A-PLUS IOM Survey Answers RE: Essential Benefits Package

Submitted 12-6-2010

1. What is your interpretation of the word “essential” in the context of an essential benefit package?

The American Plasma Users Coalition (A-PLUS) is a coalition of national patient advocacy organizations created to address the unique needs of over 125,000 patients with rare diseases that use life-saving plasma protein therapies. The disorders that the coalition represents include Alpha-1Antitrypsin Deficiency (Alpha-1), Guillain-Barré syndrome and Chronic Inflammatory Demyelinating Polyneuropathy; Hemophilia, Primary Immunodeficiency Diseases (PIDD) and Platelet Disorders. With continued access to needed treatments & therapies and specialized medical professionals, our patients lead productive lives.

A-PLUS interprets the word “essential”, in the context of the essential benefits package for new health insurance plans, as any medical treatment, procedure, service, as well as equipment and supplies indicated and approved by the United States Food and Drug Administration (FDA), for the care of patients with that specific diagnosis or follows generally accepted medical standards. Essential benefit packages should be designed to encourage patient access and not impede patients in any way from obtaining needed treatments and specialized care. Although “generally accepted medical standards” is a common term, PLANS LOOK TO MEDICAL LITERATURE, TREATMENT GUIDELINES RECOMMENDED BY MEDICAL AND PATIENT ORGANIZATIONS AND OTHER SOURCES TO DETERMINE STANDARDS OF CARE. However, new health plans should provide coverage for the full spectrum of treatments and medical services prescribed by the physician either episodically or by prophylaxis. Thus, the essential benefits definition should revoke several care-limiting tools which have been implemented in various states, such as step therapy (failing off of one product to obtain another); prior authorization; single treatment formularies; and dosage rationing by the plan. These would all limit the access of the patients in our community to the full spectrum of care needed.

Since these patients have rare and highly specialized disorders, the coalition recommends that the essential benefit packages allow patients’ access to needed specialists in their disorders. This allows the physician to formulate the best treatment regime for our patients and the appropriate site of care (whether in the hospital, outpatient clinic, office of the physician or the home setting). Site of service is critical with respect to infusion therapy for plasma based products in this patient population, particularly for patients at end stage of their disease.

Medical evidence continues to highlight that access to those needed treatment therapies and services lowers the overall long-term cost of care and increases positive health outcomes.

The fear in the rare and chronic disease community is that an essential benefits package will concentrate on a benefits package that focuses on relatively normal everyday health conditions and not pay enough attention to patients with rare and chronic diseases that require long term or even lifetime treatment. Under the current health care system, patients with rare and chronic diseases are the proverbial “square pegs” being forced into “round holes”. We A-PLUS members expect that the essential benefits package will provide a simple and expeditious pathway for patients with rare and chronic diseases to receive medical care for as long as it is needed, with the best treatment modality in the site of care most appropriate.

2. How is medical necessity defined and then applied by insurers in coverage determinations? What are the advantages/disadvantages of current definitions and approaches?
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The generally accepted definition of medical necessity is services or supplies that are needed for the diagnosis or treatment of a medical condition and meet accepted standards of medical practice.

A-PLUS recommends that the definition of medical necessity also include access to specialty care physicians and treatment regimens determined by physicians. Our patient communities require services and care from members of the medical profession who have specialized knowledge of the diagnosis, treatment and management of their respective disorder.

The A-PLUS Coalition recommends that any definition of medical necessity be expansive enough to understand that patients respond differently to treatment and the need to access to multiple treatments is imperative. A-PLUS recommends that doctors and the patient-physician relationship determine what is necessary for medical treatment plans to determine insurance coverage.

The essential benefits package should always include a process to appeal claims denials. That process should provide assurance that the insurer has an obligation to first confer with the patient’s physician to discuss a possible denial and the grounds for rendering such a decision. If the insurer executes a denial, it must be in writing with a full and clearly understood reason for the denial.

Denial notices must also comply with the following:

- Any notice of an adverse benefit determination must include information sufficient to identify the claim involved, including the date of service, health care provider, claim amount (if applicable), diagnosis code and its corresponding meaning, and the treatment code and its corresponding meaning.
- The notice of adverse benefit determination not only must include the reason for the adverse determination, but must include the denial code and its corresponding meaning, as well as a description of the plan’s standard, if any, used in denying the claim. The notice of denial on a final appeal must include a discussion of the decision.
- The plan must provide a description of available internal appeals and external review processes, including information on how to initiate an appeal.
- The plan must provide information on how to contact any applicable consumer assistance established under the Public Service Health Act to assist individuals with the claims process.

