Annexon Reports Fourth Quarter and Year-End 2023 Financial Results and Key Anticipated Milestones

March 26, 2024

Pivotal Phase 3 Data for ANX005 in Guillain-Barré Syndrome (GBS) Expected in Second Quarter 2024

Initiation of Pivotal Phase 3 ANX007 ARCHER II Trial in Geographic Atrophy (GA) Expected in mid-2024, a Global Sham-Controlled Trial Using Vision Preservation as Primary Outcome Measure

Clinical Proof-of-Concept Data with ANX1502 Oral Inhibitor of the Classical Pathway Expected in Second Half of 2024

Robust Balance Sheet with Cash, Cash Equivalents, and Short-term Investments of Approximately $260 Million as of December 31, 2023, and Anticipated Runway into mid-2026

BRISBANE, Calif., March 26, 2024 (GLOBE NEWSWIRE) -- Annexon, Inc. (Nasdaq: ANNX), a biopharmaceutical company advancing a late-stage clinical platform of novel therapies for people living with devastating classical complement-mediated neuroinflammatory diseases of the body, brain, and eye, today highlighted portfolio progress and reported fourth quarter and full year 2023 financial results.

“Our strong execution in 2023 across our three priority programs successfully achieved key clinical and regulatory milestones and created a solid foundation from which to reach several important upcoming catalysts in 2024,” said Douglas Love, president and chief executive officer of Annexon. “Importantly, our approach in blocking C1q to stop classical complement-driven neuroinflammation in diseases of the body, brain and eye continues to show unique and consistent functional outcomes across our pipeline programs.”

Mr. Love continued, “In the year ahead, we are excited by ANX005’s near-term potential to be the first targeted treatment for GBS, and we are on track to report topline data from our pivotal, placebo-controlled trial in the second quarter. For our global registrational program in GA, we plan to initiate two pivotal trials in 2024 to underscore the unique mechanism of action of ANX007 and its competitive differentiation in visual function. Finally, we anticipate clinical proof-of-concept data from our novel oral inhibitor ANX1502 later in the year. With our balance sheet strengthened and anticipated runway into mid-2026, we are poised to generate significant near- to mid-term value for patients and our shareholders.”

Recent Corporate and Clinical Program Updates

Successful $125 Million Financing: In December 2023, Annexon announced a $125 million financing and extension of operating runway into mid-2026.

Flagship Programs

ANX005 in GBS: First-in-class monoclonal antibody designed to block C1q and the entire classical complement pathway in both the body and the brain.

- Completed enrollment of randomized, double-blind, placebo-controlled, multi-center Phase 3 clinical trial (N=241) designed to evaluate the efficacy, safety, pharmacokinetics, and pharmacodynamics of ANX005 in patients with GBS.
- Initiated a real-world evidence (RWE) comparability protocol with International Guillain-Barré Syndrome Outcomes Study (IGOS), with initial RWE data expected in the first half of 2025 to support a planned Biologics License Application (BLA) submission. IGOS is a global, prospective, observational, multicenter cohort study that has enrolled ~2000 patients who were followed for one to three years.
- Granted Fast Track and orphan drug designations from the U.S. Food and Drug Administration (FDA).
- Granted orphan drug designation from the European Medicines Agency (EMA) based on a meta-analysis of past studies with ANX005 and intravenous immunoglobulin (IVIg) demonstrating notable, early improvement in muscle strength with ANX005 that translated into observable gains in health status, including a reduction in the need of mechanical ventilation.
- Hosted an R&D Day on the burden of disease, current treatment paradigm and market opportunity for GBS in March 2024.

ANX007 in GA: First-in-class, non-pegylated antigen-binding fragment (Fab) designed to block C1q and activation of the classical complement cascade locally in the eye with an intravitreal formulation.

- Gained alignment with FDA on persistent best corrected visual acuity (BCVA) ≥ 15-letter loss as primary outcome measure and trial with comparison to an injection agent; no requirement to evaluate lesion growth, a surrogate anatomical endpoint for vision used previously for the approval of other GA programs.
    - Pivotal primary endpoint alignment is based on the randomized, sham-controlled Phase 2 ARCHER trial demonstrating ANX007 is the first and only program reported to show statistically significant and consistent protection against vision loss in a broad population of patients with GA.
- Granted Fast Track designation from the FDA.
- Granted Priority Medicine (PRIME) designation by the EMA, the first and only therapeutic candidate for the treatment of GA to receive such a designation.

ANX1502 in Cold Agglutinin Disease (CAD): First-in-class oral small molecule inhibitor of the classical complement pathway designed to target chronic autoimmune diseases.

