Alexion and Caelum Biosciences Announce Start of Phase 3 Studies of CAEL-101 in AL Amyloidosis

- 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% at 37 months) and durable organ response -

BOSTON & BORDENTOWN, N.J. – SEPTEMBER 14, 2020 - Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) and Caelum Biosciences today announced the initiation of 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. 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.

"In AL amyloidosis, misfolded amyloid proteins can build up in many organs throughout the body, including the heart and kidneys, causing significant damage to these organs and impairing their function. While current treatments address the bone marrow disorder that creates the misfolded amyloid proteins, there are no approved therapies for the significant organ damage the disease causes," said John Orloff, M.D., Executive Vice President and Head of Research and Development at Alexion. "CAEL-101 has the potential to be the first treatment to target and remove the amyloid deposits from these organs. Data from Phase 1 studies suggest that this treatment approach may improve organ function and long-term survival. We look forward to investigating this further in the Phase 3 clinical program."

"AL amyloidosis is particularly devastating when it affects the heart, with median survival in these patients of less than one year following diagnosis," said Michael Spector, President and Chief Executive Officer of Caelum. "Long-term survival data from AL amyloidosis patients treated with CAEL-101 in the Phase 1a/1b study showed that 78 percent were still alive after a median follow-up time of more than three years. We recognize the urgent need for new treatments that address the organ damage caused by AL amyloidosis and are working together with the AL amyloidosis community and Alexion to advance the Phase 3 clinical program as quickly as possible."

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 and one 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, or 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 ISA 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 Medicine 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 Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development and commercialization of life-changing medicines. As a leader in rare diseases for more than 25 years, Alexion has developed and commercializes two approved complement inhibitors to treat patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), as well as the first and only approved complement inhibitor to treat anti-acetylcholine receptor (AchR) antibody-positive generalized myasthenia gravis (gMG) and 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) as well as the first and only approved Factor Xa inhibitor reversal agent. In addition, the company is developing several mid-to-late-stage therapies, including a copper-binding agent for Wilson disease, an anti-neonatal Fc receptor (FcRn) antibody for rare Immunoglobulin G (IgG)-mediated diseases and an oral Factor D inhibitor as well as several early-stage therapies, including one for light chain (AL) amyloidosis, a second oral Factor D inhibitor and a third complement inhibitor. 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, metabolic disorders and cardiology. Headquartered in Boston, Massachusetts, Alexion 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, 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.

Forward-Looking Statement

This press release contains forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Alexion and Caelum, including statements related to: the safety and efficacy CAEL-101 as a treatment for AL amyloidosis; CAEL-101 has the potential to be the first treatment to target and remove the amyloid deposits from the heart, kidney and other organs; data from the Phase 1 studies suggest that the treatment approach may improve organ function and long-term survival and enrollment of the Phase 3 trials. Forward-looking statements are subject to factors that may cause Alexion's and Caelum's results and plans to differ materially from those expected by these forward looking statements, including for example: the anticipated safety profile and the benefits of the CAEL-101 may not be realized (and the results of the clinical trials may not be indicative of future results); the inability to enroll and complete the Phase 3 trial; results of clinical trials may not be sufficient to satisfy regulatory authorities; results in clinical trials may not be indicative of results from later stage or larger clinical trials (or in broader patient populations); the possibility that results of clinical trials are not predictive of safety and efficacy and potency of

our products (or we fail to adequately operate or manage our clinical trials) which could cause us to discontinue sales of the product (or halt trials, delay or prevent us from making regulatory approval filings or result in denial of approval of our product candidates); the severity of the impact of the COVID-19 pandemic on Alexion's or Caelum's business, including on commercial and clinical development programs; unexpected delays in clinical trials; unexpected concerns regarding products and product candidates that may arise from additional data or analysis obtained during clinical trials or obtained once used by patients following product approval; future product improvements may not be realized due to expense or feasibility or other factors; delays (expected or unexpected) in the time it takes regulatory agencies to review and make determinations on applications for the marketing approval of our products; inability to timely submit (or failure to submit) future applications for regulatory approval for our products and product candidates; inability to timely initiate (or failure to initiate) and complete future clinical trials due to safety issues, IRB decisions, CMC-related issues, expense or unfavorable results from earlier trials (among other reasons); future competition from biosimilars and novel products; decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of our products; delays or failure of product candidates to obtain regulatory approval; delays or the inability to launch product candidates due to regulatory restrictions, anticipated expense or other matters; interruptions or failures in the manufacture and supply of our products and our product candidates; failure to satisfactorily address matters raised by regulatory agencies regarding our products and product candidates; uncertainty of long-term success in developing, licensing or acquiring other product candidates or additional indications for existing products; the adequacy of our pharmacovigilance and drug safety reporting processes; failure to protect and enforce our data, intellectual property and proprietary rights and the risks and uncertainties relating to intellectual property claims, lawsuits and challenges against us; the risk that third party payors (including governmental agencies) will not reimburse for the use of our products at acceptable rates or at all; delay of collection or reduction in reimbursement due to adverse economic conditions or changes in government and private insurer regulations and approaches to reimbursement; adverse impacts on supply chain, clinical trials, manufacturing operations, financial results, liquidity, hospitals, pharmacies and health care systems from natural disasters and global pandemics, including COVID-19 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 June 30, 2020 and in their other filings with the SEC. Alexion disclaims 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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