Under no circumstances should such an appeal process be used to diminish coverage by the essential benefits package. The essential benefits package should make it clear that the current practice by some insurance companies of “Coverage by Appeal” will no longer be tolerated.

3. What criteria and methods, besides medical necessity, are currently used by insurers to determine which benefits will be covered? What are the advantages/disadvantages of these current criteria and methods?
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Currently, insurance companies use a variety of methods besides medical necessity to determine benefit coverage and claims payment, including the use of medical guidelines, lack of recognition of the disease, strenuous requalification requirements, limited formularies or exorbitant co-pays.

While insurer’s guidelines can be quite specific, they are not uniform, sometimes do not cover necessary therapies (especially for patients with rare and chronic conditions), can be changed with little or no notice, and sometimes include specific provisions which hamper proper chronic disease management. For instance, several insurers’ guidelines do not cover all of the available immunoglobulin (IgG) therapies in all settings for those with primary immunodeficiency diseases (PIDD), forcing patients to switch immunoglobulin therapies. One immunoglobulin product is not the same as the next IgG product. One size does not fit all.

For some individuals with rare and chronic diseases, the insurer simply does not recognize the diseases even though the medical data is quite clear and quite accepted. For example, insurers are reluctant to recognize females with hemophilia, despite evidence that women may be more than symptomatic carriers. Another common practice is only providing “coverage by appeal” for individual with rare diseases by denying claims and then requiring additional substantiation of diagnosis, adding additional costs and stress to patients with the rare diseases. The hope of insurers may be that most, if not all, will not pursue an appeal and thus not be required to pay claims.

Some insurers have required a periodic substantiation of the diagnosis and refused to pay for key therapies even when the diagnosis is confirmed. For example, individuals receiving immunoglobulin will be periodically monitored to determine whether they should continue to receive immunoglobulin therapy, despite having a definitive diagnosis of a chronic antibody deficiency. Given this requirement, patients may have to forego immunoglobulin therapy treatment until their antibody levels decrease significantly, forcing the patient to become ill.

Insurers may also have very limited formularies, forcing patients with chronic conditions to make a difficult decision between obtaining the more optimal treatment (and paying for it out of pocket) or switching to a suboptimal therapy or prescription regimen, which is covered by the limited formulary. While requiring patients to consider generic alternatives is appropriate in many cases, forcing patients to forego optimal treatment options for chronic conditions is a very short-sighted approach to patient care management. And can ultimately result in disease exacerbations that require expensive hospitalizations which could have been avoided.

Alternatively, the insurer may require very high copayments, especially for “specialty” prescription medications, such as immunoglobulin therapy or clotting factor. As highlighted in a recent article in the *Annals of Internal Medicine*, higher copays affect the ability of patients to obtain key prescriptions. The US authors reported medication abandonment increased with the more money provided in co-payments, revealing co-payments of $40-50 and those greater than $50 were 3.40 and 4.68 times more likely to be abandoned than prescriptions without a co-payment. When patients do not pick up prescriptions, they may jeopardize their health. In addition, their medical records may be inaccurate because the information in those records will lead providers to believe that medicines are not working, when in fact they were never taken. Especially for patients with chronic conditions, these additional copayments create strong disincentives for maintaining key care.

Some insurers have opted to include immunoglobulin and clotting factor as a pharmacy benefit (which is not well reimbursed and includes higher co-pays), rather than a medical treatment. In doing so, the costs are substantially shifted to the patient. Generally, in response to such high copayments, individuals with primary immunodeficiency diseases go without or stretch their treatment to the point of ineffectiveness. If chemotherapy was treated as a pharmacy benefit rather than a medical treatment, it is probable that most people with cancer could not afford to receive treatment and thus would die. The same is true for patients whose use of plasma based biologics is their
medical treatment. Life threatening sickness is the prospect should such patients not be able to afford the treatment that leads to health and not further sickness.