- Announced Phase 1 single-ascending dose (SAD), and multiple-ascending dose (MAD) data from a clinical trial in healthy volunteers evaluating the safety, tolerability, pharmacokinetics, or PK, and pharmacodynamics. Key findings include:
  - ANX1502 was generally well tolerated across cohorts with no serious adverse events.
  - Target levels of active drug achieved in healthy volunteers with oral twice daily dosing.
  - Supportive impact on pharmacodynamic biomarker of complement activity that supports clinical advancement.

Next Wave Programs

ANX005 in Amyotrophic Lateral Sclerosis (ALS): First-in-class monoclonal antibody designed to block C1q and the entire classical complement pathway.

- Completed a signal-finding open-label Phase 2a study evaluating the safety and tolerability of chronic dosing of ANX005 in patients with ALS. Key topline findings include:
  - Study met all primary objectives, demonstrating ANX005 was generally well-tolerated, displayed rapid and sustained full C1q inhibition, and reduced downstream pharmacodynamic complement markers.
  - Consistent with what has been shown in other neurodegenerative diseases, including Huntington's disease, exploratory analyses indicated that patients with higher baseline classical complement activation who enrolled within 12 months of diagnosis achieved better outcomes, including less functional decline on the Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R) and stabilization of neurofilament light chain (NfL).
  - These analyses support a precision medicine approach to identify patients most likely to respond to anti-C1q therapy in clinical trials with recently diagnosed patients with ALS who have elevated baseline levels of classical complement activity.
  - Annexon plans to submit the Phase 2a data for presentation at an upcoming medical conference later this year.

Key 2024 Anticipated Milestones for Flagship Programs

- **ANX005 in GBS:** Topline data from pivotal, randomized, placebo-controlled Phase 3 trial expected in the second quarter of 2024. Initial data from RWE comparability protocol with IGOS expected in first half 2025 to support a planned BLA submission.

- **ANX007 in GA:** Global pivotal Phase 3 ARCHER II trial vs. sham control expected to initiate in mid-2024. Pivotal Phase 3 head-to-head ARROW trial vs. SYFOVRE® (pegcetacoplan injection) planned to initiate in the second half of 2024.

- **ANX1502 in CAD:** Proof-of-concept (POC) trial evaluating the pharmacodynamics and efficacy of an oral tablet formulation in CAD anticipated to provide initial data in the second half of 2024, with plans for program expansion in autoimmune indications upon completion of clinical POC.

Fourth Quarter and Full Year 2023 Financial Results

- **Cash and operating runway:** Cash and cash equivalents and short-term investments were $259.7 million as of December 31, 2023. Annexon continues to expect its cash, cash equivalents and marketable securities as of December 31, 2023, to be sufficient to fund the company's planned operating expenses into mid-2026.
- **Research and development (R&D) expenses:** R&D expenses were $23.3 million for the quarter ended December 31, 2023, and $113.8 million for the year ended December 31, 2023, reflecting the advancement of the Company's priority programs, including GBS, GA and ANX1502, compared to $28.5 million for the quarter ended December 31, 2022, and $112.5 million for the year ended December 31, 2022.
- **General and administrative (G&A) expenses:** G&A expenses were $6.7 million for the quarter ended December 31, 2023, and $30.0 million for the year ended December 31, 2023, compared to $8.2 million for the quarter ended December 31, 2022, and $33.1 million for the year ended December 31, 2022.
- **Net loss:** Net loss was $27.9 million for the quarter ended December 31, 2023, and $134.2 million for the year ended December 31, 2023, compared to $34.4 million for the quarter ended December 31, 2022, and $141.9 million for the year ended December 31, 2022.

About Annexon

Annexon Biosciences (Nasdaq: ANNX) is a biopharmaceutical company advancing a late-stage clinical platform of novel therapies for people living with devastating classical complement-mediated neuroinflammatory diseases of the body, brain, and eye. Annexon's novel scientific approach targets upstream C1q to block the classical complement inflammatory cascade before it starts, and its therapeutic candidates are designed to provide meaningful benefits across multiple autoimmune, neurodegenerative and ophthalmic diseases. With proof-of concept data in Guillain-Barré syndrome, Huntington's disease and geographic atrophy, Annexon is rigorously advancing its mid-to late-stage clinical trials to bring new potential treatments to patients as quickly as possible. To learn more visit annexonbio.com.

