

# **COTT** Washington Update --

VOLUME 13 NUMBER 2



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## > **KEEP COTT ALIVE!!**

From the beginning, COTT's guiding philosophy has been, Action=Life. The perspective is ongoing as COTT moves into its third decade as a grass-roots advocacy, support and policy organization serving the hemophilia and larger bleeding disorders communities. We are one of the few community advocacy organizations who do not accept support of any kind from the manufacturers of blood products, drugs or medical devices. This prohibition reflects our belief that conflict of interest played a key role in the AIDS/Blood epidemic that devastated the hemophilia community in the 1980s.

The survival of COTT is imperative if we are to continue to have an independent community voices participating in the regulatory structure as full stakeholders and partners. Your support is critical to our continued ground breaking work in blood safety and in the significant impact of trauma associated psychosocial challenges such as depression and Post Traumatic Stress Disorder, which our community members confront on a daily basis.

What can you do? Plenty. Send COTT a donation, by check in the mail or by clicking on "Donate" on the home page of our website, [www.cott1.org](http://www.cott1.org). All amounts are welcome. Then talk to friends, neighbors, relatives about blood safety and ask them to donate with you.

**KEEP THE INDEPENDENT COMMUNITY VOICE ALIVE AND ENGAGED**

## > **PUBLIC HEALTH AND THE HEMOPHILIA COMMUNITY: A ROADMAP FOR THE FUTURE**

This year COTT is joining forces with the Centers For Disease Control & Prevention's Division of Blood Disorders in planning the 2<sup>nd</sup> Conference on Blood Disorders In Public Health, to be held March 12-14, 2012 in Atlanta, Georgia. This important conference is a critical part of transitioning the entire bleeding disorders communities to a treatment model integrated with all the disorders in order to facilitate more centralized, integrated and equitable treatment and care to the entire community of bleeding disorders.

Serious improvements in the health of Americans have come through the Public Health System. From disease prevention, to surveillance, to protecting the health of our citizens, public health structures and initiatives are the root of the health of our nation. COTT has always understood this

point, even though the PHS and the government abandoned the hemophilia community during the 1970s and 1980s allowing both hepatitis C and HIV to adulterate our nation's blood supply. For the government, the blood community, manufacturers and those that collect and process blood, the hemophilia community became the unfortunate consequence of the AIDS/Blood epidemic. "Regrettable, unfortunate, yet unavoidable", became the mantra of the industry and the government.

For the end users, those facing the ravages of HIV/AIDS and hepatitis C, the contamination and action it triggered became a circus of the Good, the Bad, and the Ugly. The Good: one of the earliest being Dr. Judith Pool at Stanford, followed by Dr. Don Francis then at CDC, both of whom sounded the alarm regarding factor concentrates and the transmission of pathogens such as HCV and HIV; the Bad and the Ugly: the unconscionable lethargy exhibited by the federal regulatory structure and the entire blood community in the 1980s.

Yet throughout, COTT and the ten thousand persons with hemophilia and HIV and/or HCV remained committed to work with the federal regulatory structure and the blood community as well in rebuilding in the wake of the twin blood borne epidemics of HIV and HCV in the hemophilia community. We remained forward thinking and prioritized the safety of the blood supply in terms of all Americas.

Simultaneously in those years, COTT was an active participant in the HIV prevention cooperative exchange between the CDC, the States and local health departments. This was a successful and outcome driven process that directly involved all the communities of the HIV/AIDS epidemic working with local health departments, state HIV/AIDS programs and the CDC. A great deal was learned about what worked in addressing the exploding HIV/AIDS epidemic. Fifty-six members convened California's Community Planning Working Group in 1993; this huge group, only after months of competitive posturing, began to seriously confront the enormity of their task ... and the opportunities it could provide.

For the hemophilia community, which suffered serious isolation in the first decade of the AIDS nightmare, with some of the highest infection rate at that time, this was an opportunity to engage all the communities confronting AIDS and through our participation we learned a great deal that remains part of who we are today. This and other initiatives have consistently renewed COTT's belief in and commitment to the public health system in our nation.

