvement responded to therapy with a median time to cardiac response of 21 days

14 of 24 (63 percent) of patients demonstrated organ response

CAEL-101 dosed once weekly was found to be well tolerated with no dose-limiting toxicity

Data presented by Columbia in oral session at ASH Annual Meeting to support advancement into Phase 2b/3 trial

NEW YORK, Dec. 11, 2017 (GLOBE NEWSWIRE) -- Caelum Biosciences, Inc. ("Caelum"), a Fortress Biotech, Inc. (NASDAQ:FBIO) Company focused on developing treatments for rare and life-threatening diseases, today announced full Phase 1a/1b clinical data demonstrating CAEL-101's (mAb 11-1F4) ability to bind to light-chain amyloid fibrils and achieve early and clinically efficacious organ responses in patients with relapsed and refractory amyloid light chain ("AL") amyloidosis. The data were presented by Columbia University ("Columbia") on December 10th in an oral session at the 59th American Society of Hematology ("ASH") Annual Meeting.

Caelum in-licensed CAEL-101 from Columbia and recently completed a Phase 1a/1b clinical trial at Columbia for the treatment of AL amyloidosis, a rare systemic disorder that causes misfolded amyloid proteins produced by plasma cells to cause buildup in and around tissues, nerves and organs, gradually affecting their function.

"These data indicate CAEL-101 is a safe and well-tolerated therapy that leads to the rapid destruction of amyloid fibrils, and fast and clinically relevant organ response," said Suzanne Lentzsch, M.D., Ph.D., Professor of Medicine at Columbia University Medical Center, College of Physicians and Surgeons of Columbia University and at New York Presbyterian Hospital, and principal investigator for the Phase 1a/1b trial. "Amyloid fibril targeted therapy with CAEL-101 is a promising and innovative treatment approach that can improve organ function and, potentially, mortality in patients suffering from AL amyloidosis. We look forward to further evaluating CAEL-101 in larger randomized clinical trials."

Key Efficacy and Safety Findings

Twenty-seven patients were treated with CAEL-101 in this open-label, dose-escalation trial. In the Phase 1a trial, CAEL-101 was administered to eight patients via a single IV infusion at week one. In the Phase 1b trial, CAEL-101 was administered to 19 patients via one weekly IV infusion for four weeks. Trial investigators at Columbia determined the study achieved its primary objective of establishing maximum tolerated dose of up to 500mg/m^2 of CAEL-101.

Trial investigators presented organ response rates in the Phase 1a and the Phase 1b, with 63 percent (14 of 24) overall organ response rate, 67 percent (8 of 12) overall cardiac response rate and 50 percent (5 of 10) overall renal response rate.[1] Early organ response was demonstrated in a high-mortality population (21 days median time to cardiac response in Phase 1b; 28 days median time to renal response in Phase 1b[2]).

Trial investigators found that CAEL-101 achieved and demonstrated organ response at multiple points in time throughout the duration of treatment; all patients showed an organ response or were stable, and no patients showed organ progression. Organ response independent of a chemotherapy-free light chain response was demonstrated. No drug-related grade 4 or 5 adverse events or dose-limiting toxicities were seen in the trial. There was no mortality during the study. The investigators followed patients beyond the study and reported an overall survival rate of 93 percent (median follow-up period of 18.6 months).

“Investigators at Columbia concluded that CAEL-101 dosed once weekly demonstrated early and clinically efficacious organ response throughout the Phase 1a/1b trial. Moreover, CAEL-101 led to improvements in echocardiograms,” said Michael Spector, President and Chief Executive Officer of Caelum. “According to investigators, CAEL-101 has improved organ response independent of the free light chain response of chemotherapy. In addition, an enlarged liver returned to normal in a patient who had a hematologic response eight months after their organ response. We believe these data underscore CAEL-101’s potential to be a best-in-class treatment for AL amyloidosis, and provide clear signals that support advancement into a Phase 2b/3 trial in the second half of 2018.”

