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Caelum Biosciences Announces Updated Phase 1b Data Presented at 16th International Symposium on Amyloidosis

New data analysis demonstrates correlation between sustained decrease in NT-proBNP and improvement in global longitudinal strain in cardiac population following CAEL-101 treatment

NEW YORK, March 27, 2018 (GLOBE NEWSWIRE) -- Caelum Biosciences, Inc. ("Caelum"), a Fortress Biotech, Inc. (NASDAQ:FBIO) Company developing treatments for rare and life-threatening diseases, today announced a new analysis of data from the Phase 1b trial of CAEL-101 (mAb 11-1F4) for the treatment of relapsed or refractory amyloid light chain ("AL") amyloidosis, demonstrating a correlation between a sustained decrease in N-terminal pro-brain natriuretic peptide (NT-proBNP) levels and an improvement in global longitudinal strain (GLS) following CAEL-101 treatment in patients with cardiac AL amyloidosis. The data were presented today in an oral session at the 16th International Symposium on Amyloidosis (ISA).

The Phase 1a/1b trial (ClinicalTrials.gov Identifier: NCT02245867) examined the tolerance, safety, pharmacokinetics and possible clinical benefit of CAEL-101 in patients with AL amyloidosis. CAEL-101 was administered to eight patients via a single IV infusion at week one in the Phase 1a portion of the trial, and to 19 patients via one weekly IV infusion for four weeks in the Phase 1b portion of the trial.

Eight of 12 (67 percent) of evaluable cardiac patients in the Phase 1a/1b trial demonstrated a sustained decrease in NT-proBNP levels, an important biomarker in cardiac disease, after treatment with CAEL-101. Evaluable patients presented with cardiac amyloidosis at baseline (NT-proBNP ≥650 pg/mL) and at least one post-baseline NT-proBNP measure. The depth and magnitude of response continued through four once-weekly doses of CAEL-101.

An improvement in GLS, a measure of myocardial shortening during systole, was demonstrated in eight patients with confirmed cardiac amyloidosis at baseline who were enrolled in the Phase 1b portion of the trial, and was correlated with the reduction in NT-proBNP (Pearson correlation coefficient[i] 0.345), demonstrating CAEL-101's positive impact on cardiac response of myocardial function.

"GLS is an important biomarker for cardiac dysfunction and response, and may be a more

accurate predictor of survival than cardiac biomarkers, which can be elevated disproportionately to the severity of cardiac symptoms and based on factors unrelated to worsening cardiac dysfunction," said Camille Edwards, M.D., Fellow in the Department of Hematology and Oncology at Boston Medical Center, who presented the data at ISA. "CAEL-101 is a novel and promising treatment that has the potential to safely promote amyloid resolution and improve cardiac and overall organ function in AL amyloidosis. The presentation of a complete analysis of cardiac data from the Phase 1b trial is planned for later this year."

"The correlation of GLS improvement with NT-proBNP reduction builds upon the strong Phase 1a/1b safety and efficacy data presented at ASH 2017, and it is important to note that efficacy was demonstrated independent of plasma-cell directed therapy," said Michael Spector, President and Chief Executive Officer of Caelum.

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 is caused by 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 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.

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[i] The Pearson correlation coefficient is a measure of the linear correlation between two variables.

Source: Caelum Biosciences, Inc.