An integrated delivery system for the larger bleeding disorders community, of which hemophilia is an important part, provides both opportunities and challenges. On the opportunity side, addressing inequities in the delivery of care in all of the bleeding disorder communities. In hemophilia up to one third of our community remains outside of the Hemophilia Treatment Center (HTC) System, according to CDC data. COTT's experience indicates that what is lacking is a new and updated map of our community. Challenges abound: Who is out there, and where are they accessing treatment and care? We have a much better picture of those who are accessing care at the nation's Hemophilia Treatment Centers. Yet we seriously lack any clarity about who is out

there not accessing care at an HTC. It would be important to understand who are those outside of the HTC system and how they are accessing care. We also need an updated needs assessment for those who are getting their care from an HTC.

There is also a geographic divide that impacts equity in care delivery as well as a divide rooted in race, gender and class in hemophilia. Integration will certainly provide for a clearer picture of what is out there and hopefully inform policies that address these important inequities in the delivery of treatment and care in the hemophilia and larger bleeding disorders communities.

COTT has always taken a more expansive view of our community's place in the larger community of bleeding disorders and chronic disease communities. We also see the critical importance of working closely with the federal government, the Hemophilia Treatment Centers, the Hemophilia Federation and the National Hemophilia Foundation to better understand our community and work together to ensure the equitable delivery of care to all persons with bleeding disorders.

## **> CONGRESS**

All last fall, polls of the nation's approval of Congress in general fell, bottoming out below 10%. Although all eyes since Christmas have been on the Iowa caucuses and the early primaries, attention in late January turns to the upcoming Second Session of the 112th Congress.

Little is expected, as the strong partisan divisions clearly will continue, and the most pressing business -- funding for government operations for a year starting next fall -- is in process. There will need to be one more hiking of the federal debt limit, which will no doubt cause fireworks, but by and large all eyes, already focused on campaigns, will be busy with nothing but until the Presidential and Congressional elections next November.

## **> STATE ISSUES**

While new forces in Congress last year sought the steepest possible cuts in federal agency operations in order to reduce the massive national debt, the fourth year of a serious US recession has devastated state government operations, partly from the federal cuts but mostly from lack of state revenue from missing payroll taxes from vast numbers of unemployed workers. At the same time, Medicaid and Unemployment Insurance rolls are up. States are slashing Medicaid benefits and tightening eligibility. On top of this squeeze, state agencies are losing personnel to layoffs and furloughs.

States must nevertheless now staff up to develop the agency structure to be ready for the implementation of the Health Reform Legislation of 2010, which takes full effect in 2014. In mid-December DHHS announced the design of the structure to assure that a full spectrum of care services, including chronic disease management, will be required of any insurer approved by the state for participating in the program. DHHS did this by listing four "benchmark" plans, of different types, among which a state can choose:

- > **the largest plan by enrollment in any of the three largest small group insurance products in the State's small group market**
- > **any of the largest three State employee health benefit plans nationwide, in terms of enrollment**
- > **any of the largest three national Federal Employee Health Benefits Plan options by enrollment**
- > **the largest insured commercial non-Medicaid Health Maintenance Organization (HMO) operating in the State.**

Key in this approach are two of its elements: letting a state exercise choice in the configuration of the plan it will operate, and drawing the model for the plan it selects from existing private insurance plans in its state.

This approach also fulfills the ACA legislation's requirement that health plans under it resemble many of the plans in use today. The agency performed multiple surveys and collected input for consumers and others before deciding on this configuration. For example, it found that state mandates, fought-for protections (including hemophilia care in some cases) put in place for a state's insurance licensing, are almost completely covered in the larger plans such as those above from which states will be choosing. All, it should be noted, are based on large-scale group plans; currently individual, non-group plans cover the fewest mandated services -- at the most expensive cost. Such enrollees will find large-scale financial relief when ACA is implemented in two years' time.

Unfortunately, many months remain before the Supreme Court finds the law can stand or not, during which time states should be at the height of funding and creating the structures that will be required; these must be in place by early 2013. In fact so many states are so far behind that they plan to use the federal option, letting the US Government come into their state and build and operate the insurance program there -- so many that the federal resources needed to carry out this massive unanticipated task far exceed funding plans for the program's implementation.

