



## **Expansion Therapeutics Raises \$80 Million Series B Financing to Treat Neurodegenerative Diseases**

*-- Funding to advance the company's SMiRNA™ platform to identify clinical candidates in myotonic dystrophy type 1 (DM1), amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD) and tauopathies*

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BOSTON--([BUSINESS WIRE](#))--Expansion Therapeutics, Inc., a biotechnology company focused on developing transformative oral medicines for severe RNA-mediated diseases, today announced the close of an \$80 million Series B financing. Cormorant Asset Management led the financing with participation from new investors Westlake Village BioPartners, Surveyor Capital (a Citadel company) and Logos Capital as well as Series A investors RA Capital Management, 5AM Ventures, Kleiner Perkins, Sanofi Ventures and Novartis Venture Fund. Proceeds from the financing will be used to advance the company's small molecule RNA platform (SMiRNA™) to identify clinical candidates in myotonic dystrophy type 1 (DM1), amyotrophic lateral sclerosis (ALS), frontotemporal dementia (FTD) and various tauopathies.

"Drugging RNA with small molecules has the potential to dramatically transform the lives of patients and we are excited to be supported by world-class investors who share our commitment to patients and our vision to develop innovative therapies to treat severe RNA-mediated diseases, including neurodegenerative and neurological disorders," said Renato Skerlj, Ph.D., President and Chief Executive Officer of Expansion Therapeutics. "This financing milestone demonstrates significant investor confidence in the leadership team, the science and our mission to accelerate the preclinical and clinical development of our novel medicines utilizing our proprietary approach."

Expansion focuses on structured RNA targets that are evolutionarily conserved or genetically demonstrated to cause disease. Disease-driving RNA structures amenable to ligand binding are identified by combining patient genetics with deep informatic analysis and then screened against Expansion's proprietary RNA-focused libraries for small molecules that modulate the RNA target in a functionally relevant manner. Expansion's novel structural biology SMiRNA™ platform is then utilized for the design and advancement of lead-like chemical matter that ultimately delivers an oral medicine to treat disease. Expansion is focused on neurological diseases where few treatment options are available for patients such as DM1 which is a system wide disorder affecting muscle, cardiac and brain function. A small molecule medicine that treats all aspects of the disease offers patients a significant benefit.

"Recent advances in understanding RNA structure and the science of targeting RNA with small molecules offer tremendous opportunities to transform the way RNA-mediated diseases are treated. We believe Expansion's established focus on structured RNA targets position them as leaders in this emerging space," said Andy Phillips, Ph.D., Managing

Director at Cormorant. “We’re extremely enthusiastic about the promise that the SMiRNA™ platform holds to deliver disease-modifying therapies for a wide range of devastating neurological disorders, particularly neurodegenerative diseases,” added Raymond J. Kelleher, M.D., Ph.D., also Managing Director at Cormorant.

“The progression of Expansion’s lead programs, broad therapeutic potential of the platform, and the leadership team’s demonstrated track record in the field of neuroscience compelled us to partner with this exceptionally strong group of drug developers, as well as Cormorant and the rest of the investor syndicate,” said Andrew Levin, M.D., Ph.D., Managing Director at RA Capital Management. “We are confident in Expansion’s ability to leverage its differentiated platform to positively impact the lives of patients suffering from severe and debilitating neurological diseases who currently have limited or no treatment options,” added Laura Tadvalkar Ph.D., Principal at RA Capital Management.

As part of its initiative to broaden its portfolio of small molecule medicines, Expansion also recently in-licensed two new research programs from Scripps Research, including a program targeting tau, an important driver of dementia disorders. The tau program builds on the observation that defective pre-mRNA encoding the production of tau protein is involved in the formation of neurofibrillary tangles and has been linked to tauopathies such as Alzheimer’s disease, frontotemporal dementias, progressive supranuclear palsy and other neurodegenerative diseases.

Most recently, Expansion’s scientific founder, Matthew D. Disney, Ph.D. and colleague, Alicia Angelbello, Ph.D., were recipients of the American Chemical Society’s Nobel Laureate Signature award for their pioneering work developing RNA-targeting medications for genetic diseases, including DM1.

### **About Expansion Therapeutics**

Expansion Therapeutics is a biotechnology company focused on developing transformative oral medicines for severe RNA-mediated diseases including neurodegenerative disorders. Based on exclusive worldwide rights to groundbreaking research from the laboratory of Matthew D. Disney, Ph.D., at Scripps Research, 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 RNA modulators. Through this unique platform, Expansion is building a portfolio of novel RNA-targeted drug candidates with activity across a broad number of severe RNA-mediated disease indications. Headquartered in Boston, Massachusetts, Expansion’s research facility is located in Jupiter, Florida. For more information, visit [www.expansionrx.com](http://www.expansionrx.com).

## **Contacts**

### **Media:**

Mike Beyer

Sam Brown Inc.

312-961-2502

[mikebeyer@sambrown.com](mailto:mikebeyer@sambrown.com)