Alexion and Caelum Biosciences Announce Collaboration to Develop Targeted Therapy for Light Chain (AL) Amyloidosis

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- Collaboration provides opportunity to diversify Alexion's clinical-stage rare hematology portfolio -

- Caelum to receive up to $60 million for equity investment and option fee -

- Alexion has option to acquire Caelum based on Phase 2 data -

BOSTON & NEW YORK--(BUSINESS WIRE)--Jan. 31, 2019-- Alexion Pharmaceuticals, Inc. (NASDAQ: ALXN) and Caelum Biosciences today announced a collaboration to develop 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 to the heart and kidneys.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20190131005293/en/

"With a median survival time of less than 18 months following diagnosis and no approved therapies to address the organ damage caused by AL amyloidosis, there is a significant need for new treatments for this devastating disease," said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. "We believe CAEL-101 holds significant promise in being able to help these patients and we're excited to add it to our growing clinical-stage rare hematology portfolio."

Michael Spector, President and Chief Executive Officer of Caelum, said, "CAEL-101 appears to have a unique capability of binding to both kappa and lambda misfolded proteins. Data from the Phase 1a/1b study indicate that CAEL-101 is a well-tolerated therapy that leads to a rapid and clinically relevant organ response, particularly in the heart and kidneys. Further, CAEL-101 showed a statistically significant improvement from baseline in global longitudinal strain, an endpoint that has been correlated with survival in patients with AL amyloidosis. We are very pleased to collaborate with Alexion, a global leader in the rare disease field."

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.

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 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 Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the discovery, development and commercialization of life-changing therapies. As the global leader in complement biology and inhibition for more than 20 years, Alexion has developed and commercializes two approved complement inhibitors to treat patients with paroxysmal nocturnal hemoglobinuria (PNH), as well as the first and only approved complement inhibitor to treat atypical hemolytic uremic syndrome (aHUS) and anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG), and is also developing it for patients with neuromyelitis optica spectrum disorder (NMOSD). Alexion also has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). In addition, the company is developing several mid-to-late-stage therapies, including a second

complement inhibitor, a copper-binding agent for Wilson disease and an anti-neonatal Fc receptor (FcRn) antibody for rare Immunoglobulin G (IgG)-mediated diseases. Alexion focuses its research efforts on novel molecules and targets in the complement cascade and its development efforts on the core therapeutic areas of hematology, nephrology, neurology and metabolic disorders. Alexion has been named to the *Forbes* list of the World's Most Innovative Companies seven years in a row and is headquartered in Boston, Massachusetts' Innovation District. The company also has offices around the globe and serves patients in more than 50 countries. This press release and further information about Alexion can be found at: www.alexion.com.

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About Caelum Biosciences

Caelum Biosciences, founded by Fortress Biotech, Inc.(NASDAQ: FBIO) 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. For more information, visit www.CaelumBio.com.

Forward-Looking Statement

This press release includes forward-looking statements, including statements related to the therapeutic benefits of CAEL-101, the potential of CAEL-101 as a treatment for AL amyloidosis and the potential benefits of the collaboration. Such forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statements. The process by which an early stage product such as CAEL-101 could potentially lead to an approved product for the treatment of AL amyloidosis is long and subject to highly significant risks, including for example, decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of products, delays, interruptions or failures in manufacture and supply, failure to satisfactorily address matters raised by the U.S. Food and Drug Administration and other regulatory agencies, the possibility that results of clinical trials are not predictive of safety and efficacy results in broader patient populations, the possibility that clinical trials could be delayed, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with AL amyloidosis are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the SEC, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended September 30, 2018 and in Alexion's other filings with the SEC. Alexion and Caelum disclaim any obligation to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

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