Proteostasis Therapeutics Announces Appointments of Cystic Fibrosis Experts to Inaugural Clinical Advisory Board

Cambridge, Mass., October 20, 2014 -- Proteostasis Therapeutics, Inc., a company developing novel therapeutics to address diseases caused by defects in protein folding, trafficking and clearance, today announced the appointment of six of the world's leading cystic fibrosis experts to form its clinical advisory board. The newly appointed board will serve as a strategic resource for the upcoming selection and study of the Company's leading compounds that double the activity of the most effective combination, ivacaftor/lumacaftor in the gold-standard HBE cell assay, for the most common mutation, F508del/F508del, found in the cystic fibrosis population.

"We are honored that these world renowned cystic fibrosis clinicians have agreed to bring their expertise to form our team of clinical advisors," said Meenu Chhabra, President and Chief Executive Officer of Proteostasis Therapeutics. "The knowledge and experience that our inaugural clinical advisory board members bring to Proteostasis will be invaluable not only in the selection process of which of our proprietary PTI CFTR compounds should proceed to clinical development, but also in providing guidance on the design and conduct of the future clinical trials of selected drug candidates."

Dr. Richard B. Moss, MD, a Professor Emeritus of Pediatrics at the Lucile Salter Packard Children's Hospital at Stanford University, will serve as Chairman of the Clinical Advisory Board. He is the former Chief of Pediatric Pulmonary and Allergy Divisions, and allergy-immunology and pulmonary fellowship director. Dr. Moss also served as Director of the Cystic Fibrosis Center at Stanford, and site principal investigator for Cystic Fibrosis Therapeutics Development Network, where he was also the inaugural Chair of the Protocol Review Committee from 1991 to 2009.

Dr. Jane C. Davies, MD, FRCPCH, is Professor of Paediatric Respirology and Experimental Medicine at Imperial College London and an Honorary Consultant in Paediatric Respiratory Medicine at the Royal Brompton & Harefield NHS Foundation Trust. She is the site co-PI for the European CF Society Clinical Trials Network and leads their Lung Clearance Index Core facility. She has experience in clinical trial design for both investigator-initiated and pharma-sponsored studies.

Dr. Michael R. Knowles, MD, is Professor of Pulmonary and Critical Care Medicine at University of North Carolina (UNC), Chapel Hill. He is also the head of two multicenter studies, the first, Genetic modifiers of disease phenotype (severity) in cystic fibrosis lung and liver disease, which also includes a recently formed international consortium that is conducting a whole genome scan. The second is a consortium of eight North American sites studying rare genetic disorders of mucociliary clearance. Additionally, Dr. Knowles was the site PI at UNC Chapel Hill, and served on the Steering Committee, when the Cystic Fibrosis Therapeutic Development Network was founded.

Dr. Felix A. Ratjen, MD, PhD, is the Chief of Paediatric Respiratory Medicine at The Hospital for Sick Children, Professor of Paediatrics at The University of Toronto, and Senior Scientist at the Research Institute in the Department of Physiology and Experimental Medicine. He co-leads the Cystic Fibrosis Centre at The Hospital for Sick Children, and is the Medical Director of the Clinical Research Unit there.

Dr. Isabelle Sermet-Gaudelus, MD, PhD, is Professor of Pediatric Medicine at l'Hôpital Necker-Enfants Malades in Paris, France. She has developed and conducted several therapeutic trials for cystic fibrosis for both academic and company-initiated investigations. Her research focuses on modulation of the clinical severity of cystic fibrosis depending on fluid transfer and its therapeutic applications.

Dr. Pamela L. Zeitlin, MD, PhD, is Professor and Director of Pediatric Pulmonary Medicine and the Co-Director of the Cystic Fibrosis Center at Johns Hopkins University. Her research focuses on the role of chloride channels in inherited diseases of the respiratory tract, especially cystic fibrosis.

“As a pediatric pulmonologist, my research and clinical practice have been dedicated to helping children exercise one of their most basic rights, to breathe. The majority of my work has focused on finding the most up-to-date and effective treatments for children with cystic fibrosis, as they are still one of the few patient populations who are denied this right from birth, and have a life expectancy of 40. When Proteostasis asked me to lead their clinical advisory board, I was not only eager to learn all I could about their proprietary screening and drug development platform, but excited at the prospect of working with other clinicians who will bring their knowledge and experience of cystic fibrosis to an executive team making great strides in our field,” commented Dr. Moss, inaugural chairman of the clinical advisory board.

“The appointment of Proteostasis' clinical advisory board confirms our corporate commitment to developing innovative therapeutics for orphan diseases such as cystic fibrosis,” states Dr. Markus Haeberlein, PhD, Senior Vice President and Chief Scientific Officer at Proteostasis. “Our platform technology focuses on developing small molecules for protein folding trafficking and clearance diseases with few or no treatment options. Our proprietary screening and drug discovery process is broadly applicable to a number of diseases, and under the guidance of this advisory board, we are in an even better position to realize its true potential, starting with the cystic fibrosis patient population.”

About Proteostasis Therapeutics

Proteostasis is a drug discovery company addressing diseases caused by defects in protein folding, trafficking and clearance for orphan and neurodegenerative diseases. The Company’s Disease Relevant Translation (DRT™) platform combines a proprietary screening approach with state-of-the-art medicinal chemistry, to generate highly selective drug candidates and is advancing a pipeline inclusive of a lead program in cystic fibrosis and a partnership with Biogen Idec for neurodegenerative diseases.

The Company’s DRT™ platform utilizes functionally disease relevant cellular models with high translatability to develop therapies based on the modulation of protein homeostasis pathways within the cell. These pathways are part of cellular ‘quality control’ machinery, known as the protein homeostasis network or Proteostasis Network (PN). By modifying the function and capacity of the PN, the Company’s therapeutic product candidates correct for imbalances in the PN resulting from the

cumulative effects of disease, genetic mutations, environmental factors and aging. For more information visit www.proteostasis.com.

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