Principles for Health Care Reform and the Rare Disease Community

The Plasma Users Coalition (PUC) is a group of national patient organizations that include the Alpha-1 antitripsin, primary immunodeficiency diseases, hemophilia, Guillain- Barré, CIDP, platelet disorders and other **rare disease** communities, all of which represent patients whose lives depend on plasma derived therapies. The PUC believes our health care system must provide affordable, quality coverage for all Americans, and as the health care reform debate intensifies, we have concerns regarding continued access to the specialized, expensive care and therapies for individuals with **rare diseases**.

The patients we represent have **rare disorders** and need life-long access to expensive, life-saving treatments. For example, the average annual cost of treating an adult male with hemophilia is $300,000 per year; the cost of treatment for an individual with alpha-1 antitrypsin deficiency is approximately $150,000 per year; and persons with Primary Immune Deficiency diseases (PIDD) who infuse or inject their therapy every four weeks costs on average $35,000 to $50,000 per year to treat. Individuals managing acute neurological disorders such as Guillan Barré Syndrome or Chronic Inflammatory Demyelinating Polyneuropathy, who also infuse a plasma therapy on a regular basis, cost approximately $30,000 to $60,000 per year.

We applaud the recent efforts of Congress to develop a health care reform proposal. Our health care system must meet the diverse needs of all Americans, including the day-to-day needs of relatively healthy Americans, who essentially require preventative care and acute treatment for illness and injury. Likewise, the health care system must address the highly individualized needs of people with **rare diseases**. These Individuals present unique challenges within the health care system, as many **rare diseases** require expensive, complex and specialized treatments.

Rare disease patients face chronic, life threatening diseases and some of this country's highest treatment costs forcing many into personal bankruptcy and poverty! With thoughtful and reasonable healthcare reform, Congress can ensure this will never happen, ever again. The following principles highlight our unique needs and concerns:

PRINCIPLES

Private Insurance Market Reforms

The Coalition is pleased that the proposals outlined to date include guaranteed insurance coverage with guaranteed renewability, choice of health plans and no pre-existing condition clauses. We are also pleased that lifetime and annual limits of coverage are eliminated. While we applaud the annual caps on out-of-pocket costs, we are concerned that they are not adequate for the **rare disease** community. Annual out-of-pocket costs and co-pays for individuals with **rare diseases** are still too high and continue to thwart access to medical care. It is crucial that out-of-pocket limits are set at dollar amounts and not as percentages. High out-of-pocket costs and co-pays *disproportionately* impact individuals with **rare diseases** whose therapies can be extraordinarily expensive and life-long.

Affordability

Affordability can be defined as the percentage of income a household can devote to health care while still having sufficient income to address other necessities of life. Securing reasonably priced insurance and covering the uninsured is an important component of health care reform. In addition, health care reform legislation must also limit deductibles and co-payments, which can deter patients from getting the care they need and limit patient access. Co-payment percentages benchmarked at 10% or 20% that

may seem appropriate for traditional pharmaceuticals are not at all reasonable for **rare disease** patients. With regard to individual insurance subsidy levels, users of plasma protein therapies need subsidy levels at or above 400% of the federal poverty level (FPL). Patients with **rare diseases** treated with plasma protein therapies are able to lead productive lives and attend school or work but they must be able to afford the therapy.

Access to Specialists

Streamlined access to specialists is critical for people with **rare diseases**. These individuals require services and care from members of the medical profession who have specialized knowledge of the diagnosis, treatment and management of their disorders. The current proposals do not explicitly address how individuals with **rare diseases** will have unobstructed access to specialists.

Access to Therapies

Individuals with **rare diseases** must have access to the full range of medically necessary treatments appropriate for their condition. Decisions regarding which treatments are most suitable must be reserved for the physician in consultation with the individual patient. Without appropriate treatment, individuals face detrimental health outcomes. Furthermore, payers risk facing unnecessary costs from potential complications that arise from any limitations placed on the full range of therapies.

Comparative Effectiveness Research

The Coalition supports comparative effectiveness research that identifies treatments and services that are effective in *clinically* treating **rare diseases.** We are concerned, however, that under certain circumstances patients with **rare diseases** could be disadvantaged should comparative "cost" effectiveness studies occur rather than comparative "clinical" studies. To address this concern, we propose creation of a single point of contact, a "Rare Disease Ombudsman", to serve as a liaison for patients with **rare diseases**. We also support implementation of an expert **rare disease advisory panel** to evaluate comparative effectiveness research studies on "orphan" therapies or services for **rare diseases.** When treatment for a disease is chosen for evaluation, the research team should include expert clinicians, consumer representatives impacted by the disorder, and physicians or researchers from the relevant agency.

General Recognition of Rare Diseases

We call upon all policymakers engaged in the current health care reform process to ensure that the system fully addresses the needs of all Americans including the specialized needs of individuals with **rare diseases**. Payers and providers of health services must recognize the unique and on-going needs of the **rare disease** community to have access to specialists and highly specialized therapies. For individuals with these conditions, "one size does not fit all."

Alpha-1 Association
Alpha-1 Foundation
GBS/CIDP Foundation International
Committee of Ten Thousand
Hemophilia Federation of America
Immune Deficiency Foundation
National Hemophilia Foundation
Platelet Disorder Support Association
Patient Services Incorporated