



Gene Therapy Company AavantiBio Launches with \$107 Million Series A Financing from Perceptive Advisors, Bain Capital Life Sciences, RA Capital Management and Sarepta Therapeutics

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- *AavantiBio will pursue a diversified gene therapy pipeline, including a lead program in Friedreich's Ataxia (FA), a rare inherited genetic disease characterized by cardiac and central nervous system dysfunction*
- *Bo Cumbo, most recently Sarepta's Chief Commercial Officer and Executive Vice President, appointed Chief Executive Officer and President of AavantiBio*
- *Co-founded by pioneering gene therapy researchers Barry Byrne, M.D., Ph.D., and Manuela Corti, P.T., Ph.D., AavantiBio will maintain strong ties to the University of Florida's renowned Powell Gene Therapy Center*

CAMBRIDGE, Mass., Oct. 22, 2020 (GLOBE NEWSWIRE) -- A premier syndicate of life sciences investors including Perceptive Advisors, Bain Capital Life Sciences ("Bain Capital"), and RA Capital Management ("RA Capital") (collectively the "Investor Group") together with Sarepta Therapeutics, Inc. ("Sarepta") (NASDAQ: SRPT), a leader in precision genetic medicine for rare diseases, today announced a \$107 million Series A financing to create AavantiBio, a gene therapy company focused on transforming the lives of patients with rare genetic diseases. The private financing round includes a \$15 million equity investment from Sarepta.

Alexander "Bo" Cumbo, a seasoned biopharmaceutical executive, has been appointed AavantiBio's Chief Executive Officer and President, effective immediately. Mr. Cumbo joins the Company after eight years at Sarepta, where he served as Chief Commercial Officer and Executive Vice President. He will continue to serve as an advisor to Sarepta through December 31, 2020.

Headquartered in the greater Boston area, AavantiBio is co-founded by renowned gene therapy researchers Barry Byrne, M.D., Ph.D., and Manuela Corti, P.T., Ph.D., who together bring thirty years of experience to the Company. AavantiBio's lead program is in Friedreich's Ataxia (FA), a rare inherited genetic disease that causes cardiac and central nervous system dysfunction. AavantiBio's research efforts expand on foundational research conducted by Drs. Byrne and Corti in Friedreich's Ataxia, among other rare genetic disorders. AavantiBio will benefit from strategic partnerships with the University of Florida's renowned Powell Gene Therapy Center and the MDA Care Center at UF Health where Drs. Byrne and Corti maintain their research and clinical practices. Initial funding in AavantiBio was provided by GoFAR, an Italian patient advocacy group, and the Muscular Dystrophy Association Venture Philanthropy Fund.

"The past eight years have been some of the most rewarding of my career as Sarepta has emerged as a pioneer for Duchenne muscular dystrophy and limb-girdle muscular dystrophy patients and transformed into a genetic medicine leader," Mr. Cumbo stated. "It has been a privilege to contribute to this growth and play a role in serving these communities. As I look ahead to the bright future of AavantiBio and the exciting opportunity to lead this innovative Company, this same dedication to serving unmet patient needs and to leveraging deep scientific expertise will be core to our mission. I am also thrilled to continue to collaborate with the talented team at Sarepta."

"Our equity participation in AavantiBio serves our strategy to build our gene therapy engine through targeted investment in potentially life-enhancing therapies as well as partnering with renowned genetic medicine pioneers such as Drs. Byrne and Corti," stated Doug Ingram, president and CEO, Sarepta Therapeutics. Mr. Ingram continued: "Bo Cumbo has built a first-in-class rare disease commercial organization and has made tremendous contributions to Sarepta. We look forward to continuing to work with Bo as he builds a strong AavantiBio team and advances therapies to treat FA and other rare diseases."

Prior to joining Sarepta in 2013, Mr. Cumbo served as Vice President of Sales and Treatment Education at Vertex Pharmaceuticals where he built a sales force to market their first commercial drug, Incivek—an oral treatment for hepatitis C. Previously, he spent nine years at Gilead Sciences where he served in multiple commercial roles supporting the company's HIV, HBV, and cardiovascular franchises. Mr. Cumbo has extensive experience building global commercial infrastructure, and throughout his career, he has contributed to the launch of 11 specialty products across multiple organizations. He received his Bachelor of Science in Laboratory Technology from Auburn University.

"AavantiBio has a unique opportunity to change the lives of those living with FA and other rare diseases," Mr. Cumbo added. "Central to our mission is building on the advancements in gene transfer therapies and harnessing these revolutionary technologies to realize their potential as life-altering medicines. I'm excited to build a company focused on deep science, innovation, and collaboration, and grateful for the partnership with three of the biotechnology industry's most respected investors in Perceptive Advisors, Bain Capital, and RA Capital."

"Our investment in AavantiBio represents a truly unique opportunity to thoughtfully advance a differentiated pipeline of innovative gene transfer therapies in areas of high unmet medical need," the Investor Group said in a statement. "We are excited to collaborate with Bo Cumbo, an accomplished executive leader, and to provide the necessary resources to enable the Company to build a best-in-class gene therapy platform that brings transformative products to patients and families impacted by these devastating diseases."