Forward Looking Statements

This press release contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. In some cases, you can identify forward-looking statements by terminology such as “aim,” “anticipate,” “assume,” “believe,” “contemplate,” “continue,” “could,” “design,” “due,” “estimate,” “expect,” “goal,” “intend,” “may,” “objective,” “plan,” “positioned,” “potential,” “predict,” “seek,” “should,” “target,” “will,” “would” and other similar expressions that are predictions of or indicate future events and future trends, or the negative of these terms or other comparable terminology. All statements other than statements of historical facts contained in this press release are forward-looking statements. These forward-looking statements include, but are not limited to, statements about: the timing of completion of Phase 3 trial of ANX005 in patients with GBS; the potential therapeutic benefit of ANX005; potential benefit of ANX005, if approved, compared to existing therapies; anticipated timing of the completion of a RWE comparability study and BLA submission for ANX005; timing of initiation of the ARCHER II and ARROW trials; ANX007’s distinct potential neuroprotective mechanism of action and potential to provide protection from vision loss; timing of proof-of-concept data for ANX1502;; continued development of ANX007 and ANX1502;; anticipated cash runway into mid-2026; the potential benefits from treatment with anti-C1q therapy; and continuing advancement of the company’s portfolio. Forward-looking statements are not guarantees of future performance and are subject to risks and uncertainties that could cause actual results and events to differ materially from those anticipated, including, but not limited to, risks and uncertainties related to: the ongoing off-treatment follow-up portion of the ARCHER trial and final results from the ARCHER trial; the company’s history of net operating losses; the company’s ability to obtain necessary capital to fund its clinical programs; the early stages of clinical development of the company’s product candidates; the effects of public health crises on the company’s clinical programs and business operations; the company’s ability to obtain regulatory approval of and successfully commercialize its product candidates; any undesirable side effects or other properties of the company’s product candidates; the company’s reliance on third-party suppliers and manufacturers; the outcomes of any future collaboration agreements; and the company’s ability to adequately maintain intellectual property rights for its product candidates. These and other risks are described in greater detail under the section titled “Risk Factors” contained in the company’s Annual Report on Form 10-K and Quarterly Reports on Form 10-Q and the company’s other filings with the SEC. Any forward-looking statements that the company makes in this press release are made pursuant to the Private Securities Litigation Reform Act of 1995, as amended, and speak only as of the date of this press release. Except as required by law, the company undertakes no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise.

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ANNEXON, INC.
Condensed Consolidated Statements of Operations
(in thousands, except share and per share amounts)

	Three Months Ended December 31,		Year Ended December 31,	
	2023	2022	2023	2022
	(unaudited)			
Operating expenses:				
Research and development (1)	$ 23,267	$ 28,535	$ 113,756	$ 112,501
General and administrative (1)	6,742	8,160	29,967	33,098
Total operating expenses	30,009	36,695	143,723	145,599
Loss from operations	(30,009)	(36,695)	(143,723)	(145,599)
Interest and other income, net	2,118	2,312	9,486	3,652
Net loss	$ (27,891)	$ (34,383)	$ (134,237)	$ (141,947)
Net loss per share, basic and diluted	$ (0.36)	$ (0.48)	$ (1.77)	$ (2.60)
Weighted-average shares used in computing net loss per share, basic and diluted	78,217,945	72,368,539	75,673,081	54,673,572
(1) Includes the following stock-based compensation expense:				
Research and development	$ 2,077	$ 2,365	$ 8,878	$ 8,874
General and administrative	$ 2,290	$ 2,468	$ 9,305	$ 9,642

ANNEXON, INC.
Condensed Consolidated Balance Sheets
(in thousands)

	December 31,	
	2023	2022
Assets		
Current assets:		
Cash and cash equivalents	$ 225,110	$ 140,020
Short-term investments	34,606	102,637
Prepaid expenses and other current assets	4,144	5,441
Total current assets	263,860	248,098
Restricted cash	1,032	1,032
Property and equipment, net	14,773	16,838
Operating lease right-of-use assets	18,009	19,128
Total assets	$ 297,674	$ 285,096
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 5,487	$ 7,416
Accrued liabilities	10,235	13,448
Operating lease liabilities, current	2,165	1,316
Other current liabilities	41	180
Total current liabilities	17,928	22,360
Operating lease liabilities, non-current	29,190	31,542
Total liabilities	47,118	53,902
Stockholders' equity:		
Common stock	78	48
Additional paid-in capital	823,029	669,780
Accumulated other comprehensive loss	(52)	(372)
Accumulated deficit	(572,499)	(438,262)
Total stockholders' equity	250,556	231,194
Total liabilities and stockholders' equity	$ 297,674	$ 285,096