



August 25, 2008 08:03 AM Eastern Daylight Time

Proteostasis Therapeutics Secures a \$45 Million Series A Financing to Develop Proteostasis Regulator™ Drugs

CAMBRIDGE, Mass.--([BUSINESS WIRE](#))--Proteostasis Therapeutics, Inc. (PTI) today announced that it has secured \$45 million in a Series A financing to develop Proteostasis Regulator™ drugs as novel therapies to treat multiple genetic and degenerative disorders associated with protein homeostasis deficiencies. Investors included HealthCare Ventures, Fidelity Biosciences, New Enterprise Associates, Novartis Option Fund and Genzyme Ventures.

“Proteostasis Therapeutics brings together innovative discoveries, leading scientists and a foundation of intellectual property related to the Proteostasis Network™ and Proteostasis Regulators arising from Northwestern University, the Salk Institute and the Scripps Research Institute,” commented David D. Pendergast, Ph.D., CEO of PTI. “Proteostasis Therapeutics is uniquely positioned to commercialize this fundamentally new way of thinking about therapeutic intervention for a broad range of diseases.”

“The Company has established a map of the Proteostasis Network, leading to breakthroughs in understanding how stress on the network, including aging, environmental and cellular stresses, can lead to the onset of multiple serious diseases,” stated Andrew Dillin, Ph.D., co-founder of PTI. “With this new knowledge, PTI has identified unique targets as well as small molecules that offer the promise of restoring the balance of the network while preventing the toxicity of damaged proteins associated with disease,” continued Richard Morimoto, Ph.D., co-founder of PTI.

“Proteostasis Therapeutics has discovered a new frontier in medicine with the identification of several classes of small molecule Proteostasis Regulators that can precisely rebalance the capacity of the Proteostasis Network,” commented Jeffery W. Kelly, Ph.D., co-founder of PTI. “These cell-permeable small molecules exhibit the capacity to ameliorate multiple genetic, degenerative and metabolic diseases, spanning nearly all the classical therapeutic areas.”

“Our initial funding of PTI over a year ago provided the basis for this emerging area of science to mature so that it now offers the promise of new drug candidates entering the clinic in the next 3-5 years,” said Christopher Mirabelli, Ph.D., Managing Director of HealthCare Ventures and Chairman of the Board of PTI. “The significant Series A round is designed to support PTI’s strategy to advance proprietary compounds into the clinic, as well as to continue to develop the unique technology platform that will serve as the basis for strategic partnerships.”

Joining Drs. Pendergast and Mirabelli on the Board of Directors of Proteostasis Therapeutics and representing the new investors are Stephen Knight, M.D., Managing Partner, Fidelity Biosciences; James Barrett, Ph.D., General Partner at New Enterprise Associates; Lauren Silverman, Ph.D., Managing Director, Novartis Option Fund; and Alan Walts, Ph.D., Managing Director, Genzyme Ventures.

About the Founding Team

[David D. Pendergast, Ph.D.](#)

Dr. Pendergast was formerly the President of Human Genetics Therapies at Shire Pharmaceuticals plc, and has more than 30 years of experience in pharmaceutical development and corporate operations. In his position at Shire, Dr. Pendergast was responsible for the discovery, development, manufacture and commercialization of protein therapeutics. Prior to joining Shire, Dr. Pendergast served as Chief Executive Officer of Transkaryotic Therapies, Inc. (acquired by Shire in 2005), where he managed the company during the acquisition, and Chief Operating Officer, where he oversaw operational functions ranging from discovery to commercialization of

protein therapies. Dr. Pendergast was also previously Vice President of Product Development and Quality at Biogen Inc. (now Biogen Idec) and held senior positions at Fisons Ltd. Pharmaceutical Division and at The Upjohn Company (now part of Pfizer). Dr. Pendergast is also Chairman of Altus Pharmaceuticals.

Andrew Dillin, Ph.D., Founder

Andrew Dillin is currently Associate Professor and Pioneer Developmental Chair in the Molecular and Cell Biology Laboratory at the Salk Institute, where he is focused on researching the process of aging as well as the etiology of neurodegenerative diseases. Dr. Dillin recently identified a key regulatory pathway essential for the response to dietary restriction mediated longevity, a key pathway for proteostasis regulation. He received his Ph.D. degree in molecular and cell biology at the University of California at Berkeley, and carried out postdoctoral studies at the University of California in San Francisco defining the genetic pathways required for successful aging.

Jeffery W. Kelly, Ph.D., Founder

Jeffery W. Kelly is the Lita Annenberg Hazen Professor of Chemistry at The Scripps Research Institute, where his research is focused on understanding protein homeostasis, as well as developing new therapeutic strategies for genetic diseases associated with loss-of-function and neurodegenerative diseases associated with protein aggregation. He co-founded FoldRx Pharmaceuticals. Dr. Kelly received his Ph.D. in organic chemistry from the University of North Carolina at Chapel Hill and performed postdoctoral research at The Rockefeller University in the area of chemistry and biology.

Richard I. Morimoto, Ph.D., Founder

Richard I. Morimoto is the Bill and Gayle Cook Professor of Biology, Professor of Biochemistry, Molecular Biology and Cell Biology, and Director of the Rice Institute for Biomedical Research at Northwestern University. He received his Ph.D. in Biology from the University of Chicago and did postdoctoral research at Harvard University in Cambridge. His research on the regulation of the heat shock stress response and the function of molecular chaperones addresses questions on the integration of organismal stress in response to physiologic and environmental stress and the chronic expression of misfolded and damaged proteins.

About the Proteostasis Network™

The Proteostasis Network is comprised of over a dozen biological pathways that maintain a critical balance among protein synthesis, folding, aggregation, trafficking and degradation to ensure health. When functioning properly, the Proteostasis Network ensures that every protein in the cell will either reach its final destination in a fully functional state or be eliminated to prevent damage. Stress on the Proteostasis Network from aging, genetic or environmental insults can lead to imbalances that result in serious diseases. The maintenance of the Proteostasis Network is as important to health and well-being as maintenance of the genome. Thus, therapeutics that can control the Proteostasis Network have the potential to change the practice of medicine.

About Proteostasis Regulators

Advances in our ability to characterize and pharmacologically control protein homeostasis, or the Proteostasis Network, create new opportunities to ameliorate a wide range of diseases. It is now possible to use orally available small molecules, or Proteostasis Regulators, to restore the natural balance of the Proteostasis Network. It has been demonstrated that Proteostasis Regulators can fold mutated proteins, potentially offering a significant benefit to patients suffering from genetic diseases. Scientists have also shown that Proteostasis Regulators can control other biological pathways within the Proteostasis Network to ameliorate degenerative diseases associated with protein aggregation, such as Alzheimer's and Huntington's diseases.

About Proteostasis Therapeutics, Inc.

Proteostasis Therapeutics is discovering and developing novel small molecule therapeutics designed to control the body's protein homeostasis, or Proteostasis Network. The Proteostasis Network maintains the body's natural balance of proteins to protect us from numerous diseases. These novel therapies, or Proteostasis Regulators, are designed to treat multiple genetic and degenerative disorders associated with deficiencies of the Proteostasis Network, such as emphysema, type II diabetes, Alzheimer's Disease and Huntington's Disease. www.proteostasis.com

Contacts

MacDougall Biomedical Communications
Douglas MacDougall or Jennifer Greenleaf, 781-235-3060

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