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

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HFA interprets the word "essential" in the context of the essential benefits package 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.

Prophylaxis is the scheduling of infusion therapy before a bleed (internal, joint, etc.) has occurred or it can be scheduled infusion therapy when a bleed has occurred. Prophylaxis is considered a standard of care and strong method of preventing poor health outcomes including long-term joint damage and disability in individuals with hemophilia. 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 insurance plans. These would all limit the access of patients to the full spectrum of care needed.

Hemophilia and other bleeding disorders are rare and highly specialized disorders. HFA recommends that the essential benefit package allows patients' access to needed specialists. This allows the physician to formulate the best treatment regime for patients and the appropriate site of care (whether in the hospital, outpatient clinic, office of the physician, hemophilia treatment center (HTCs) or the home setting). 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 hemophilia. Medical evidence continues to highlight that access to these 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". HFA expects 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. HFA recommends that the definition of medical necessity also include access to specialty care physicians and treatment regiments determined by physicians. Our patient population requires services and care from members of the medical profession who have specialized knowledge of the diagnosis, treatment and management of their respective disorder.

HFA recommends that any definition of medical necessity be expansive enough to understand that patients respond differently to treatment and access to multiple treatments is imperative. HFA recommends that doctors and the patient-physician relationship determine what is medically necessary.

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 insurance companies of "Coverage by Appeal" will no longer be tolerated.

HFA also encourages essential benefits to include standards. An example of such a standard is MASAC #188, the Medical and Science Advisory Committee at the National Hemophilia Foundation. This standard of service for pharmacy providers could be identified as a minimum expectation for any insurer contracting with a pharmacy to provide clotting factors concentrates for home use to patients with bleeding disorders. Minimum expectations include:

A. Pharmacy Provider Staff Knowledge of Clotting Factor Concentrates and Ancillary Supplies

- Pharmacy provider staff shall have sufficient knowledge and understanding of bleeding disorders to accurately follow the instructions of the prescribing physician and assure high quality service for the patient.
- Pharmacy provider staff shall be experienced with filling and handling prescriptions for the full range of clotting factor concentrates.
- Pharmacy provider staff shall be knowledgeable about necessary ancillary supplies.
- Pharmacy provider staff shall be knowledgeable about containers for the disposal of hazardous waste.
- Pharmacy provider staff shall direct patients to contact their established treating physicians for all medical and therapeutic questions.
- Pharmacy provider staff shall direct staff medical questions and concerns to the treating physician.

B. Clotting Factor Concentrates and Ancillaries

- Pharmacy providers shall be able to provide the full range of available concentrates, including all available assays and vial sizes.
- Pharmacy providers shall be able to provide all necessary ancillary supplies for administration of clotting factor concentrates. Examples of ancillary supplies include, but

are not limited to: needles; syringes; gauze; anesthetic creams; sterile field pads; sterile gloves.

- Pharmacy providers shall provide containers for the disposal of hazardous waste, and the collection of such containers shall be arranged pursuant to state and federal law.
- Some consumers of clotting factor concentrates require additional services, such as nursing services. If the pharmacy providers do not offer these services directly, they shall coordinate with the nursing agencies to ensure that all of the patient's needs are adequately met.

C. Processing of Prescription Orders

- Pharmacy provider staff shall work with prescribing physicians to ensure that prescription orders are filled within 48 hours.
- Prescriptions of clotting factor concentrates shall be dispensed as written by the prescribing physician. No changes or substitutions shall be made unless approved by the physician.
- If the prescription does not indicate a specific brand name of product, the pharmacist shall ask the prescribing physician which product should be dispensed.
- Filling of all prescription orders shall be within plus or minus 5-10 % of prescribed assays, barring extenuating circumstances. This standard shall not be violated by dispensing a number of vials so excessive that it would compromise compliance or so low a dose that it would compromise medical outcome.
- Clotting factor concentrates shall have acceptable outdates based on diagnosis and frequency of treatment. Short-dated product (outdate within 6 months) shall only be dispensed after consultation with the prescribing physician.
- Pharmacy provider staff shall supply any ancillary supplies required by the patients and prescribed by their physicians.

D. Hours of Operation / Access to Staff

- Pharmacy providers shall be open, at a minimum, Monday through Friday, excluding holidays, during regular business hours (9:00 am to 5:00 pm) in their service area time zones. If a pharmacy serves all 48 contiguous states, it will need to be open from 9:00 am until 8:00 pm Eastern Time, Monday through Friday, not including holidays.
- Pharmacy staff shall provide 24-hour emergency access including multilingual interpreters in case of emergency.
- If the pharmacy receives a call about an emergency situation, the treating physician shall be notified immediately. Pharmacy provider in consultation with the treating physician shall have plan in place to ensure that, in case of emergent need, patient shall have access to factor concentrate within 12 hours of expressed need, with a goal of 3 hours where logistically possible.

E. Delivery

- Routine orders from established patients shall be correctly filled and delivered within 48 hours from the time the order is placed.
- If the pharmacy receives a call about an emergency situation, the treating physician shall be notified immediately. Pharmacy provider in consultation with the treating physician

shall have plan in place to ensure that, in case of emergent need, patient shall have access to factor concentrate within 12 hours of expressed need, with a goal of 3 hours where logistically possible.
- Pharmacy providers shall have a plan in place to meet delivery requirements in the event of a natural disaster.
- Product shall be delivered to the location requested by the patient that has been determined by the pharmacy provider to be appropriate and safe.
- Shipping of all clotting factor concentrates shall meet all federally mandated standards, including those for temperature control.
- Pharmacy providers shall adhere to all HIPAA confidentiality guidelines.
- Pharmacy providers shall have an emergency contact number for customers to report problems with deliveries.

