Annexon Reports Second Quarter 2024 Portfolio and Financial Results, and Key Anticipated Milestones

August 12, 2024

Single, Well-Tolerated Infusion of ANX005 Significantly Accelerated Recovery of GBS Patients vs. Placebo in Pivotal Phase 3 Trial; Potential to be First Targeted Therapy for GBS; Topline Real-World Evidence (RWE) Comparability Data Now Expected by Year-End 2024

Dosing Initiated in ARCHER II Registrational Trial for ANX007; Only Program to Demonstrate Significant Vision Protection and Structural Protection in Regions of the Eye Important for Vision Acuity in GA; Phase 3 Data Expected Second Half 2026

First-in-Kind Oral C1s Inhibitor ANX1502 Successfully Completed Bridging Study to Twice-Daily Tablet; Proof-of-Concept Data in Autoimmune Disease Expected Q4 2024

Robust Balance Sheet with Cash, Cash Equivalents, and Short-term Investments of Approximately $368.7 Million as of June 30, 2024, and Anticipated Runway into Second Half 2026

BRISBANE, Calif., Aug. 12, 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 second quarter 2024 financial results.

“Annexon is at the cross section where decades of pioneering research and development are translating into clinical wins for scores of patients. This was exemplified in the first half of 2024 by the robust functional and biomarker data from two of our flagship programs: Guillain-Barré syndrome (GBS) and geographic atrophy (GA). These data further highlight the powerful disease modifying potential of our platform approach by blocking neuroinflammation where it starts in diverse acute and chronic conditions of high unmet need,” said Douglas Love, president and chief executive officer of Annexon. “In that regard, ANX005 helped GBS patients suffering from an acute neuromuscular emergency get better sooner and more completely in the first placebo-controlled pivotal study in 40 years. Moreover, ANX007 is the only program shown to help GA patients significantly preserve their vision while protecting associated retina structures critical for vision. We’re also pleased to report that the bridging study for our oral inhibitor ANX1502, the first clinical stage oral inhibitor of the classical pathway, has completed and the safety and pharmacokinetics profile confirmed the findings from the healthy volunteer trial.”

Mr. Love continued, “With these data, we’re actively working to build on our strong momentum across our flagship programs with several key catalysts over the next 24-months that have the potential to drive significant benefit for millions of patients and our shareholders. Importantly, we now expect earlier topline data by year-end 2024 for our real-world evidence comparability study for ANX005 in GBS to support our BLA submission targeted for the first half of 2025. Additionally, our ~630 patient potentially best-in-disease global Phase 3 GA program has been initiated, and we anticipate pivotal data in the second half of 2026. Lastly, we remain on pace for proof-of-concept data for ANX1502 in Cold Agglutinin Disease (CAD) in the latter part of the year, which has the potential to enable advancement in an array of autoimmune indications. With a strong cash position, we are more excited than ever and remain sharply focused on delivering multiple first-in-kind targeted therapies to improve the lives of millions of patients living with devastating neuroinflammatory diseases.”

Recent Clinical Program Updates

Flagship Programs

ANX005 in Guillain-Barré syndrome (GBS): First-in-kind monoclonal antibody designed to block C1q and the entire classical complement pathway in both the body and the brain.

- ANX005 demonstrated a highly clinically relevant and statistically significant effect on multiple measures of the primary endpoint GBS-DS; positive Phase 3 topline results and additional analyses reported at the June 2024 Peripheral Nerve Society (PNS) Annual Meeting
- Increased likelihood of being in a better state of health seen by week 1, and observed at all subsequent time points through 26-weeks
- Early, robust and durable treatment effects expedited recovery and led to patients walking and off ventilation approximately one month earlier
- Single infusion of ANX005 was generally well-tolerated with a profile similar to placebo and adverse events balanced across groups
- Patients with baseline characteristics similar to those of patients in North America and Europe had greater responses to ANX005 over placebo
- Initiated a real-world evidence (RWE) comparability study with global experts in GBS using the International Guillain-Barré Syndrome Outcomes Study (IGOS), a global, prospective, observational, multicenter cohort study that has enrolled 2,000 patients who were followed for one to three years

ANX007 in Geographic Atrophy (GA): First-in-kind, 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.

- Patient dosing initiated in the global registrational Phase 3 ARCHER II trial, a well-powered, sham-controlled study with a robust safety database expected to enroll approximately 630 patients
- Regulatory alignment with the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) on key study elements of Phase 3 program, including first use of best corrected visual acuity (BCVA) protection against ≥15-letter loss as the primary outcome measure in GA
- New Phase 2 ARCHER data on the protection of vision and vision-related retinal structure presented at the 2024 American Society of Retina Specialists (ASRS) Annual Scientific meeting
- ANX007 is the only investigational medicine for GA to date to show significant vision protection in both standard and low light conditions, and protection of photoreceptors in the central fovea, the region of the retina needed for important activities such as reading, driving and recognizing faces
- Treatments for GA have primarily focused on addressing lesion growth by measuring the protection of supportive retinal pigment epithelium (RPE) and have not translated to protection of clinically meaningful vision for patients

ANX1502 for Autoimmune Conditions: First-in-kind oral small molecule inhibitor of the classical complement pathway designed to target chronic autoimmune diseases.

