Cogent Biosciences Announces Submission of New Drug Application (NDA) for Bezuclastinib in Advanced Systemic Mastocytosis (AdvSM)

June 30, 2026

Submission based on results of APEX pivotal trial which demonstrated bezuclastinib objective response rate (CR+CRh+PR+CI) of 65% per mIWG criteria and 81% ORR per PPR criteria with a well-tolerated safety profile for AdvSM patients

APEX NDA is third submission for bezuclastinib in six months; PEAK and SUMMIT reviews ongoing and on track for anticipated Q4 2026 approval

WALTHAM, Mass. and BOULDER, Colo., June 30, 2026 (GLOBE NEWSWIRE) -- Cogent Biosciences, Inc. (Nasdaq: COGT), a biotechnology company focused on developing precision therapies for genetically defined diseases, today announced it has submitted its New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for bezuclastinib in Advanced Systemic Mastocytosis (AdvSM). The submission is based on positive clinical data from the APEX pivotal trial.

“We are pleased to announce the submission of our NDA for patients with Advanced Systemic Mastocytosis based on the impressive results from the APEX trial which demonstrated that a highly selective KIT mutant inhibitor like bezuclastinib can deliver clear clinical benefit without the severe tolerability challenges associated with currently available treatments,” said Andrew Robbins, President and Chief Executive Officer. “We are working closely with the FDA on all three of our submissions and remain confident that bezuclastinib will achieve its first approvals later this year. I’d also like to congratulate our small, but exceptionally talented, Cogent development team, whose amazing work analyzing, presenting and submitting data from three pivotal trials in less than twelve months has rapidly positioned the company to achieve our vision of creating best-in-class therapies for patients fighting rare, mutational driven diseases.”

The NDA submission is supported by data from the pivotal APEX trial, which were most recently presented at the 2026 European Hematology Association (EHA) Congress. As of the March 31, 2026 data cutoff, 81 AdvSM patients were treated with 150 mg of bezuclastinib, including 57 patients with SM-AHN, 11 patients with ASM and 13 patients with MCL. The primary endpoint of response per mIWG-MRT-ECNM was assessed on 68 evaluable patients and showed 65% ORR (CR+CRh+PR+CI), including 57% of patients who achieved CR, CRh or PR as best response.

Additional highlights included:

- Key secondary endpoint of response per pure pathological response (PPR) criteria was assessed on 81 patients which showed an 81% ORR (CR+CRh+PR).
- Bezuclastinib demonstrated reversal of bone marrow pathobiology including rapid and deep reductions in aberrant CD25 and CD30 expression, normalization of mast cell morphology, normalization of bone marrow cellularity, and improvement in myelofibrosis.
- Bezuclastinib demonstrated durable clinical activity and prolonged PFS with a 12-month PFS rate of 79% and a 12-month OS rate of 87%. Median duration of PFS and OS were immature at the time of the data cutoff.
- Bezuclastinib achieved clear and clinically significant reductions in objective disease markers for these AdvSM patients:

Outcome measure	**Bezuclastinib**
Proportion with ≥50% reduction in serum tryptase (n=80)	89%
Proportion with ≥50% reduction in bone marrow mast cells or clearance of aggregates (n=80)	89%
Proportion with ≥50% reduction in KIT D816V variant allele frequency (n=43)	91%

In the APEX study, bezuclastinib also demonstrated robust improvement in disease pathology, with effects observed as early as eight weeks, including high PPR rates, improvement (including normalization) in bone marrow mast cell distribution, improvement in broader bone marrow characteristics and a majority of patients achieving normalization of serum tryptase. In addition, approximately one-third of patients treated with bezuclastinib achieved undetectable levels of KIT D816V VAF, suggesting modification of the underlying AdvSM disease with bezuclastinib treatment.

As of the data cutoff, bezuclastinib was well-tolerated, with infrequent need for dose reduction or discontinuation for treatment-related adverse events (TRAEs). The most frequent TRAEs reported on bezuclastinib treatment were hair color change (31%), neutropenia (31%), altered taste (28%), thrombocytopenia (25%), and ALT/AST elevations (21%). The majority of transaminase

elevations were of low grade, asymptomatic and reversible. Of the two patients who experienced Grade 3 transaminase elevation, one discontinued treatment and one remains on therapy following dose reduction.

