Expansion Therapeutics Raises $55.3 Million in Series A Financing to Advance Portfolio of Novel RNA Targeted Small Molecule Medicines to Treat Rare Diseases

- *Names Kevin M. Forrest, Ph.D. President and CEO*
- *Proprietary technology based on pioneering work from the lab of Matthew D. Disney, Ph.D. of The Scripps Research Institute Florida*

San Diego, January 3, 2018 -- Expansion Therapeutics, Inc., a new 5AM Ventures formed private company focused on the discovery and development of ribonucleic acid (RNA) targeted small molecule medicines, announced today the close of a $55.3 million Series A financing co-led by 5AM Ventures, Kleiner Perkins, Novartis Venture Fund, and Sanofi Ventures with participation from RA Capital Management and Alexandria Venture Investments. Proceeds will advance Expansion's portfolio of small molecule drugs targeting key human disease-driving RNAs with an initial focus on expansion repeat disorders, a set of approximately 30 genetic diseases that currently have no satisfactory treatments.

"In a short period of time Expansion has assembled a leading team, the key scientific founder in Matt Disney of The Scripps Research Institute Florida, and a capital efficient plan to advance the emerging field of RNA targeted small molecule medicines," said Scott M. Rocklage, Ph.D., Managing Partner of 5AM Ventures and founding investor and Chairman of the Board of Directors of Expansion Therapeutics. "We look forward to continuing to work with the company to develop medicines for patients with few treatment options."

"The science underpinning Expansion's RNA targeted medicines offers great promise in the treatment of a set of RNA triggered diseases, such as myotonic dystrophy, that currently have no viable therapies. We are excited to invest in a company that could make a real difference to patients suffering from these incurable diseases," said Beth Seidenberg, M.D., General Partner of Kleiner Perkins.

Coincident with the close of the Series A, Expansion co-founder Kevin M. Forrest, Ph.D., was named president and chief executive officer. Dr. Forrest previously served as founding chief operating officer and chief financial officer for the San Diego-based anti-infectives company Cidara Therapeutics (Nasdaq: CDTX). Prior, he was a Principal at 5AM Ventures. Dr. Forrest holds a B.S. in biology from Boston College and a Ph.D. in molecular biology from Princeton University where he published on various RNA regulatory processes.

RNA Targeted Small Molecule Medicines

Incubated within 5AM Ventures' 4:59 Initiative, and subsequently seed funded by 5AM and Sanofi Ventures, Expansion's approach is based on key patent-protected platform technologies and enabling tools pioneered in the laboratory of Dr. Disney of The Scripps Research Institute, who is the leader in the field of small molecule targeting of RNA.

"I am gratified that our efforts over the past dozen years have culminated in this important opportunity," said Dr. Disney. "It is clear that disease-related RNA is now an addressable target with

small molecule medicines and we are now on the verge of developing treatments for patients with the most urgent medical needs. We will work tirelessly to fulfill this promise."

Expansion repeat disorders include myotonic dystrophy type I (DM1), which is the leading cause of adult onset muscular dystrophy. "Expansion repeat disorders, in particular DM1, represent an attractive first application of our technology as it is well established that toxic RNA drives disease," said Dr. Forrest. "Furthermore, our small molecule approach has the potential to address both peripheral and central symptoms that are debilitating for patients."

Expansion Names Board of Directors and Forms Scientific Advisory Board

Following the close of the financing, the Expansion board of directors will include:

- Scott M. Rocklage, Ph.D., of 5AM Ventures and Chairman of the Expansion Board of Directors
- Matthew D. Disney, Ph.D., of The Scripps Research Institute
- Kevin M. Forrest, Ph.D., of Expansion Therapeutics
- Jason P. Hafler, Ph.D., of Sanofi Ventures
- Yujiro S. Hata, M.B.A., of Ideaya Biosciences
- Campbell Murray, M.D., M.B.A., M.P.P., of Novartis Venture Fund
- Beth Seidenberg, M.D., of Kleiner Perkins
- Andrew Levin, M.D., Ph.D., of RA Capital Management joins as a board observer

Expansion has also formed a scientific advisory board comprised of leaders in the field of RNA targeted small molecule chemistry and biology, RNA folding, and structural biology, including:

- Robert T. Batey, Ph.D., of the University Colorado, Boulder
- Dale L. Boger, Ph.D., of The Scripps Research Institute
- Ronald R. Breaker, Ph.D., of the Howard Hughes Medical Institute and Yale University
- M.G. Finn, Ph.D., of the Georgia Institute of Technology
- David H. Mathews, M.D., Ph.D., of the University of Rochester
- Michael Zuker, Ph.D., of the Rensselaer Polytechnic Institute

About RNA Targeted Small Molecule Medicines

Ribonucleic acid, or RNA, is a biomolecule that was once thought to be a simple messenger between DNA, or deoxyribonucleic acid, and protein. Recent advances in biology, however, have shown that RNA plays a much greater role than previously appreciated. This includes control of gene expression via long non-coding RNAs, RNA stability via small interfering RNAs, RNA translation via transfer RNAs and microRNAs, and even cellular communication (RNA exosomes). RNA can form higher order structures that form the basis of "druggable" surfaces and pockets that can be targeted by small molecule therapeutics. The best examples of RNA targeted small molecule medicines include several FDA approved classes of antibiotics that bind and inactivate key structures in bacterial RNAs.

About Myotonic Dystrophy Type I

Myotonic dystrophy type I (DM1) affects at least 1 in 8,000 people, or 40,000 individuals in the U.S. alone, and is the most frequent cause of adult onset muscular dystrophy. DM1 is caused by a toxic expansion in RNA, which leads to multi-systemic symptoms including muscular, cardiac, respiratory, gastrointestinal, endocrine, and central nervous system defects. This genetic disease often affects entire families, with progressively worsening disease across generations, and there are no effective treatment options available for DM1.

For more information about myotonic dystrophy type I, visit www.myotonic.org.

About Expansion Therapeutics, Inc.

Expansion Therapeutics is a drug discovery and development company pursuing the vast potential of small molecule medicines for RNA-mediated diseases. Based on exclusive worldwide rights to groundbreaking research from the laboratory of Matthew D. Disney, Ph.D., at The Scripps Research Institute, Expansion has assembled the intellectual property, know-how, and proprietary enabling technologies and tools necessary to facilitate the creation of potent and specific small molecule binders of RNA. Through this unique platform, Expansion is building a portfolio of novel RNA-targeted drug candidates with activity across a broad number of disease indications. The company's initial development focus is on therapies for patients with expansion repeat diseases who currently have limited and unsatisfactory treatment options. Expansion is based in San Diego, California and Jupiter, Florida.

For more information, visit www.expansionrx.com.

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