



Caelum Biosciences Announces Two Oral Presentations of Additional Data from Phase 1b Study of Anti-Amyloid mAb CAEL-101 and Preclinical Imaging Study at 60th American Society of Hematology Annual Meeting

Phase 1b data confirm efficacy in AL Amyloidosis with two cardiac markers correlated with survival

Preclinical data demonstrate the potential of using radiolabeled CAEL-101 for real-time imaging of human amyloidosis in vivo

NEW YORK, NY – December 4, 2018 – Caelum Biosciences, Inc. (“Caelum”), a company focused on developing treatments for rare and life-threatening diseases, today announced additional global longitudinal strain (“GLS”) data from the Phase 1b study of CAEL-101, a light chain fibril-reactive monoclonal antibody (“mAb”) 11-1F4, in patients with cardiac amyloid light chain (“AL”) amyloidosis, which further confirmed CAEL-101’s efficiency improving GLS and NT-proBNP. The Company also announced imaging data from a preclinical study that demonstrate the potential of using radiolabeled CAEL-101 for real-time imaging of human amyloidosis in vivo. The data were presented during two oral sessions at the 60th American Society of Hematology (“ASH”) Annual Meeting by Divaya Bhutani, M.D., and Jing Fu, Ph.D., of Columbia University Medical Center (“CUMC”).

In the Phase 1b study of relapsed and refractory systemic AL amyloidosis, 10 out of 19 patients had cardiac involvement, with a median NT-proBNP of 1186 (range 699-3964) at screening. CAEL-101 was administered weekly for four weeks with sequential doses of 0.5, 5, 10, 50, 100, 250 and 500 mg/m² in a dose-escalation design. All patients underwent transthoracic echocardiograms at screening and 12 weeks post-therapy.

Eight of 10 patients who had cardiac involvement were considered evaluable for NT-proBNP response. Six out of the eight patients (75%) with cardiac involvement met cardiac response criteria by having a decrease in NT-proBNP \geq 30%. Among echocardiographic parameters, mean GLS improved significantly in nine out of 10 patients from $-15.58 \pm -4.14\%$ at screening to $-17.37 \pm -3.53\%$ at week 12, $p = 0.004$ of the trial. Four out of seven patients (57%) with renal involvement met renal response criteria with \geq 50% decrease in 24-hour urine proteinuria.

Median time to organ response was three weeks. There was no change in patients without cardiac involvement. Seventeen of 19 patients studied were alive at follow up of 19 months.

“We are pleased that these data demonstrate CAEL-101’s ability to induce rapid organ responses in patients with AL amyloidosis. We also found that improvement in GLS corresponds with improvement in NT-proBNP in patients with cardiac AL amyloidosis treated with CAEL-101. It is important to note that GLS is a sensitive measure of left ventricular function that is correlated with survival in AL amyloidosis,” said Dr. Bhutani, Assistant Professor of Medicine at CUMC.

The preclinical study explored the diagnostic potential of CAEL-101 radiolabeled with a positron emitting radioisotope for systemic amyloidosis and its use as a companion biomarker to stratify patients for CAEL-101 immunotherapy. CAEL-101 successfully imaged 100% of mice bearing human amyloid extracts of both kappa and lambda subtypes derived from the heart, spleen, liver and kidney. These findings



indicate that using CAEL-101 with positron emission tomography (PET) imaging may facilitate diagnosis of systemic amyloidosis, stratify patients for CAEL-101 immunotherapy and quantify peripheral organ amyloid fibril deposition pre- and post-anti-amyloid therapy. The study of mAb CAEL-101 confirmed the findings of previous studies with the murine monoclonal antibody, which was evaluated in preclinical and clinical studies.

“We successfully used PET imaging to visualize cardiac-derived amyloid fibrils from AL amyloidosis patients, with clinically significant imaging of both kappa and lambda fibrils. Because heart failure is the most critical condition that can impede AL amyloidosis patients’ survival, successfully conducting in vivo imaging of cardiac amyloidosis is essential for diagnostic purposes,” said Dr. Fu, Associate Research Scientist at CUMC. “As a result of our findings, we anticipate that dedicated gated cardiac PET and computerized tomography (CT) imaging of radiolabeled CAEL-101 will be successful in visualizing cardiac amyloid deposits in patients, especially with the rich blood flow in cardiac tissue and newer-generation, highly sensitive, high-resolution digital PET scanners.”

About AL Amyloidosis

AL amyloidosis is a rare systemic disorder caused by an abnormality of plasma cells in the bone marrow. Misfolded amyloid proteins produced by plasma cells cause buildup in and around tissues, nerves and organs, gradually affecting their function. This can cause progressive and widespread organ damage and high mortality rates.

AL amyloidosis affects roughly 30,000 – 40,000 patients in total throughout the U.S. and Europe, and it is estimated that there are approximately 3,000 – 4,000 new cases of AL amyloidosis annually in the U.S., though actual incidence is likely higher as a result of under-diagnosis. Amyloidosis has a one-year mortality rate of 47 percent, 76 percent of which is caused by cardiac amyloidosis.

About CAEL-101 (mAb 11-1F4)

CAEL-101 is a light chain fibril-reactive monoclonal antibody (mAb) that has completed a Phase 1a/1b trial at Columbia University for the treatment of patients with AL amyloidosis. While current treatment with chemotherapy is aimed at reducing production of the amyloid-forming light-chain protein, CAEL-101 attempts to reduce and / or eliminate the amyloid deposits.

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 (mAb 11-1F4), 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 safe and well-tolerated therapy that promotes amyloid resolution. CAEL-101 has received Orphan Drug Designation from the U.S. Food and Drug Administration as a therapeutic agent for patients with AL amyloidosis, and as a radio-imaging agent in amyloidosis. For more information, visit www.caelumbio.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 required by law.

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