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Fortress Biotech Announces Initiation of Phase 3 Studies of CAEL-101 in AL Amyloidosis

- Caelum Biosciences, a company founded by Fortress, is collaborating with Alexion Pharmaceuticals on the studies

- Phase 2 study met primary objective, supporting initiation of two parallel Phase 3 studies that will enroll ~370 AL amyloidosis patients

- Positive long-term Phase 1a/1b data presented at the International Symposium on Amyloidosis (ISA) 2020 demonstrate prolonged overall survival (78 percent at 37 months) and durable organ response

NEW YORK, Sept. 14, 2020 (GLOBE NEWSWIRE) -- Fortress Biotech, Inc. (Nasdaq: FBIO) ("Fortress"), an innovative revenue-generating company focused on acquiring, developing and commercializing or monetizing promising biopharmaceutical products and product candidates cost-effectively, today announced that Caelum Biosciences, Inc. ("Caelum"), in collaboration with Alexion Pharmaceuticals, Inc. ("Alexion"), initiated the Cardiac Amyloid Reaching for Extended Survival (CARES) Phase 3 clinical program to evaluate CAEL-101, a first-in-class amyloid fibril targeted therapy, in combination with standard-of-care (SoC) therapy in AL amyloidosis.

In 2019, Caelum, a company founded by Fortress, entered into a collaboration agreement with Alexion under which Alexion acquired a minority equity interest in Caelum and an exclusive option to acquire the remaining equity in the company based on Phase 3 CAEL-101 data. Fortress maintains a 40 percent ownership in Caelum's issued and outstanding stock and is eligible to receive approximately 43 percent of the proceeds from an Alexion acquisition option exercise.

"We are pleased that CAEL-101 has progressed into Phase 3 development," said Lindsay A. Rosenwald, M.D., Chairman, President and Chief Executive Officer of Fortress. "CAEL-101 has the potential to fill the urgent need for new treatment options for patients with AL amyloidosis, as the data thus far suggest it may improve organ function and overall survival. The partnership between Caelum and Alexion represents an exciting opportunity to help patients and create value for our shareholders. We look forward to the continued late-stage development of this important therapy."

The CARES clinical program includes two parallel Phase 3 studies – one in patients with Mayo stage IIIa disease and one in patients with Mayo stage IIIb disease – and will collectively enroll approximately 370 patients globally. Enrollment is underway in both studies. The primary objective of the clinical program is to assess overall survival.

AL amyloidosis, also called primary amyloidosis, is a rare disease that occurs when an abnormal protein called amyloid builds up in organs and interferes with their normal function. It affects many organs, including the heart and kidneys, causes significant damage and impairs organ function. Median survival in patients with AL amyloidosis that has affected the heart is less than a year following diagnosis.

About the CARES Phase 3 Clinical Program

The CARES clinical program consists of two parallel double-blind, randomized, event-driven global Phase 3 studies, which are evaluating the efficacy and safety of CAEL-101 in AL amyloidosis patients who are newly diagnosed and naïve to standard of care (SoC) treatment (cyclophosphamide-bortezomib-dexamethasone (CyBorD) chemotherapy). One study is enrolling approximately 260 patients with Mayo stage IIIa disease andone study is enrolling approximately 110 patients with Mayo stage IIIb disease. The studies will be conducted at approximately 70 sites across North America, the United Kingdom, Europe, Israel, Japan, and Australia.

In each study, participants are being randomized in a 2:1 ratio to receive either CAEL-101 plus SoC or placebo plus SoC once weekly for four weeks. This will be followed by a maintenance dose administered every two weeks until the last patient enrolled completes at least 50 weeks of treatment. Patients will continue follow-up visits every 12 weeks.

The primary study objectives are overall survival and the safety and tolerability of CAEL-101. Key secondary objectives will assess functional improvement in the six-minute walk test (6MWT), quality of life measures (Kansas City Cardiomyopathy Questionnaire Overall Score & Short Form 36 version 2 Physical Component Score) and cardiac improvement (Global Longitudinal Strain (GLS)).

Phase 2 Study Results

The Phase 2 open-label dose escalation study was conducted to investigate higher doses of CAEL-101 than had been evaluated in Phase 1 studies with a primary objective to identify the best dose to advance into Phase 3 development. The study evaluated the safety and tolerability of CAEL-101 in 13 AL amyloidosis patients at three study sites who received up to 1000 mg/m2 of CAEL-101 (two times the Phase 1 dose) administered in combination with SoC treatment. The study met its primary objectives, supporting the safety and tolerability of CAEL-101 and the selection of the 1000 mg/m2 dose for the Phase 3 study.

Phase 1a/1b Long-Term Follow-Up Results Presented at International Symposium on Amyloidosis 2020

As previously reported, the Phase 1a/1b study of CAEL-101 was the first clinical trial to demonstrate improvement in cardiac function via GLS after treatment with an amyloid fibril targeted therapy in AL amyloidosis patients with amyloid cardiac involvement. New long-term follow-up data from the Phase 1a/1b study will be presented at the virtual International Symposium on Amyloidosis (ISA), September 14 to 18, 2020, in the poster titled, “Long term follow-up of patients with AL amyloidosis treated on a phase 1 study of Anti-Amyloid Monoclonal Antibody CAEL-101” (Abstract #342, Divaya Bhutani, M.D., et. al, Columbia

University Medical Center). These data demonstrate 78 percent survival (15/19) at a median follow-up of more than three years (37 months) in AL amyloidosis patients treated with CAEL-101 as well as durable organ response among evaluable patients, further supporting the advancement of CAEL-101 into Phase 3 development.