The AavantiBio Board of Directors will be comprised of 8 Directors, including Mr. Cumbo, Drs. Byrne and Corti, representatives of the Investor Group, and two Independent Directors. Louise Rodino-Klapac, Ph.D., senior vice president of gene therapy for Sarepta, will serve as a Board Observer.

About Friedreich's Ataxia

Friedreich's Ataxia (also known as FA or Friedreich Ataxia) is a rare inherited genetic disease that causes movement problems and nervous system damage. Over time, the disease leads to degeneration in the spinal cord, peripheral nerves and cerebellum (the part of the brain that controls

synchronization and balance) and causes impaired muscle coordination (ataxia) that gets worse over time. Poor coordination is one of the first noticeable features of FA. The neurological degeneration caused by the disease results in unsteady movements, impaired sensory function, and even the loss of speech. Affected individuals can also develop heart problems, diabetes, or curvature of the spine. However, the disorder does not affect cognitive ability. Though rare, FA affects 1 in every 40,000-50,000 people and is the most common form of hereditary ataxia in the United States.

About AavantiBio, Inc.

AavantiBio's vision is to harness the transformative science of gene transfer therapy and gene editing technologies to improve the lives of people living with fatal diseases. Founded by distinguished gene therapy researchers out of the University of Florida, the Company is advancing a pipeline of innovative gene transfer therapies in areas of high unmet medical need, which have the potential to become the next generation of life-changing medicines. Learn more at: www.aavantibio.com.

About Sarepta Therapeutics

At Sarepta, we are leading a revolution in precision genetic medicine and every day is an opportunity to change the lives of people living with rare disease. The Company has built an impressive position in Duchenne muscular dystrophy (DMD) and in gene therapies for limb-girdle muscular dystrophies (LGMDs), mucopolysaccharidosis type IIIA, Charcot-Marie-Tooth (CMT), and other CNS-related disorders, with more than 40 programs in various stages of development. The Company's programs and research focus span several therapeutic modalities, including RNA, gene therapy and gene editing. For more information, please visit www.sarepta.com or follow us on [Twitter](#), [LinkedIn](#), [Instagram](#) and [Facebook](#).

About Perceptive Advisors

Founded in 1999 and based in New York, NY, Perceptive Advisors is an investment management firm focused on supporting the progress of the life sciences industry by identifying opportunities and directing financial resources to the most promising technologies in healthcare. For more information visit www.perceptivelife.com.

About Bain Capital Life Sciences

Bain Capital Life Sciences (www.baincapitallifesciences.com) pursues investments in biopharmaceutical, specialty pharmaceutical, medical device, diagnostics and enabling life science technology companies globally. The team focuses on companies that both drive medical innovation across the value chain and enable that innovation to improve the lives of patients with unmet medical needs. Since 1984, Bain Capital has developed global reach, deep expertise and a proven track record in life sciences industries across its Private Equity, Credit, Public Equity, Venture and Real Estate business units.

About RA Capital Management

RA Capital is a multi-stage investment manager dedicated to evidence-based investing in public and private healthcare and life science companies that are developing drugs, medical devices, and diagnostics. The flexibility of its strategy allows RA Capital to provide seed funding to startups and to lead private, IPO, and follow-on financings for its portfolio companies, both facilitating the crossover process and allowing management teams to drive value creation from inception through commercialization.

Sarepta Forward-Looking Statements

This press release contains "forward-looking statements." Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements. These forward-looking statements include statements regarding *AavantiBio's plan to pursue a diversified gene therapy pipeline; the expectation that AavantiBio will maintain strong ties to the University of Florida's Powell Gene Therapy Center; AavantiBio's potential to benefit from strategic partnerships with the University of Florida's Powell Gene Therapy Center and the MDA Care Center at UF Health; AavantiBio's mission to build on the advancements in gene transfer therapies and harness revolutionary technologies to realize their potential as life-altering medicines; AavantiBio's potential to build a best-in-class gene therapy platform that brings transformative products to patients and families impacted by devastating diseases; and AavantiBio's potential to become the next generation of life-changing medicines.*

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others: the expected benefits and opportunities related to the investment in AavantiBio may not be realized or may take longer to realize than expected due to challenges and uncertainties inherent in product research and development; the investment in AavantiBio may not result in any viable treatments suitable for clinical research or commercialization due to a variety of reasons including the results of future research may not be consistent with past positive results or may fail to meet regulatory approval requirements for the safety and efficacy of product candidates or may never become commercialized products due to other various reasons including any potential future inability of the parties to fulfill their commitments and obligations under the agreements, including any inability by Sarepta to fulfill its financial commitments to AavantiBio; even if the investment results in new commercialized products, Sarepta may not achieve any significant revenues from the sale of such products; and those risks identified under the heading "Risk Factors" in Sarepta's most recent Annual Report on Form 10-K for the year ended December 31, 2019, and most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) as well as other SEC filings made by the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect the Company's business, results of operations and the trading price of Sarepta's common stock. For a detailed description of risks and uncertainties Sarepta faces, you are encouraged to review the SEC filings made by Sarepta. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof.

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