



## Caelum and Alexion Announce Upcoming Data Presentations at the 62nd American Society of Hematology Annual Meeting and Exposition

November 4, 2020

- Accepted abstracts include Phase 2 safety, efficacy, and tolerability data for CAEL-101 in AL amyloidosis -

- Phase 3 studies of CAEL-101 in AL amyloidosis are underway -

BORDENTOWN, N.J. & BOSTON--(BUSINESS WIRE)--Nov. 4, 2020--

[Caelum Biosciences](#) and [Alexion Pharmaceuticals, Inc.](#) (NASDAQ:ALXN) today announced that two abstracts on CAEL-101, a first-in-class amyloid fibril targeted therapy, have been accepted for presentation at the 62<sup>nd</sup> American Society of Hematology (ASH) Annual Meeting and Exposition, taking place virtually from December 5 to 8, 2020. New data, from Cleveland Clinic, will be presented on the safety, efficacy and tolerability of CAEL-101 in combination with standard-of-care therapy in AL amyloidosis from the Phase 2 open-label dose escalation study that suggest early evidence of organ response. Data, from Caelum, that further demonstrate the safety and tolerability of CAEL-101 and support the selection of the 1000 mg/m<sup>2</sup> dose for the Phase 3 study will also be presented.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20201104005506/en/>

The accepted abstracts are listed below and are now available on the ASH website:

### Oral Presentation

[Safety, Tolerability and Efficacy of CAEL-101 in AL Amyloidosis Patients Treated on a Phase 2, Open-Label, Dose Selection Study to Evaluate the Safety and Tolerability of CAEL-101 in Patients with AL Amyloidosis](#), Abstract #729. An oral symposium presentation is scheduled for December 7, 2020, 2:45 p.m. PST.

### ePoster Presentation

[CAEL-101 is Well-Tolerated in AL Amyloidosis Patients Receiving Concomitant Cyclophosphamide-Bortezomib-Dexamethasone \(CyborD\): A Phase 2 Dose-Finding Study \(NCT04304144\)](#), Abstract #2277 – poster presentation, poster session II, December 6, 2020, 7:00 a.m. - 3:30 p.m. PST.

As was previously [announced](#), the Cardiac Amyloid Reaching for Extended Survival (CARES) Phase 3 clinical program to evaluate CAEL-101 in combination with standard-of-care (SoC) therapy in AL amyloidosis has begun. Enrollment is underway in 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. The Phase 2 program continues with the addition of a study arm to evaluate CAEL-101 in combination with SoC therapy plus daratumumab.

### **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 potential 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 systemic 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](http://www.caelumbio.com).

### **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 hematology, nephrology, neurology, metabolic disorders, cardiology, ophthalmology and acute care. 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](http://www.alexion.com).

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### **Forward-Looking Statement**

This press release may contain “forward-looking statements,” including as such term is defined within 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 Caelum’s or Alexion’s growth strategy and our respective product development programs, plans related to clinical trials (including, commencement, completion and future patient enrollment), the anticipated benefits of CAEL-101 and any other statements that are not historical facts. Forward-looking statements are based on Caelum and Alexion management’s current expectations and are subject to risks and uncertainties that could negatively affect each of our businesses, operating results, financial condition and stock price. Factors that could cause actual results to differ materially from those currently anticipated include: risks that CAEL-101 is not shown to be safe and effective in clinical trials or does not receive regulatory approval to be marketed; risks relating to each company’s growth strategy; 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 (including, commencement, completion and future patient enrollment); 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 each Company’s employees’ and consultants’ ability to complete work in a timely manner and on the 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; Caelum’s need for substantial additional funds; government regulation; patent and intellectual property matters; competition; as well as other risks described in each Company’s SEC filings. Alexion and Caelum 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 respective 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 or any other protections afforded by applicable law. 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.

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