Annexon Reports Fourth Quarter and Year-End 2024 Financial Results, Portfolio Progress and Key Anticipated Milestones

March 3, 2025

Robust, Consistent Phase 3 Data and Real-World Evidence Outcomes Support ANX005 as Potential First Targeted Therapy for GBS; Pre-BLA Meeting Targeted for 1H 2025 Ahead of Planned Biologics License Application (BLA) Submission

Established Groundbreaking Global Registration Path for ANX007 to be Potential First Vision-Preserving Treatment for Dry AMD with GA in Europe and U.S.; Topline Phase 3 ARCHER II Data Expected Second Half 2026

First-in-Kind Oral C1s Inhibitor POC Trial Ongoing with Dosing in Three Patients with Cold Agglutin Disease; Expanded ANX1502 Dataset to Include Up to Seven Patients Expected Mid-2025

Strong Balance Sheet with Cash, Cash Equivalents, and Short-term Investments of Approximately $312 Million as of December 31, 2024, and Anticipated Runway into Second Half 2026

BRISBANE, Calif., March 03, 2025 (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 2024 financial results.

“Our 10-year journey has focused on disrupting the classical complement space by delivering multiple transformative treatments for neuroinflammatory diseases of the body, brain and eye. Importantly, our three flagship programs are showing tremendous promise to be game-changing, best-in-class therapies in their respective blockbuster therapeutic markets,” said Douglas Love, president and chief executive officer of Annexon. “Our lead program, ANX005 for the treatment of Guillain-Barré Syndrome (GBS), has consistently demonstrated early and durable functional improvements with a differentiated safety profile, offering the potential to help the tens of thousands of patients annually whose lives are suddenly turned upside down by this devastating disease. We look forward to the pre-BLA meeting with FDA targeted for the first half of 2025 ahead of our BLA submission.”

Mr. Love continued, “We are also enthusiastic about recent positive regulatory engagements resulting in a groundbreaking global registration path for ANX007 to become the potential first drug approved in Europe and the U.S. for dry age-related macular degeneration (AMD) with geographic atrophy (GA) based on the global Phase 3 ARCHER II program. As demonstrated in the Phase 2 ARCHER trial, ANX007 is the only program to have shown significant vision preservation as well as significant preservation of central retina photoreceptor neurons responsible for visual acuity. Finally, in patients treated with the oral small molecule ANX1502, we are encouraged by the changes in multiple measures of hemolysis consistent with complement inhibition. Our ongoing ANX1502 proof-of-concept (POC) trial is designed to build a robust dataset in up to seven patients with data expected mid-2025. Strong execution continues across our portfolio, and we are well-positioned for a breakthrough year.”

Recent Corporate and Clinical Program Updates

Flagship Programs

ANX005 in Guillain-Barré Syndrome (GBS): First-in-kind monoclonal antibody designed to block C1q, the initiating molecule of the classical complement cascade, with a single infusion to halt ongoing neuroinflammation and nerve damage in the acute phase of disease with potential to be the first targeted therapy for GBS.

- A robust data package has been generated with close regulatory guidance over the nine year development path that includes a placebo-controlled Phase 1b trial that established POC for ANX005 as a first-line treatment for GBS, a successful Phase 3 trial showing that ANX005 was generally well tolerated and resulted in faster and more complete functional recovery versus placebo, a Real-World Evidence (RWE) study that showed improved outcomes against current standards of care in matched patient populations, and a drug-drug interaction study with ANX005 and intravenous immunoglobulin (IVIg) strengthening the safety profile for ANX005 in GBS
- Phase 3 data to be featured in an oral presentation on Tuesday, April 8 at the upcoming American Academy of Neurology (AAN) 2025 Annual Meeting taking place April 5–9, 2025, in San Diego, California. Abstracts for AAN will be released on Thursday, March 6
- GBS is a rare, neuromuscular emergency that affects at least 150,000 people worldwide each year, with no FDA-approved therapy. The life-altering consequences of GBS were recently detailed in a new patient survey released by the GBS/CIDP Foundation that underscores the significant unmet need notwithstanding standard of care
- **Next Milestone:** Pre-BLA meeting targeted for first half of 2025 ahead of planned BLA submission

ANX007 in Dry Age-Related Macular Degeneration (AMD) Patients with Geographic Atrophy (GA): First-in-kind, non-pegylated antigen-binding fragment (Fab) designed to block C1q and the classical complement cascade locally in the eye.