About CAEL-101
CAEL-101 is a first-in-class monoclonal antibody (mAb) designed to improve organ function by reducing or eliminating amyloid deposits in the tissues and organs of patients with AL amyloidosis. The antibody is designed to bind to misfolded light chain protein and amyloid and shows binding to both kappa and lambda subtypes. In a Phase 1a/1b study, CAEL-101 demonstrated improved organ function, including cardiac and renal function, in 27 patients with relapsed and refractory AL amyloidosis who had previously not had an organ response to standard of care therapy. CAEL-101 has received Orphan Drug Designation from both the U.S. Food and Drug Administration and European Medicines Agency as a therapy for patients with AL amyloidosis.

About AL Amyloidosis
AL amyloidosis is a rare systemic disorder caused by an abnormality of plasma cells in the bone marrow. Misfolded immunoglobulin light chains produced by plasma cells aggregate and form fibrils that deposit in tissues and organs. This deposition can cause widespread and progressive organ damage and high mortality rates, with death most frequently occurring as a result of cardiac failure. Current standard of care includes plasma cell directed chemotherapy and autologous stem cell transplant, but these therapies do not address the organ dysfunction caused by amyloid deposition, and up to 80 percent of patients are ineligible for transplant.

AL amyloidosis is a rare disease but is the most common form of amyloidosis. There are approximately 22,000 patients across the United States, France, Germany, Italy, Spain and the United Kingdom. AL amyloidosis has a one-year mortality rate of 47 percent, 76 percent of which is caused by cardiac amyloidosis.

About Caelum Biosciences
Caelum Biosciences, Inc. (“Caelum”) is a clinical-stage biotechnology company developing treatments for rare and life-threatening diseases. Caelum’s lead asset, CAEL-101, is a novel antibody for the treatment of patients with amyloid light chain (“AL”) amyloidosis. In 2019, Caelum entered a collaboration agreement with Alexion under which Alexion acquired a minority equity interest in Caelum and an exclusive option to acquire the remaining equity in the company based on Phase 3 CAEL-101 data. Caelum was founded by Fortress Biotech, Inc. (NASDAQ: FBIO). For more information, visit www.caelumbio.com.

About Fortress Biotech
Fortress Biotech, Inc. (“Fortress”) is an innovative biopharmaceutical company that was recently ranked number 10 in Deloitte’s 2019 Technology Fast 500™, an annual ranking of the fastest-growing North American companies in the technology, media, telecommunications, life sciences and energy tech sectors, based on percentage of fiscal year revenue growth over a three-year period. Fortress is focused on acquiring, developing and commercializing high-potential marketed and development-stage drugs and drug candidates. The company has five marketed prescription pharmaceutical products and over 25 programs in development at Fortress, at its majority-owned and majority-controlled partners and at partners it founded and in which it holds significant minority ownership

positions. Such product candidates span six large-market areas, including oncology, rare diseases and gene therapy, which allow it to create value for shareholders. Fortress advances its diversified pipeline through a streamlined operating structure that fosters efficient drug development. The Fortress model is driven by a world-class business development team that is focused on leveraging its significant biopharmaceutical industry expertise to further expand the company's portfolio of product opportunities. Fortress has established partnerships with some of the world's leading academic research institutions and biopharmaceutical companies to maximize each opportunity to its full potential, including Alexion Pharmaceuticals, Inc., AstraZeneca, City of Hope, Fred Hutchinson Cancer Research Center, InvaGen Pharmaceuticals Inc. (a subsidiary of Cipla Limited), St. Jude Children's Research Hospital and Nationwide Children's Hospital. For more information, visit www.fortressbiotech.com.

Forward-Looking Statement

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. As used below and throughout this press release, the words "we", "us" and "our" may refer to Fortress individually or together with one or more partner companies, as dictated by context. 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; risks relating to the COVID-19 outbreak and its potential impact on our employees' and consultants' ability to complete work in a timely manner and on our ability to obtain additional financing on favorable terms or at all; 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 Securities and Exchange Commission 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 may be required by law, and we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995. The information contained herein is intended to be reviewed in its totality, and any stipulations, conditions or provisos that apply to a given piece of information in one part of this press release should be read as applying mutatis mutandis to every other instance of such information appearing herein.

Company Contacts:

Jaclyn Jaffe and William Begien
Investor Relations
(781) 652-4500
ir@fortressbiotech.com

Michael Spector, President & Chief Executive Officer
Caelum Biosciences, Inc.
mspector@caelumbio.com

Investor Relations Contact:
Daniel Ferry
LifeSci Advisors, LLC
(617) 430-7576
daniel@lifesciadvisors.com

Media Relations Contact:
Tony Plohoros
6 Degrees
(908) 591-2839
tplohoros@6degreespr.com

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