Bezuclastinib - Expanded Access Program

Working with the FDA, Cogent has established active Expanded Access Programs (EAPs) for U.S. patients SM or GIST who meet disease-specific criteria and could benefit from treatment with bezuclastinib or the combination of bezuclastinib and sunitinib. For more information please visit: **https://www.cogentbio.com/bezuclastinib-program-development/#our-expanded-access-policy**.

Inducement Grants Under Nasdaq Listing Rule 5635(c)(4)

Cogent also announced today that, on June 26, 2026, the Compensation Committee of Cogent's Board of Directors, made up entirely of independent directors, approved the grants of "inducement" equity awards to two new employees under the company's 2020 Inducement Plan with a grant date of June 29, 2026. The awards were approved in accordance with Listing Rule 5635(c)(4) of the corporate governance rules of the Nasdaq Stock Market. The employees received, in the aggregate, (i) nonqualified options to purchase 7,600 shares of Cogent common stock and (ii) 7,000 restricted stock units (RSUs). Each option has a 10-year term, an exercise price equal to the closing price of Cogent's common stock on the grant date, and a 4-year vesting schedule with 25% vesting on the 1-year anniversary of the grant date and the remainder vesting in equal monthly installments over the subsequent 36 months, provided such employee remains employed through each such vesting date. The RSUs vest annually in equal installments over 4 years from the grant date, provided such employee remains employed through each such vesting date.

About Cogent Biosciences, Inc.

Cogent Biosciences is a biotechnology company focused on developing precision therapies for genetically defined diseases. The most advanced clinical program, bezuclastinib, is a selective tyrosine kinase inhibitor that is designed to potently inhibit the KIT D816V mutation as well as other mutations in KIT exon 17. KIT D816V is responsible for driving systemic mastocytosis, a serious disease caused by unchecked proliferation of mast cells. Exon 17 mutations are also found in patients with advanced gastrointestinal stromal tumors (GIST), a type of cancer with strong dependence on oncogenic KIT signaling. In addition, the Cogent Research Team is developing a portfolio of novel targeted therapies to help patients fighting serious, genetically driven diseases targeting mutations in ErbB2, PI3Kα, KRAS and JAK2. Cogent Biosciences is based in Waltham, MA and Boulder, CO. Visit our website for more information at **www.cogentbio.com**. Follow Cogent Biosciences on social media: **X** (formerly known as Twitter) and **LinkedIn**. Information that may be important to investors will be routinely posted on our website and **X**.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding: the expectation that both of the company's NDAs for bezuclastinib in GIST and NonAdvSM will be approved by the FDA in the fourth quarter of 2026 and the expectation that the company will achieve its vision of creating best-in-class therapies for patients fighting rare, mutational driven diseases. The use of words such as, but not limited to, "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "might," "plan," "potential," "predict," "project," "should," "target," "will," or "would" and similar words expressions are intended to identify forward-looking statements. Forward-looking statements are neither historical facts nor assurances of future performance. Instead, they are based on our current beliefs, expectations and assumptions regarding the future of our business, future plans and strategies, our clinical results, the rate of enrollment in our clinical trials and other future conditions. New risks and uncertainties may emerge from time to time, and it is not possible to predict all risks and uncertainties. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements. We may not actually achieve the forecasts or milestones disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Such forward-looking statements are subject to a number of material risks and uncertainties including but not limited to those set forth under the caption "Risk Factors" in Cogent's most recent Annual Report on Form 10-K, as supplemented by Quarterly Reports on Form 10-Q and other filings Cogent makes with the SEC from time to time . Any forward-looking statement speaks only as of the date on which it was made. Neither we, nor our affiliates, advisors or representatives, undertake any obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to the date hereof.

Contact:

Christi Waarich
Senior Director, Investor Relations
617-830-1653

christi.waarich@cogentbio.com