- Following positive engagement with EU and U.S. regulators regarding the Phase 3 ARCHER II trial design, groundbreaking global registration path established supporting the potential of ANX007 to be the first treatment approved in both Europe

and the U.S. for protection of vision in patients who have dry AMD with GA. ANX007 is also the only program to receive PRIME designation from the European Medicines Agency for GA

- ANX007 is the first and only investigational therapy to show significant vision preservation on assessments of best corrected visual acuity (BCVA) and low luminance visual acuity, demonstrating significant protection from vision loss in both normal and low light conditions, as well as significant preservation of central retinal photoreceptors necessary for visual acuity as demonstrated in the Phase 2 ARCHER trial
- ARCHER II is a global, sham-controlled, double-masked, Phase 3 trial enrolling approximately 630 patients who have dry AMD with GA. The single-study program will be analyzed as two sub-studies for the U.S. in accordance with the FDA's two-trial recommendation. The primary endpoint of ARCHER II is prevention of ≥15-letter loss of BCVA, and an objective secondary structural measure is prevention of ellipsoid zone loss, both of which measures were met in the Phase 2 ARCHER trial. Accordingly, Annexon no longer plans to conduct a second injection-controlled head-to-head Phase 3 trial
- Dry AMD with GA is a leading cause of blindness affecting more than 8 million patients worldwide, and there are no approved therapies targeting the preservation of vision in this disease
- **Next Milestone:** Phase 3 ARCHER II trial enrollment expected to be completed in second half of 2025; data expected in second half of 2026

ANX1502 for Autoimmune Conditions: First-in-kind oral small molecule inhibiting the activated form of C1s, an enzyme associated with C1q to drive initiation of the classical cascade, has the potential to offer the advantages of selective upstream classical complement inhibition with the convenience and flexibility of oral administration.

- An ongoing open-label, single arm, POC study is evaluating ANX1502 in patients with cold agglutinin disease (CAD) for up to four weeks to evaluate tolerability, pharmacokinetics, pharmacodynamic and clinical efficacy endpoints (e.g., hemolysis as measured by reduction of elevated bilirubin)
- Enteric coated tablets allow flexible dosing 4-5-times above target concentrations of 100nM for rigorous testing in CAD, a classical complement-mediated disease
- Three patients enrolled to date with observed reduction in key clinical and biomarker outcomes consistent with complement inhibition; awaiting full data
- **Next Milestone:** Dataset in up to seven CAD patients to be reported in mid-2025

Expanded board of directors with addition of William "BJ" Jones, M.B.A: Mr. Jones brings 30 years of U.S. and global commercial and launch experience in the biotechnology industry. Mr. Jones joins Annexon with demonstrated commercial success at both large pharmaceutical and small biotechnology companies, with experience driving mass-market product launch strategies for industry-leading brands. He currently serves as the chief commercial officer of NewAmsterdam Pharma Company N.V. where he leads all commercial functions, including marketing, market access, sales, medical science engagement and commercial operations.

Fourth Quarter and Full Year 2024 Financial Results

- **Cash and operating runway:** Cash, cash equivalents and short-term investments were $312.0 million as of December 31, 2024. Annexon continues to expect its cash, cash equivalents and short-term investments as of December 31, 2024, to be sufficient to fund its planned operating expenses into the second half of 2026
- **Research and development (R&D) expenses:** R&D expenses were $43.4 million for the quarter ended December 31, 2024, and $119.4 million for the year ended December 31, 2024, reflecting the advancement of Annexon's priority programs, including GBS, dry AMD with GA and ANX1502, compared to $23.3 million for the quarter ended December 31, 2023, and $113.8 million for the year ended December 31, 2023
- **General and administrative (G&A) expenses:** G&A expenses were $9.1 million for the quarter ended December 31, 2024, and $34.6 million for the year ended December 31, 2024, compared to $6.7 million for the quarter ended December 31, 2023, and $30.0 million for the year ended December 31, 2023
- **Net loss:** Net loss was $48.6 million for the quarter ended December 31, 2024, and $138.2 million for the year ended December 31, 2024, compared to $27.9 million for the quarter ended December 31, 2023, and $134.2 million for the year ended December 31, 2023

