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# Fortress Biotech Announces CAEL-101 Strategic Partnership with Alexion Pharmaceuticals

*Fortress-founded Caelum Biosciences to receive \$60 million in a strategic equity investment and option fee*

*Alexion has option to acquire Caelum Biosciences based on Phase 2 data*

NEW YORK, Jan. 31, 2019 (GLOBE NEWSWIRE) -- Fortress Biotech, Inc. (NASDAQ: FBIO) ("Fortress"), a biopharmaceutical company dedicated to acquiring, developing and commercializing novel pharmaceutical and biotechnology products, today announced that Caelum Biosciences, Inc. ("Caelum") has signed a strategic agreement with Alexion Pharmaceuticals, Inc. ("Alexion") (NASDAQ: ALXN) to advance the development of CAEL-101 for light chain (AL) amyloidosis. CAEL-101 is a first-in-class amyloid fibril targeted therapy designed to improve organ function by reducing or eliminating amyloid deposits in patients with AL amyloidosis. AL amyloidosis is a rare systemic disorder that causes misfolded immunoglobulin light chain protein to build up in and around tissues, resulting in progressive and widespread organ damage, most commonly the heart and kidneys. Founded by Fortress, Caelum is a company focused on developing treatments for rare and life-threatening diseases.

Under the terms of the agreement, Alexion will acquire a minority equity interest in Caelum and an exclusive option to acquire the remaining equity in the company based on Phase 2 data for pre-negotiated economics. Alexion will make payments to Caelum totaling up to \$60 million, including the purchase price for the equity and milestone-dependent development funding payments. The collaboration also provides for potential additional payments of up to \$500 million, including the upfront and regulatory and commercial milestone payments, in the event Alexion exercises the acquisition option. The collaboration will leverage Alexion's expertise in rare disease antibody development and commercial franchise in hematology. Alexion and Caelum will collaborate on the design of the ongoing development program for CAEL-101. Caelum will be responsible for conducting the development program through the end of Phase 2 and for manufacturing CAEL-101.

Lindsay A. Rosenwald, M.D., Fortress' Chairman, President and Chief Executive Officer, said, "This collaboration with Alexion, a global leader in the rare disease field, is a testament to the Fortress team's expertise in identifying and developing promising treatments to their full potential. Fortress founded Caelum in January 2017 on the potential of CAEL-101, with

the goal of improving treatment options for patients with AL amyloidosis. This partnership represents an exciting opportunity for CAEL-101 to help patients, as well as create value for Fortress investors. We look forward to seeing what Caelum and Alexion will achieve through this collaboration while we continue to develop a robust pipeline of compelling product candidates at Fortress.”

Fortress has more than 25 programs in development, including seven late-stage and one mid-stage product candidates, at the Fortress level, at its majority-owned and majority-controlled subsidiaries and at entities in which it holds minority ownership positions. In addition to CAEL-101, late-stage and mid-stage programs include:

- Intravenous (IV) tramadol for post-surgical acute pain
- MB-107 for X-linked severe combined immunodeficiency (X-SCID)
- CUTX-101 (Copper Histidinate) for Menkes disease
- CK-101, a third-generation irreversible mutant selective EGFR, for frontline small cell lung cancer with EGFR mutations
- CK-301, an anti-PD-L1 mAb, for selected recurrent or metastatic cancers
- Triplex for cytomegalovirus (CMV)
- CEVA101 for pediatric and adult severe traumatic brain injury

Fortress also has seven marketed products, which include Targadox®, Exelderm®, Cercade®, Luxamend®, Ala-Quin®, Ala-Scalp® and Triderm™.

### **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, gradually affecting their function. This can cause progressive and widespread 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 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 insoluble light chain amyloid protein, including 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 the U.S. Food and Drug Administration as a therapy for patients with AL amyloidosis, and as a radio-imaging agent in AL amyloidosis.

### **About Caelum Biosciences**

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. Phase 1a/1b data support CAEL-101's potential to be a well-tolerated therapy that promotes amyloid resolution. Fortress Biotech founded Caelum in January 2017 and owns approximately 40% of Caelum's issued and outstanding capital stock after the Alexion equity subscription. Fortress is also eligible to receive approximately 43% of the proceeds from an Alexion acquisition option exercise. For more information, visit [www.caelumbio.com](http://www.caelumbio.com).

### **About Fortress Biotech**

Fortress Biotech is an innovative biopharmaceutical company focused on identifying, licensing and developing high-potential clinical-stage assets. The company has over 25 programs in clinical development at Fortress, at its majority-owned and majority-controlled subsidiaries and at entities it founded and in which it holds significant minority ownership positions. Such product candidates span six large-market therapeutic areas, including oncology, rare diseases and gene therapy, which allow it to create value while mitigating risk 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., City of Hope, Fred Hutchinson Cancer Research Center, InvaGen Pharmaceuticals, Inc. (a subsidiary of Cipla Limited), St. Jude Children's Research Hospital, Harvard Medical School and University College London (UCL). For more information, visit [www.fortressbiotech.com](http://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 may be required by law.

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