Therefore, it would be most helpful if the Secretary adopted national clinical guidelines for rare and chronic diseases that are developed by specialty medical organizations or others that deal most closely with the diseases and patients with rare and chronic conditions and not rely upon those developed by insurers. By doing so, those guidelines would recognize actual rare, chronic conditions, not require periodic substantiation, be uniform, relatively stable, and not hamper proper chronic disease management. In addition, the guidelines should take into account the best specialty clinician to provide such care and not rely on gatekeepers with little or no knowledge of rare and chronic diseases for those with complex chronic conditions.

4. What principles, criteria, and process(es) might the Secretary of HHS use to determine whether the details of each benefit package offered will meet the requirements specified in the Affordable Care Act?

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Besides adopting national guidelines for care (as discussed under question 3), the Secretary should examine multiple care measures, including those related to access (e.g., access to specialists, wait times for seeing various practitioners, ability to be seen as a new patient, etc.), quality of care (e.g., appropriate chronic disease management, various service sites for care), and patient-centered care (e.g., meeting the particular patient needs). The Secretary should employ an open, transparent process, possibly utilizing an advisory committee, to advise the Secretary on these issues.

5. What type of limits on specific or total benefits, if any, could be allowable in packages given statutory restrictions on lifetime and annual benefit limits? What principles and criteria could/should be applied to assess the advantages and disadvantages of proposed limits?

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As A-PLUS represents the interest of over 125,000 patients that utilize expensive plasma protein treatments, our coalition was involved heavily in advocating for the removal of lifetime benefit limits. These patients need life-long access to life-saving and life sustaining treatments which for some chronic conditions can be very expensive. For example, the average annual cost of treatment for an adult male with the bleeding disorder hemophilia is $300,000 per year but could come close to $1 million; the cost of treatment per year for an individual with Alpha-1 is approximately $150,000; and persons with Primary Immunodeficiency diseases (PIDD) who infuse or inject their therapy every four weeks cost on average $35,000 to $50,000. Individuals managing acute neurological disorders such as Guillain-Barré Syndrome and Chronic Inflammatory Demyelinating Polyneuropathy can use approximately $45,000 to $90,000 in treatment per year. These are very expensive disorders and can run into lifetime and annual benefit limits very rapidly.
For over a decade our advocacy organizations have sought a legislative remedy to the problem of lifetime limits that cause the patients we represent to lose their coverage when reaching their limit. When a lifetime limit is reached, the patient sees few options for additional coverage. Pre-existing condition exclusions usually prevent the patient from obtaining coverage in the individual commercial market. The patient could turn to a state high risk health insurance pool, if that state offers a high risk plan. However the premiums for the plan are usually prohibitively expensive. The passage of the Patient Protection and Affordable Care Act (PPACA-public law 111-148) has added a layer of protection for the patients we represent.

The A-PLUS Coalition opposes any additional limits on specific or total benefits in the packages given statutory restrictions on lifetime and annual benefit limits. The treatment needs of the patient populations we serve are so specialized the limits could place life threatening consequences on the patient. The A-PLUS coalition recommends against allowing providers to apply treatment caps of any kind. For example caps placed on dosage amount of treatment dispensed to patients or limits on the type of treatment the patient may receive. The coalition recommends that no limits should be placed on any treatment regimen, either by cost or in limits on treatment, approved by the United States Food and Drug Administration (FDA). There should be no constraints that could be used as a proxy or substitute for lifetime or annual limits prohibition established by the law.

6. How could an “appropriate balance” among the ten categories of essential care be determined so that benefit packages are not unduly weighted to certain categories? The ten categories are: ambulatory patient services; emergency services; hospitalization; maternity and newborn care; mental health and substance use disorders services, including behavioral health treatment; prescription drugs; rehabilitative and habilitative services and devices; laboratory services; preventive and wellness services and chronic disease management; pediatric services, including oral and vision care?

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By providing all of the key benefit areas, the Secretary will have provided an “appropriate balance” among the ten categories. However, each insured individual will likely use those categories to best suit his or her needs. For individuals with rare and chronic diseases, it will be most important that those individuals have access to specialists and all prescription medications. For instance, patients with primary immunodeficiency diseases rely upon an early diagnosis (which usually requires prompt access to a specialist) and appropriate lifelong therapies (usually immunoglobulin to replace missing or improperly functioning antibodies needed to fight infection) to maintain healthy, productive lives.

