

Expansion Therapeutics Names Renato Skerlj, Ph.D., Chief Executive Officer and President

Experienced life sciences executive to lead Expansion as it advances portfolio of medicines targeting RNA-mediated diseases into the clinic

San Diego, October 5, 2020 -- Expansion Therapeutics, Inc., a biotechnology company advancing transformative medicines to patients with RNA-mediated diseases, today announced the appointment of Renato Skerlj, Ph.D., as the company's new Chief Executive Officer and President, effective immediately. Dr. Skerlj will also serve on the company's Board of Directors.

"Renato has a distinguished and proven track record in discovering and developing small molecule drugs for rare diseases which will be invaluable to Expansion as we move our programs targeting expansion repeat disorders toward the clinic," said Scott M. Rocklage, Ph.D., Founding Partner at 5AM Ventures, and Chairman of the Board of Directors of Expansion. "Renato shares our vision to bring transformative therapies to patients with severe RNA-mediated diseases."

"I am extremely excited about the potential of Expansion's pipeline to deliver upon an unmet need for therapies that target a range of serious diseases caused by RNA. I look forward to working with the Expansion team to advance the pioneering work from the lab of Dr. Matt Disney to help patients suffering from these debilitating diseases, such as myotonic dystrophy, for which there are currently no approved therapies," added Dr. Skerlj.

Dr. Skerlj has more than 25 years of experience leading the discovery and development of disease modifying small molecule drugs to treat genetically defined rare diseases. He has authored 67 publications, holds 52 patents and has advanced multiple drug candidates into clinical development.

Most recently, Dr. Skerlj served as Chief Scientific Officer for X4 Pharmaceuticals, Inc., where he also was one of the company's scientific founders and co-inventor of its lead drug candidate, mavorixafor. Prior to joining X4, Dr. Skerlj held leadership roles in drug discovery and development at Lysosomal Therapeutics where he co-invented LTI-291, a CNS drug in clinical development for Parkinson's disease. Previous to that, he was interim Head of Small Molecule Discovery at Genzyme and part of the executive team at AnorMED, a publicly traded company that was acquired by Genzyme in 2006. Dr. Skerlj is an accomplished drug developer with two approved drugs; plerixafor, a CXCR4-targeted stem cell mobilizer approved by the U.S. Food and Drug Administration (FDA) in 2008, and ertapenem, an anti-bacterial approved by the FDA in 2001. Dr. Skerlj received his Ph.D. in synthetic organic chemistry from the University of British Columbia, and completed postdoctoral fellowships at the University of Oxford and Ohio State University.

About Expansion Therapeutics

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 have limited and unsatisfactory treatment options. Expansion is relocating its corporate headquarters to the Boston, Massachusetts area and will maintain its research facility in Jupiter, Florida. For more information, visit www.expansionrx.com.

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