ASX Announcement

23 April 2026

FDA Confirms Single Pivotal Trial Pathway for Elate Ocular®

Substantially reduces development cost, accelerates timeline, and increases probability of BLA success

Sydney, Australia; 23 April 2026: Cambium Bio Limited (ASX:CMB) ("Cambium Bio", "Cambium" or "Company"), a clinical-stage regenerative medicine company focused on ophthalmology and tissue repair, is pleased to announce that following a Type D meeting with the U.S. Food and Drug Administration (FDA) held on 22 April 2026, the Agency has confirmed that a single adequate and well-controlled pivotal clinical study, together with confirmatory evidence, is a reasonable approach to support a Biologics License Application (BLA) for Elate Ocular® for the treatment of moderate-to-severe dry eye disease (DED).

This confirmation aligns the Elate Ocular® development programme with the FDA's updated regulatory policy articulated by FDA officials Dr Vinay Prasad and Dr Martin Makary in the *New England Journal of Medicine* on 19 February 2026, which established a single pivotal clinical trial plus confirmatory evidence as the FDA's new default standard for marketing authorisation, replacing the historical two-trial requirement.[1]

Key Highlights

FDA confirms single-trial pathway. Cambium Bio may proceed with one adequate and well-controlled pivotal Phase 3 study, CAMOMILE-3, as the principal clinical evidence package for the Elate Ocular® BLA submission, without a requirement for a second pivotal trial.

Substantial reduction in development cost and accelerated enrolment. Moving from a two-trial to a one-trial pathway materially reduces clinical development capital requirements and is expected to accelerate enrolment by consolidating clinical sites and patient recruitment into one pivotal Phase 3 programme.

Increased probability of success. A single-trial pathway concentrates statistical risk into one well-powered study rather than requiring independent success in two separate pivotal studies, improving the overall probability of generating a successful BLA data package.

Phase 3 design confirmed. The FDA confirmed that the previously agreed randomised, double-masked, vehicle-controlled pivotal study design (n=400 evaluable; co-primary sign and symptom endpoints; 9-week masked treatment period) remains acceptable to support the single-trial BLA pathway.

Fast Track Designation enables rolling BLA submission. Elate Ocular® holds FDA Fast Track Designation (granted 4 December 2024), which allows rolling BLA submission and supplementation of the application as confirmatory evidence is generated.

[1] Prasad V, Makary MA. One Pivotal Trial, the New Default Option for FDA Approval — Ending the Two-Trial Dogma. *N Engl J Med* 2026;394:815–817. DOI: 10.1056/NEJMsb2517623.

Confirmatory evidence strategy to be developed post-readout. The FDA confirmed that the nature and scope of confirmatory evidence will be discussed with the Agency following Phase 3 readout. Cambium Bio is evaluating a range of options, including additional product-specific nonclinical studies, to support Elate Ocular's mechanism of action.

Clear operational focus. The Company's immediate priority is to initiate the single n=400 pivotal Phase 3 study as soon as operationally feasible, with First Patient In expected later in 2026.

FDA Regulatory Update

The Type D meeting held with FDA's Center for Biologics Evaluation and Research (CBER), Office of Therapeutic Products (OTP) on 22 April 2026 addressed two questions central to the Elate Ocular® development programme: (i) whether a single pivotal trial plus confirmatory evidence would provide substantial evidence of effectiveness sufficient to support a BLA submission; and (ii) whether the previously agreed Phase 3 study design remains acceptable.

On question (i), the FDA confirmed in its written preliminary response and reiterated at the meeting that a single-trial plus confirmatory evidence approach is reasonable and aligns with the Agency's current recommendations, with the adequacy of the data to be assessed as a review issue at the time of BLA submission.

On question (ii), the FDA confirmed the existing Phase 3 study design, including the co-primary sign endpoint (Corneal Fluorescein Staining Index) and symptom endpoint (VAS Eye Discomfort Score), n=400 evaluable subjects, 9-week masked treatment period, and vehicle-controlled randomised design.

Key Implications for the Elate Ocular® Development Programme

Lower clinical development capital requirement. A single-trial programme materially reduces the capital required to progress Elate Ocular® to BLA submission compared with the historical two-trial paradigm. The specific capital saved will depend on final operational parameters, and the Company will provide further guidance as the pivotal study commences.