Moreover, utilization of specialized treatment facilities, such as the federally recognized hemophilia treatment centers (HTCs), does not neatly fit into particular categories of services. HTCs provide comprehensive, multi-disciplinary services in a single setting, and have been shown to improve quality and reduce morbidity and mortality of individuals living with this chronic disease. Weighting certain services, even in order to provide "balance", could disrupt this highly efficient model of care. Allowing access to comprehensive care centers ensures that the most appropriate balance of care is provided to the patient by medical professionals.

7. How could it be determined that essential benefits are “not subject to denial to individuals against their wishes” on the basis of age, expected length of life, present or predicted disability, degree of medical dependency or quality of life? Are there other factors that should be determined?
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Essential benefits must be based on medical need which can be determined through the use of quantitative and qualitative data analysis garnered from clinically relevant sources with a patient centric/end-user focus that illustrate improved health outcomes.

Factors that should be determined include rare diseases, and/or genetic or autoimmune chronic illness which must be considered separately from behavioral chronic illness. People affected by rare, chronic diseases have specific health needs which are best served by a comprehensive system; this care model includes specialists, home care services, delivery, and treatment locations. Quality adjusted life years must be a key determinant when measuring health outcomes.

8. How could it be determined that the essential health benefits take into account the health care needs of diverse segments of the population, including women, children, persons with disabilities, and other groups?

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Essential health benefits must take into account the health care needs of diverse segments of the population, including individuals with rare, chronic disease. This can be done by analyzing data generated by multiple data sources as part of defining a valid clinical outcome, as well as patient centric qualitative data sources specific to a genetic or autoimmune chronic illness population (i.e. advocacy group, government, non-profit/non-conflict entity). Quality adjusted life years must be a key determinant when measuring health care needs and outcomes.

9. By what criteria and method(s) should the Secretary evaluate state mandates for inclusion in a national essential benefit package? What are the cost and coverage implications of including current state mandates in requirements for a national essential benefit package?

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State mandates have been invaluable to rare, chronic and high cost disease groups, who might otherwise be excluded from private insurance coverage. Any decision to phase out a given state mandate, where the national
program provides similar protection, should be made cognizant of the possible disruption in coverage that might ensue for these particularly vulnerable groups.

That said, the Secretary should evaluate state mandates for inclusion in a national essential benefit package with criteria and method(s) based on cost data, as well as, outcome data such as quality adjusted life years.

A thorough cost benefit analysis, as well as a qualitative data assessment may help ease transition, and in situations where a benefit is eliminated a sunset period for transition may be devised.

10. What criteria and method(s) should HHS use in updating the essential package? How should these criteria be applied? How might these criteria and method(s) be tailored to assess whether: (1) enrollees are facing difficulty in accessing needed services for reasons of cost or coverage, (2) advances in medical evidence or scientific advancement are being covered, (3) changes in public priorities identified through public input and/or policy changes at the state or national level?

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The criteria and methods HHS should use must involve an annual meta analysis of data sources to accommodate changes in technology, clinical outcomes, and changes in quality adjusted life years.

Full medical assessments must take into account health maintenance in chronic illness. Medical assessments could be adjusted (up or down) to base coverage on personal circumstance, and/or application of a waiver in cases where enrollees are facing difficulty in accessing needed services for reasons of cost or coverage.

Advances in medical evidence should be reflected in the essential benefits packages as well as scientific advancement as determined through FDA approval process, and peer reviewed medical journals stating the improved/decreased health outcome. HHS must put in place expeditious pathways for the inclusion of new therapies and treatments in the essential benefits package when new products or information on outcomes become available.

An example for individuals with primary immunodeficiency diseases is the development of subcutaneous immunoglobulin. For years, immunoglobulin products were only developed to be administered intravenously. With the development and FDA approval of subcutaneous immunoglobulin products, this method of administration is now a valuable treatment option for patients with primary immunodeficiency diseases. HHS must make certain that improvements and new therapies can quickly be included in the essential benefits package.

Changes in public priorities identified through public input and/or policy changes at the state or national level must involve a patient centric approach and consumer advisory boards or consumer seats must be represented by end-
users in the discussion and decision making process