U. of Rochester Initiates Phase 2 Clinical Trial of Insmed's iPlex for the Treatment of Myotonic Muscular Dystrophy; Trial Funded by NIH and MDA Grants

RICHMOND, Va. & ROCHESTER, N.Y. & TUCSON, Ariz., Jan 04, 2006 (BUSINESS WIRE) -- -- Insmed Incorporated (NASDAQ: INSM), a Richmond, Va., biotechnology company, the University of Rochester School of Medicine, and the Muscular Dystrophy Association (MDA) announced today the initiation of a Phase 2 clinical study investigating the use of iPlex(TM) (mecasermin rinfabate (rDNA origin) injection), a once-daily IGF-1 therapy, for the treatment of myotonic muscular dystrophy (MMD), the most common form of adult muscular dystrophy.

iPlex is a proprietary drug product for the delivery of recombinant insulin-like growth factor 1 (IGF-1). It is administered as a preformed complex with a recombinant form of its natural binding protein, insulin-like growth factor binding protein 3 (rhIGFBP-3). The novel compound is administered as a once-daily subcutaneous injection, which can restore and maintain IGF-1 levels to physiologically relevant levels. (The original name of the Insmed compound was SomatoKine.)

It has been known for decades that MMD patients do not respond normally to insulin. Recent research has identified an abnormality in an insulin receptor protein as the underlying cause and IGF-1 as a potential remedy.

Myotonic dystrophy affects an estimated 40,000 individuals in the United States and causes progressive muscle wasting and weakness in the hands, forearms, legs, neck and face. It often involves many other systemic effects, including endocrine abnormalities, especially with respect to insulin, a regulator of blood sugar (glucose); neurological changes, including excessive sleepiness and apathy; cataracts, usually requiring surgical excision; gastrointestinal problems; and cardiac rhythm abnormalities, often requiring pacemaker insertion.

The disease can lead to severe disability, and death can result from respiratory muscle weakness or fatal cardiac dysrhythmias.

At present, there is no treatment to reverse the muscle weakness or wasting or the defective insulin utilization in MMD.

"For decades we have studied various potential therapies for patients afflicted with myotonic dystrophy," stated Richard T. Moxley, III, M.D., Professor of Neurology and Pediatrics at the University of Rochester and the Principal Investigator in the Phase II trial. "This study is based on preliminary clinical data demonstrating IGF-1's ability to restore or preserve muscle strength as well as improve glucose control. We are optimistic that iPlex given once daily will be effective and well tolerated in these patients."

The Phase 2 study of iPlex to investigate the safety and tolerability of once-daily subcutaneous injections of iPlex in patients with MMD will involve two sequential studies each involving 15 patients. The first study is a 24-week, dose-escalation study of iPlex to identify an optimal dose for the subsequent 24-week, fixed-dose study. Both studies will evaluate a number of safety parameters in a prospective manner, as well as several key efficacy measures such as muscle mass and strength.

Kenneth M. Attie, M.D., Chief Medical Officer of Insmed, added, "Myotonic dystrophy is an example of a serious disease, characterized by muscle wasting and insulin resistance, for which iPlex may be an ideal therapeutic intervention. We are very pleased that NIH has endorsed Dr. Moxley's protocol and that NIH and MDA are supporting this important clinical trial. Insmed is committed to working with the University of Rochester to

advance the study of iPlex for this devastating disease."

The University of Rochester, designated by the National Institutes of Health (NIH) as one of several "centers of excellence" for muscular dystrophy research, is receiving up to $1 million per year for five years in federal NIH funding and up to $500,000 per year for three years from MDA, for a total of up to $6.5 million, to identify potential muscular dystrophy therapies.

Robert Ross, President and CEO of MDA, added, "With the compassionate support of the American people, MDA, together with the NIH, is very proud to support the research being conducted by Dr. Moxley as he continues the lifesaving mission of finding a treatment for those with myotonic dystrophy."

About Myotonic Muscular Dystrophy

Myotonic muscular dystrophy (also known as myotonic dystrophy, dystrophia myotonica or Steinert's disease, and abbreviated MMD, MyD, or DM) is the most common type of adult muscular dystrophy, which affects 1 in 8000 individuals (approximately 40,000 people in the United States). Two genetic abnormalities have been identified that are responsible for myotonic dystrophy types 1 and 2 (DM-1, DM-2). Myotonic dystrophy patients develop progressive muscle wasting and weakness in the hands, forearms, legs, neck and face, as well as cataracts and cardiac arrhythmias, and eventually can become totally disabled, dying usually from respiratory or cardiac failure. At present, there is no treatment to reverse most of these symptoms. Previous preclinical and human studies have demonstrated that IGF-I therapy may be an effective treatment for myotonic muscular dystrophy(1,2). For more information about MMD, please visit www.mdausa.org.

About The Muscular Dystrophy Association

MDA is a voluntary health agency -- a dedicated partnership between scientists and concerned citizens aimed at conquering neuromuscular diseases that affect more than a million Americans. MDA combats neuromuscular diseases through programs of worldwide research, comprehensive medical and community services, and far-reaching professional and public health education. MDA is the world's largest non-governmental sponsor of research seeking the causes of and effective treatments for neuromuscular diseases, sponsoring some 400 research projects annually.

About Insmed Incorporated

Insmed is a biopharmaceutical company focused on the discovery and development of drug candidates for the treatment of metabolic diseases and endocrine disorders with unmet medical needs. For more information, please visit www.insmed.com.

1. Furlin D, Marette A, Puymirat J. Insulin-Like Growth Factor I Circumvents Defective Insulin Action in Human Myotonic Dystrophy Skeletal Muscle Cells. Endocrinology. 1999; 140:4244-4250.

2. Vlachopapadopoulou E, Zachwieja JJ, Gertner JM, Manzione D, Bier DM, Matthew DE, Slonim AE. Metabolic and Clinical Response to Recombinant Human Insulin-Like Growth Factor I in Myotonic Dystrophy-A Clinical Research Center Study. J Clin Endocrinol Metab. 1995; 80:3715-3723.

Statements included within this press release, which are not historical in nature, may constitute forward-looking statements for purposes of the safe harbor provided by the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements regarding expected financial position, results of operations, cash flows, dividends, financing plans, business strategies, operating efficiencies or synergies, budgets, capital and other expenditures, competitive positions, growth opportunities for existing or proposed products or services, plans and objectives of management, demand for new pharmaceutical products, market trends in the pharmaceutical business, inflation and various economic and business trends. Such forward-looking statements are subject to numerous risks and uncertainties, including risks that product candidates may fail in the clinic or may not be successfully marketed, the company may lack financial resources to complete development of product candidates, competing products may be more successful, demand for new pharmaceutical products may decrease, the biopharmaceutical industry may experience negative market trends and other risks detailed from time to time in the company's filings with the Securities and Exchange Commission. As a result of these and other risks and uncertainties, actual results may differ materially from those described in this press release.