- Completed bridging study in healthy volunteers from a liquid suspension formulation to a twice daily tablet with safety and pharmacokinetic profile similar or better than previous studies
- Ongoing proof-of-concept (POC) trial in CAD will characterize pharmacodynamics on complement and hemolysis measures, and is designed to enable advancement in multiple antibody-mediated autoimmune indications, many with clinical validation and treatments approved for weekly or every other week infusions
- ANX1502 has the potential to offer the advantages of selective upstream classical complement inhibition with the convenience and dosing flexibility of oral administration

Key Anticipated Milestones for Flagship Programs

- **ANX005 in GBS:** Initial topline data from RWE comparability protocol with IGOS now expected by year-end 2024 to support a planned BLA submission in the first half of 2025
- **ANX007 in GA:** Phase 3 ARCHER II trial topline data expected in the second half of 2026. Plans for an injection-controlled study, ARROW, to assess the prevention of ≥15-letter loss of BCVA, are also ongoing
- **ANX1502 in CAD:** POC trial evaluating the pharmacodynamics and efficacy of an oral tablet formulation in CAD anticipated to provide initial data in the fourth quarter of 2024

Second Quarter 2024 Financial Results

- **Cash and operating runway:** Cash, cash equivalents and short-term investments were $368.7 million as of June 30, 2024. Annexon continues to expect its cash, cash equivalents and short-term investments as of June 30, 2024, to be sufficient to fund the company's planned operating expenses into the second half of 2026
- **Research and development (R&D) expenses:** R&D expenses were $25.0 million for the quarter ended June 30, 2024, reflecting the advancement of the Company's priority programs, including GBS, GA and ANX1502, compared to $30.3 million for the quarter ended June 30, 2023
- **General and administrative (G&A) expenses:** G&A expenses were $8.6 million for the quarter ended June 30, 2024, compared to $7.4 million for the quarter ended June 30, 2023
- **Net loss:** Net loss was $29.6 million or $0.23 per share for the quarter ended June 30, 2024, compared to $35.2 million or $0.47 per share for the quarter ended June 30, 2023

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, if approved, compared to existing therapies; anticipated timing of the completion of a RWE comparability study and BLA submission for ANX005; the potential therapeutic benefit of ANX007; timing of the ARCHER II trial and initiation of ARROW trial; 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 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 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 June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Operating expenses:				
Research and development (1)	$ 25,026	$ 30,251	$ 45,989	$ 62,596
General and administrative (1)	8,554	7,440	16,163	16,337
Total operating expenses	33,580	37,691	62,152	78,933
Loss from operations	(33,580)	(37,691)	(62,152)	(78,933)
Interest and other income, net	3,970	2,503	7,366	5,069
Net loss	$ (29,610)	$ (35,188)	$ (54,786)	$ (73,864)
Net loss per share, basic and diluted	$ (0.23)	$ (0.47)	$ (0.43)	$ (0.99)
Weighted-average shares used in computing net loss per share, basic and diluted	130,132,960	75,230,003	126,403,081	74,546,995
(1) Includes the following stock-based compensation expense:				
Research and development	$ 2,311	$ 2,307	$ 4,593	$ 4,558
General and administrative	$ 2,631	$ 2,353	$ 5,009	$ 4,709

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

	June 30, 2024	December 31, 2023
	(unaudited)	
Assets		

Current assets:		
Cash and cash equivalents	$ 157,304	$ 225,110
Short-term investments	211,395	34,606
Prepaid expenses and other current assets	5,270	4,144
Total current assets	373,969	263,860
Restricted cash	1,032	1,032
Property and equipment, net	13,702	14,773
Operating lease right-of-use assets	17,382	18,009
Total assets	$ 406,085	$ 297,674
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 4,126	$ 5,487
Accrued liabilities	10,657	10,235
Operating lease liabilities, current	2,345	2,165
Other current liabilities	20	41
Total current liabilities	17,148	17,928
Operating lease liabilities, non-current	27,858	29,190
Total liabilities	45,006	47,118
Stockholders' equity:		
Common stock	106	78
Additional paid-in capital	988,347	823,029
Accumulated other comprehensive loss	(89)	(52)
Accumulated deficit	(627,285)	(572,499)
Total stockholders' equity	361,079	250,556
Total liabilities and stockholders' equity	$ 406,085	$ 297,674