

Axonis Therapeutics Announces \$115 Million Series A Financing

-- Oversubscribed financing brings together leading syndicate of life sciences investors --

-- Proceeds to support clinical development of first-in-class therapies for neurological disorders --

BOSTON, Mass., October 30, 2024 – Axonis Therapeutics, a biotechnology company focused on the development of novel neuromedicines, announced today the successful completion of an oversubscribed \$115 million Series A financing. Proceeds from the financing will be used to advance Axonis’ lead development candidate, AXN-027, through clinical proof-of-concept in patients. AXN-027 is a first-in-class oral small molecule designed to potentiate the function of KCC2, a major CNS chloride transporter essential for inhibitory neurotransmission, for the treatment of epilepsy and pain. Proceeds will also enable the development of next generation compounds targeting KCC2 in these and other indications, including psychiatric and neurodevelopmental disorders.

Cormorant Asset Management and venBio Partners co-led the financing with significant investments from Sofinnova Investments, MRL Ventures Fund, Perceptive Advisors, Lumira Ventures and Solasta Ventures. In conjunction with this financing, Axonis named Raymond J. Kelleher, M.D., Ph.D., Managing Director at Cormorant Asset Management and Jonathan Leff, M.D., Executive Partner, Private Equity, from Sofinnova Investments to its Board of Directors. Eos Bioinnovation, Hatteras Venture Partners, SCI Ventures, Alexandria Venture Investments, Tachyon Ventures, BoxOne, Iaso Ventures and Pathway Bioventures also participated in the financing.

“Axonis’ vision is to advance a pipeline of oral KCC2 therapeutics to restore functional inhibition in the CNS to treat neurological disorders without disabling side effects,” said Joanna Stanicka, Ph.D., Chief Executive Officer, President, and co-founder of Axonis. “As we head into the clinic later this year, this financing is a significant milestone for Axonis as we translate our exciting science into efficacious and well-tolerated treatments for patients suffering from neurological disorders.”

“We’re delighted that this accomplished group of investors share our mission to translate Axonis’ science into impactful medicines,” said Corey Goodman, Ph.D., Executive Chair and co-founder of Axonis. “I’m pleased to see how far the science has progressed at Axonis; what the team has accomplished is remarkable. We are now well positioned to bring our lead therapeutic program through clinical proof of concept and expand into related arenas and build a leading biotechnology company.”

Despite the approval of several drugs for epilepsy and pain, many patients do not tolerate or respond to current first-line therapies due to impaired synaptic inhibition, which can contribute to

drug resistance. Axonis' oral KCC2 potentiators are designed to restore functional inhibition and overcome drug resistance in patients.

"I'm enthusiastic about the potential of Axonis' novel precision approach to re-shape how we treat patients with neurological disorders, particularly epilepsy and pain where modulation of synaptic inhibition has great therapeutic promise," said Raymond J. Kelleher, M.D., Ph.D., Managing Director at Cormorant. "Axonis' scientists have done amazing work. We're very excited about what this program could mean for patients and their families."

Leadership Team

Axonis is led by Joanna Stanicka, Ph.D., co-founder and co-inventor of Axonis' underlying IP. She is a Harvard-trained scientist, drug hunter, and entrepreneur, and a recipient of the Massachusetts Next Generation Award, a prize in leadership for woman-CEO in life sciences. Shane Hegarty, Ph.D., is the company's Chief Scientific Officer, co-founder, and IP co-inventor. He was previously a Research Fellow at Harvard Medical School and Boston Children's Hospital, faculty member in Neuroscience Department of University College Cork, and recipient of the Neuroscience Ireland Investigator Award.

In conjunction with the financing, Axonis also announces the appointments of Donald Manning, MD, Ph.D., as Chief Medical Officer and Jeff Imbaro, M.B.A., as Chief Operating Officer. Dr. Manning is a clinician-scientist with comprehensive experience in acute and chronic clinical practice, research and management. He brings to Axonis over 25 years of executive experience, having overseen the filings of seven INDs and five NDAs that have led to nine approved products. Mr. Imbaro brings over 20 years of company building expertise, having led Business Development and General and Administrative functions of several successful biotech and pharmaceutical companies. He was a member of the founding team of Semma Therapeutics, which was later acquired by Vertex Pharmaceuticals in 2019 for nearly \$1 billion.

About Axonis Therapeutics

Axonis Therapeutics is a neuro-focused biotechnology company developing first- and best-in-class medicines targeting KCC2, the critical mediator of inhibitory neurotransmission within the brain, by translating breakthrough discoveries spun out from Boston Children's Hospital, Harvard and Université Laval. The company has built a proprietary KCC2 discovery engine, based on several years of world-leading know-how, to become the leaders in this potential blockbuster drug space. Axonis is grateful for grant awards and support received from National Institutes of Health, Department of Defense SCIRP, ISS National Lab, CURE Epilepsy, Wings for Life, Massachusetts Life Center, SynGAP Research Fund, Praxis Spinal Cord Institute, Creative Destruction Lab, Springboard Enterprises and MassChallenge. The company is headquartered in Boston, MA. For more information, visit www.axonis.us.

About venBio Partners

Since inception in 2011, venBio has raised roughly \$2 billion in capital commitments and led investment rounds in 40+ companies, including Labrys Biologics (acquired by Teva), Aragon Pharmaceuticals (acquired by Johnson & Johnson), Seragon Pharmaceuticals (acquired by Roche), Aurinia Pharmaceuticals (NASDAQ: AUPH), Apellis Pharmaceuticals (NASDAQ: APLS), Checkmate Pharmaceuticals (NASDAQ: CMPI, acquired by Regeneron), Turning Point Therapeutics (NASDAQ: TPTX, acquired by BMS), Akero Therapeutics (NASDAQ: AKRO), Harmony Biosciences (NASDAQ: HRMY), Pharvaris (NASDAQ: PHVS), CinCor Pharma (NASDAQ: CINC, acquired by Astra Zeneca), RayzeBio (NASDAQ: RYZB, acquired by BMS), and many others. For more information, visit the venBio website at www.venbio.com and follow on LinkedIn.

About Cormorant Asset Management

Founded in 2013, Cormorant Asset Management, LP ("Cormorant") is a leading life sciences-focused investment firm with over \$3 billion of assets under management. Cormorant is driven by a deep focus on fundamental scientific principles and provides financial resources to support the most innovative and promising publicly traded and private companies in biotech and allied sectors. Cormorant is among the most active crossover investors in life sciences, with investments in over 100 privately held, life science-focused companies during the past decade.

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Media Contact

Argot Partners

Sarah Sutton

sarah@argotpartners.com