Accelerated time to BLA submission and potential approval. The single-trial pathway removes the requirement to conduct a second pivotal study in parallel, allowing the Company to concentrate clinical sites, investigator attention, and patient recruitment into one pivotal Phase 3 programme. This is expected to accelerate enrolment timelines and reduce the operational complexity associated with running two concurrent studies competing for the same investigator sites and patient population. Cambium Bio now expects to progress Elate Ocular® through a single pivotal study, rolling BLA submission under Fast Track Designation, and potential approval on an accelerated timeframe.

Improved probability of regulatory success. Under the historical two-trial paradigm, a development programme must demonstrate statistical significance independently in two separate pivotal studies. Under the single-trial paradigm, the programme must demonstrate statistical significance in one well-powered study supported by confirmatory evidence. The overall probability of generating a regulatory data package sufficient to support BLA approval is accordingly improved.

Confirmatory Evidence Strategy

The FDA indicated at the 22 April 2026 meeting that discussion of confirmatory evidence is most appropriate following Phase 3 readout, when the strength of the pivotal clinical data will inform the nature and quantity of confirmatory evidence required. Consistent with the September 2023 FDA guidance on demonstrating substantial evidence of effectiveness with one adequate and well-controlled clinical investigation,[2] a range of confirmatory evidence types may be considered, including mechanistic or pharmacodynamic evidence, evidence from relevant animal models, real-world evidence, and other categories.

Cambium Bio is evaluating a range of product-specific confirmatory evidence options to support Elate Ocular's mechanism of action, including additional nonclinical characterisation studies. The specific confirmatory evidence package to be submitted as part of the BLA will be determined in consultation with the FDA following Phase 3 topline data, and will be calibrated to the strength of the pivotal clinical results. Elate Ocular's Fast Track Designation allows the BLA to be submitted and reviewed on a rolling basis, providing flexibility to supplement the application with confirmatory evidence generated during and after the Phase 3 open-label extension.

CEO Commentary

Karolis Rosickas, Chief Executive Officer of Cambium Bio, commented:

"Cambium Bio is very pleased to see the FDA taking a more pragmatic and measured approach to drug development, and we welcome the confirmation that a single pivotal clinical trial plus confirmatory evidence is the appropriate pathway for Elate Ocular®. This represents a substantial acceleration of our development plan, a meaningful reduction in the capital required to reach BLA submission, and an improvement in the overall probability of successfully bringing this therapy to patients in need. Dry eye disease affects tens of millions of people globally, and for the moderate-to-severe segment the treatment options remain limited. The Company's operational focus is now unambiguous: to initiate the single pivotal Phase 3 study as soon as operationally feasible, with First Patient In expected later in 2026, and to drive the programme efficiently toward topline readout and BLA submission."

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About Cambium Bio Limited

Cambium Bio Limited (ASX:CMB) is a Sydney-based clinical-stage regenerative medicine company focusing on the development of innovative biologics for ophthalmology and tissue repair applications. The Company's proprietary technology, based on human platelet lysate, is being leveraged to create a pipeline of novel therapeutics, with a primary focus on ophthalmology. Cambium Bio's lead product candidate, Elate Ocular®, is being developed to address significant unmet medical needs in the treatment of dry eye disease. In addition, the Company's stem cell platform, Progenza™, is being applied to the development of therapies for knee osteoarthritis and other tissue repair indications. Cambium Bio is committed to advancing its pipeline through

[2] FDA Draft Guidance for Industry: *Demonstrating Substantial Evidence of Effectiveness With One Adequate and Well-Controlled Clinical Investigation and Confirmatory Evidence* (September 2023).

clinical development and commercialisation, with the goal of providing transformative treatments to improve patient outcomes. For more information about the Company and its programs, please visit www.cambium.bio

Authorisation & Additional Information

This announcement was authorised by the Board of Directors of Cambium Bio Limited.

Forward-Looking Statements

This announcement contains certain forward-looking statements regarding Cambium Bio Limited's clinical development plans, regulatory pathway, and the expected timing, scope and outcome of the Phase 3 pivotal clinical study for Elate Ocular®. Forward-looking statements involve known and unknown risks, uncertainties and other factors that may cause actual results to differ materially from those expressed or implied. These include, without limitation, the risk that Phase 3 clinical results may not be positive, that the FDA may require additional clinical or nonclinical evidence, that regulatory timelines may be extended, that confirmatory evidence may not be accepted as proposed, and other risks inherent in biopharmaceutical development. Readers are cautioned not to place undue reliance on forward-looking statements, which speak only as of the date of this announcement. The Company undertakes no obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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