



## **OrPro Therapeutics Receives NIH Grant Award to Advance Development of Recombinant Thioredoxin for Treatment of Cystic Fibrosis**

July 31, 2012, SAN DIEGO -- OrPro Therapeutics Inc. today announced that the company has been awarded a National Heart, Lung and Blood Institute Small Business Innovation Research (SBIR) grant from the National Institutes of Health (NIH). The grant enables OrPro to advance development of its lead product, ORP-100, for the treatment of cystic fibrosis (CF), an inherited genetic disease that affects approximately 80,000 people worldwide.

“This award focuses on an inhaled formulation of ORP-100 for topical delivery to the lung surface, a key milestone in our preclinical development program,” said Peter B. Heifetz, Ph.D., OrPro president and chief technology officer. “NIH grants are highly competitive, and our proposal received excellent reviews and scores acknowledging OrPro’s strong team and innovative approach to CF therapy.”

ORP-100 is a recombinant engineered variant of thioredoxin, a human lung protein that has demonstrated in laboratory studies a potent ability to increase the fluidity of mucus. Clearance of the thickened mucus that is characteristic of CF remains a central and poorly met treatment objective. ORP-100 will be administered through an advanced aerosol delivery system, and in contrast to DNA-degrading mucolytics, targets the adhesive protein gel network that is common to all patients with obstructive mucus.

“We are excited about the potential for ORP-100 to be a next-generation mucus-thinning (mucolytic) drug that will offer a convenient, safe and effective treatment alternative to the people who suffer from CF” said Jeff Raser, chief operating officer. “New therapeutic strategies to correct underlying CF defects have promise, but there still remains a critical need for superior mucolytic therapies in CF and other obstructive pulmonary diseases such as bronchiectasis.”

The mucolytic properties of thioredoxin were discovered at leading respiratory hospital National Jewish Health (NJH) in Denver, CO by pediatric pulmonologist Dr. Carl White, M.D. In 2011, OrPro licensed from NJH an intellectual property portfolio claiming compositions and uses of the thioredoxin active site for the treatment of pulmonary disease. Dr. White (now at University of Colorado Denver/Children’s Hospital Colorado), and his NJH colleague Dr. David Nichols are medical advisors to the company, and will collaborate on the NIH-funded project.

“We have assembled a world-class team of experts in research, preclinical/clinical development, biologics manufacturing, and finance to help guide OrPro in the development of this exciting new product candidate,” said Heifetz, who is also principal investigator on the SBIR grant. “We believe the support from NIH will act as a catalyst for the company and we look forward to completing an initial round of financing to accelerate our development efforts.”

### **About OrPro**

OrPro Therapeutics, Inc. is a pre-clinical stage biopharmaceutical company headquartered in San Diego, CA. The company’s goal is to develop a breakthrough class of safe, well-tolerated and more effective inhaled non-systemic drugs based on the thioredoxin active site for the treatment of patients with cystic fibrosis (CF), COPD/emphysema, bronchiectasis, severe asthma, and other serious obstructive pulmonary diseases. These diseases are characterized by thickened mucus resulting in impaired lung function. Poor clearance of abnormal, sticky mucus is associated with chronic infection and premature death, especially in CF. Despite advances in antibiotic therapy and other treatments there remains a large unmet medical need for improved mucus-reducing (mucolytic) drugs which in many cases are the most efficacious means of mitigating disease symptoms. Effective new treatments for CF are forecast to achieve peak annual sales in excess of \$1 billion annually. OrPro’s lead product, ORP-100, an aerosolized variant of recombinant human thioredoxin, will target abnormal viscosity due to excess protein gels in CF and has the potential for broader clinical efficacy than existing mucolytic approaches.

### **About National Jewish Health**

National Jewish Health is known worldwide for treatment of patients with respiratory, cardiac, immune and related disorders, and for groundbreaking medical research. Founded in 1899 as a nonprofit hospital, National Jewish Health remains the only facility in the world dedicated exclusively to these disorders. Since 1998, *U.S. News & World Report* has ranked National Jewish the #1 respiratory hospital in the nation.

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