A copy of the presentation can be viewed online through the Caelum website at www.caelumbio.com/pipeline/publications.

About AL Amyloidosis

AL amyloidosis is a rare systemic disorder caused by an abnormality of plasma cells in the bone marrow. Misfolded amyloid proteins produced by plasma cells cause buildup in and around tissues, nerves and organs, gradually affecting their function. This can cause progressive and widespread organ damage, and high mortality rates.

AL amyloidosis affects roughly 30,000 – 40,000 patients in total throughout the U.S. and Europe, and it is estimated that there are approximately 3,000 – 4,000 new cases of AL amyloidosis annually in the U.S., though actual incidence is likely higher as a result of under diagnosis. Amyloidosis has a one-year mortality rate of 47 percent, 76 percent of which are caused by from cardiac amyloidosis.

About CAEL-101 (mAb 11-1F4)

CAEL-101 is a chimeric fibril-reactive monoclonal antibody (mAb) that has completed a

Phase 1a/1b clinical trial at Columbia University for the treatment of patients with amyloid light chain ("AL") amyloidosis. While current treatment with chemotherapy is aimed at reducing production of the amyloid-forming light-chain protein, CAEL-101 attempts to reduce and / or eliminate the amyloid deposits.

About Caelum Biosciences
Caelum Biosciences, Inc. ("Caelum"), a Fortress Biotech (NASDAQ:FBIO) Company, is a clinical-stage biotechnology company developing treatments for rare and life-threatening diseases. Caelum's lead asset, CAEL-101 (mAb 11-1F4), is a novel antibody for the treatment of patients with amyloid light chain ("AL") amyloidosis. Phase 1a/1b data presented at the American Society of Hematology's 59th Annual Meeting in December 2017 support CAEL-101's potential to be a safe and well-tolerated therapy that promotes amyloid resolution. CAEL-101 has received Orphan Drug Designation from the U.S. Food and Drug Administration as a therapeutic agent for patients with AL amyloidosis, and as a radio-imaging agent in amyloidosis. For more information, visit www.caelumbio.com.

About Fortress Biotech
Fortress Biotech, Inc. ("Fortress") is a biopharmaceutical company dedicated to acquiring, developing and commercializing novel pharmaceutical and biotechnology products. Fortress develops and commercializes products both within Fortress and through certain subsidiary companies, also known as Fortress Companies. In addition to its internal development programs, Fortress leverages its biopharmaceutical business expertise and drug development capabilities and provides funding and management services to help the Fortress Companies achieve their goals. Fortress and the Fortress Companies may seek licensings, acquisitions, partnerships, joint ventures and/or public and private financings to accelerate and provide additional funding to support their research and development programs. For more information, visit www.fortressbiotech.com.

Forward-Looking Statements
This press release may contain "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, as amended. Such statements include, but are not limited to, any statements relating to our growth strategy and product development programs and any other statements that are not historical facts. Forward-looking statements are based on management's current expectations and are subject to risks and uncertainties that could negatively affect our business, operating results, financial condition and stock price. Factors that could cause actual results to differ materially from those currently anticipated include: risks relating to our growth strategy; our ability to obtain, perform under and maintain financing and strategic agreements and relationships; risks relating to the results of research and development activities; uncertainties relating to preclinical and clinical testing; risks relating to the timing of starting and completing clinical trials; our dependence on third-party suppliers; our ability to attract, integrate and retain key personnel; the early stage of products under development; our need for substantial additional funds; government regulation; patent and intellectual property matters; competition; as well as other risks described in our SEC filings. We expressly disclaim any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in our expectations or any changes in events, conditions or circumstances on which any such statement is based, except as required by law.

[1] Response rates are based on the number of evaluable patients.

[2] First renal response evaluation point was 28 days for all but one patient, who was evaluated at 21 days.

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