F. Recordkeeping, Billing and Product Recall

- Pharmacy providers shall have an accurate record-keeping system that meets state and federal requirements. In addition, pharmacy providers shall have treatment prescription information available for patients and prescribing physicians.
- Pharmacy providers shall explain patient copay, deductible and coinsurance payment responsibilities, and lifetime cap limits clearly at the time the first order is placed and annually when updating insurance information, or sooner if there has been a change in insurance.
- Pharmacy providers shall provide a statement of factor cost per unit dispensed to the consumer.
- Pharmacy providers must be able to trace the path any bottle of clotting factor concentrate has taken and the way it has been handled from the time it left the manufacturer until the time it is delivered to the consumer.
- Pharmacy providers shall participate in the National Patient Notification System for clotting factor concentrate recalls.

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.

Guidelines. While insurer's guidelines can be quite specific, they are not uniform. Guidelines sometimes do not cover necessary therapies (especially for patients with hemophilia or other rare bleeding disorders), can be changed with little or no notice, and can include specific provisions which hamper proper disease management.

The treatment of bleeding disorders and the biologic products used in treatment are not the same across the board. There is ongoing qualitative evidence that treatment/products used with one individual does not necessarily work with the body chemistry of another individual with the same bleeding disorder, even within families who share the disorder. Dosing schedules may also be different from patient to patient based on patient growth (in children) and bleeding patterns (in all). It is important that insurers' guidelines do cover <u>all</u> of the available clotting factors so that patients have access to the product that is most efficacious to that particular patient.

Lack of disease recognition, requalification requirements, and high copays. For some individuals with bleeding disorders, the insurer simply does not recognize the disease 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 of hemophilia and actually express lower than normal levels of clotting factor. 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.

Insurers may also have very limited formularies, forcing patients with rare bleeding disorders 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. Individuals with bleeding disorders rely on treatments (clotting factor) that are considered biologics. Biologic drugs are made using living cells and in some cases the biologic that an individual with hemophilia may be taking can be the only drug on the market (orphan drug) to treatment their form of hemophilia. Biologics tend to have a higher cost to patients and providers therefore gain more scrutiny when insurers look at ways to streamline prescription drug access.

Alternatively, the insurer may require very high copayments, especially for "special" prescription medications, such as clotting factor. In hemophilia treatment, these high copayment can add thousands of dollars of cost per month. For patients with chronic conditions, like hemophilia these additional copayments create strong disincentives for maintaining key care. Some insurers have opted to include 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. 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 with bleeding disorders whose use biologics as their medical treatment. Life threatening bleeding episodes and lifelong disability is the reality should such patients not be able to afford the treatment.

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. Entities such as the Medical & Scientific Advisory Council (MASAC) of the National Hemophilia Foundation and can be of value when identifying best practices for individuals with rare diseases including bleeding disorders.

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 above), 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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HFA represents the interest of patients with bleeding disorders. These patients need life-long access to expensive life-saving and life sustaining treatments. For example the average annual cost of treatment for an adult male with hemophilia is $300,000 per year but could come close to $1 million or more. Hemophilia is a very expensive disorder to treat and an individual with this condition can run through lifetime and annual benefit limits very rapidly.

For over a decade HFA has 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 have prevented the patient from obtaining coverage in the individual commercial market. The patient could turn to a state high risk health insurance pool, 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.

HFA 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. HFA recommends prohibiting the implementation treatment caps of any kind by providers. HFA recommends that no limits should be placed on any treatment regime, 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 bleeding disorders, it will be most important that those individuals have access to specialists and all prescription medications. For instance, patients with bleeding disorders rely upon an early diagnosis (which usually requires prompt access to a specialist) and appropriate lifelong therapies, usually clotting factor 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. 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 centered/end-user focus that illustrates improved health outcomes. Factors that should be determined include rare diseases, and/or genetic 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. As a measure of disease burden, quality adjusted life years considers the quality and the quantity of life lived, and offers a tool to help assess the value and cost of a medical intervention.

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 sources to illustrate valid clinical outcomes. These data sources should be qualitative patient centered and specific to the particular patient population. 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. A thorough cost benefit analysis and qualitative data assessment by HHS should be the basis of any decision to include or exclude benefits.

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. In situations where an individual is in a state where the decision is made to end a benefit that is not mandated by the federal essential benefits package, HHS should provided guidance on a transition period for patients. 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.

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.

As advances in medical evidence or scientific advancement are being uncovered and approved through the FDA, as well as a 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.

A past example for individuals with bleeding disorders is the emergence in the 1990's of recombinant clotting factors. Prior to the development and FDA approval of a means to produce human blood clotting factors using recombinant DNA technologies, all human blood clotting factors were produced from donated blood that was inadequately screened for HIV. Thus, HIV infection posed a significant danger to patients with hemophilia who received human blood clotting factors. Currently being evaluated is the emergence of longer lasting clotting factor products. Several clinical trials are currently in process for these products. These new products have the potential enhance the quality of life of individuals with bleeding disorders. HHS must make certain that improvements and new therapies like this 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, including patient representation on consumer advisory boards to represented the end-users in the discussion and decision making process.