About Annexon

Annexon Biosciences (Nasdaq: ANNX) is developing therapeutics that stop classical complement-driven neurodegeneration as first-in-kind treatments for millions of people living with serious neuroinflammatory diseases of the body, brain and eye. Our novel scientific approach focuses on C1q, the initiating molecule of classical complement's potent inflammatory pathway that when misdirected can lead to tissue damage and loss. By targeting C1q, our immunotherapies are designed to stop this neuroinflammatory cascade in disease before it starts. Our pipeline spans three diverse therapeutic areas – autoimmune, neurodegenerative and ophthalmic diseases – and includes targeted investigational drug candidates designed to address the unmet needs of over 8 million people worldwide. Annexon's mission is to deliver game-changing therapies to patients so that they can live their best lives. 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 potential therapeutic benefit of ANX005, if approved, compared to existing therapies; anticipated timing of the pre-BLA meeting and BLA submission for ANX005; potential benefit of ANX005, if approved, compared to intravenous immunoglobulin /plasma exchange or other existing therapies; the company’s ability to achieve regulatory approval for ANX005; the potential therapeutic benefit of ANX007; timing of completion of enrollment and results from the Phase 3 ARCHER II trial; ANX007’s distinct potential neuroprotective mechanism of action and potential to provide protection from vision loss; the potential for ANX007 to be the first drug approved in Europe and the U.S. for dry AMD with GA; timing of proof-of-concept data for ANX1502; the company’s ability to commercialize its product candidates, if approved; continued development of ANX007 and ANX1502; anticipated cash runway into the second half of 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 final results from the Phase 3 ARCHER II 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,	
	2024	2023	2024	2023
Operating expenses:				
Research and development (1)	$ 43,354	$ 23,267	$ 119,448	$ 113,756
General and administrative (1)	9,125	6,742	34,625	29,967
Total operating expenses	52,479	30,009	154,073	143,723
Loss from operations	(52,479)	(30,009)	(154,073)	(143,723)
Interest and other income, net	3,889	2,118	15,873	9,486
Net loss	$ (48,590)	$ (27,891)	$ (138,200)	$ (134,237)
Net loss per share, basic and diluted	$ (0.33)	$ (0.36)	$ (1.01)	$ (1.77)
Weighted-average shares used in computing net loss per share, basic and diluted	147,812,160	78,217,945	137,404,145	75,673,081

(1) Includes the following stock-based compensation expense:

Research and development	$ 2,752	$ 2,077	$ 9,670	$ 8,878
General and administrative	$ 2,470	$ 2,290	$ 9,763	$ 9,305

ANNEXON, INC.
Condensed Consolidated Balance Sheets

(in thousands)

	December 31	
	2024	**2023**
Assets		
Current assets:		
Cash and cash equivalents	$ 49,498	$ 225,110
Short-term investments	262,519	34,606
Prepaid expenses and other current assets	4,444	4,144
Total current assets	316,461	263,860
Restricted cash	1,032	1,032
Property and equipment, net	12,638	14,773
Operating lease right-of-use assets	16,705	18,009
Other non-current assets	3,235	—
Total assets	$ 350,071	$ 297,674
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 10,426	$ 5,487
Accrued and other current liabilities	17,568	10,276
Operating lease liabilities, current	2,518	2,165
Total current liabilities	30,512	17,928
Operating lease liabilities, non-current	26,454	29,190
Total liabilities	56,966	47,118
Stockholders' equity:		
Common stock	109	78
Additional paid-in capital	1,003,685	823,029
Accumulated other comprehensive loss	10	(52)
Accumulated deficit	(710,699)	(572,499)
Total stockholders' equity	293,105	250,556
Total liabilities and stockholders' equity	$ 350,071	$ 297,674