



Contact: Catherine Valenti
Vice-President for Programs
Alpha-1 Association
208-895-8630
valenti@alpha1.org

CMS Reimbursement for Orphan Drug Favorable for Alpha-1 Patients
Concerns Remain for Others Affected by Rare Disorders

Washington, DC, November 9, 2002 – The Alpha-1 Association (A1A), a membership organization for people affected by Alpha-1 Antitrypsin Deficiency (Alpha-1), was pleased with the November 1, 2002 decision of the Centers for Medicare and Medicaid (CMS) to remove three orphan drugs from the Hospital Outpatient Prospective Payment System (HOPPS), and reimburse them on a reasonable cost basis. One of those drugs is Prolastin (alpha-1 proteinase inhibitor), the only treatment available specifically for Alpha-1. This decision, which takes effect January 1, 2003, assures access to care for hundreds of Medicare patients with Alpha-1 who currently rely on this life-sustaining drug.

However, the relief the Alpha-1 community feels over this ruling is tempered by the concern for the effect this ruling will have on thousands of patients diagnosed with rare disorders whose lives often depend on access to certain orphan drugs.

"The Alpha-1 community is very fortunate that Prolastin was one of the biologics exempted from the CMS rule," said Abbey Meyers, President of the National Organization for Rare Disorders (NORD). "However, people who need the approximately 100 other biologics will not have access to their life saving drugs. It is therefore important that we work together to solve this problem."

The A1A has been working with NORD and other organizations on the HOPPS reimbursement, and has renewed its commitment to continue to support efforts to ensure access to care for thousands of individuals affected under the November 1 ruling.

"We were not alone in our efforts to receive fair reimbursement for Prolastin," said Dennis J. Barbour, President and CEO of the A1A. "NORD assumed a leadership position on this issue and worked with us every step of the way. Our drug's removal from HOPPS does not lessen our concern for the entire family of patients suffering from rare disorders. We will continue to work together with NORD and other organizations to address reimbursement concerns for orphan drugs."

Background

Alpha-1 Antitrypsin Deficiency (Alpha-1) is a genetic, inherited disorder that often results in progressive, incurable disease usually in the form of emphysema in adults and cirrhosis in adults and children. Diseases associated with Alpha-1 occur due to the Alpha-1 protein becoming stuck in the liver instead of secreting out to the bloodstream. As it accumulates, it can cause cirrhosis and other liver disease. Since one of the primary functions of the Alpha-1 protein is to travel to the lungs and protect delicate tissue there, the absence of this protein can result in severe lung damage, often in the form of emphysema. There are an estimated 100,000 individuals with a severe deficiency of Alpha-1 who are at high risk of presenting with lung and liver disease, and 20 million more individuals who are affected as carriers of this disorder.

About The Alpha-1 Association

The Alpha-1 Association (A1A) is a patient-driven, non-profit membership organization dedicated to identify those affected by Alpha-1 Antitrypsin Deficiency (Alpha-1) and to improve the quality of their lives through support, education, advocacy, and research. The Association offers comprehensive assistance to its members through support groups, a peer mentoring program, personal patient assistance, educational materials, strong public policy efforts, and a compassionate care program. Professional educational resources and information packets are offered to health care providers who care for patients affected by Alpha-1.

The A1A, located in Washington DC, is a member of various health related organizations including the National Organization for Rare Disorders, Genetic Alliance, National Health Council, COPD Coalition, National Emphysema/COPD Association (NECA), National Society of Genetic Counselors, and Research America.