## > **AGENCIES**

### Blood Products Advisory Committee

COTT was pleased to learn that Corey Dubin, President and Board Chair of COTT, has been appointed again to the consumer representative seat on the FDA's Blood Products Advisory Committee (BPAC). Corey was originally appointed to a term on the committee in 1995, just after the Institute of Medicine's report HIV and the Blood Supply: An Analysis of Crisis Decisionmaking -- calling for increased consumer voice on committees such as this one. It was then-FDA Commissioner David Kessler who specifically instructed the committee's staff secretary to declare a consumer seat and place Mr. Dubin in it. His service there -- despite much early skepticism by the academic and industry-linked other members -- paved the way for all subsequent

consumer representation, on BPAC and also on the Advisory Committee on Blood Safety and Availability, in the DHHS Office of the Secretary (on which Corey Dubin has also served).

For the second year in a row, COTT has served on the FDA committee to review nominations for Consumer Representative seats on all FDA Advisory Committees.

### Informed Consent

In December COTT attended a meeting of the DHHS Advisory Committee on Blood Safety and Availability. The single topic for both days of the meeting was Informed Consent. Although the majority of presenters focused on transplants, whole blood transfusion cases were given as well. However, there was no focus on Informed Consent in chronic conditions, except for the Public Hearing presentation by COTT (included in this **Update** as Attachment #1) . COTT is also preparing a session for the 2nd National Conference on Blood Disorders in Public Health on Informed Consent. The Conference, sponsored by CDC, will be held in Atlanta March 12-14, 2012.

### Affordable Care Act Input

In October, COTT testified at a Consumer Advocates Input Meeting held by the federal Department of Health and Human Services (DHHS) concerning a section of the regulations being developed for operation of the Affordable Care Act, (ACA), also referred to as the Health Reform legislation. The section in question -- referred to as the "Essential Health Benefits" (EHB) section -- will define the set of minimum services which any participating insurance plan in any state must provide. (The draft guidance on this issue, discussed above under STATE ISSUES, has already been released.)

Witnesses at the October Consumer Input Session, in addition to COTT, NHF, and the A-PLUS Coalition in which COTT is active, also represented many large and small organizations in the health community. They unanimously objected to the emphasis on affordability over breadth of coverage, and to the small employer model, only a limited set of services and coverages were found at all.

"Utilization Management" methods are increasing in use by America's insurer-controlled care systems instead of clinically appropriate health care delivery models: Step Therapy, also known as Fail First; Prior Authorization requirements, Preferred Drug Lists, and the alleged 'cost-sharing' efforts to boost patient co-pay proportions above the 20% required by Medicare and many providers, up to an impossible 40 or 50%, an impossibility in hemophilia. Witnesses were in strong agreement on the need for protection of vulnerable disease communities from these "Utilization Management" tactics.

COTT's testimony is included in this **Update** as Attachment #2. COTT's participation in coalition actions on Essential Health Benefits is discussed further in the section on Coalitions, below.

### Blood Collection Release Errors

COTT staff attended a workshop in September sponsored by the FDA, the AABB (formerly American Association of Blood Banks), and America's Blood Centers. Growing out of needs increasingly recognized in recent years, this workshop focused on a disturbing area in whole blood collection and distribution: Quarantine Release Errors. This topic refers to blood units which, after being collected, are discovered to be unsuitable for release, either through testing, donor information, or any other reason. Due to the high volume of operations associated with blood collection, these units are not disposed of at once, but placed separately from those approved for release and use, in Quarantine. Blood can also be put there when the required testing on a unit has not yet been completed, or for other reasons.

The purpose of the workshop was to analyze data and patterns showing the release of such unacceptable units for use. This is a rare occurrence: in a recent two-year period, out of almost 30 million units collected, there were 20 quarantined units known to have been inadvertently released. These releases were shown to be somewhat due to computer error in these large operations, but much more typically due to human error ... including incorrect programming of the computer software in use. In terms of the steps involved in the process of blood collection, Donor Screening, and even more so actual Donor Deferral, were the areas contributing most to this risk. Areas for staff training in need of increased attention were identified.

#### Hemophilia Treatment Center (HTC) Operations Data

COTT attended a two-day meeting in July concerning data on and produced by the nation's network of 122 Hemophilia Treatment Centers. Presentations of past and present data collection activities were given by CDC, the Homeostasis and Thrombosis Research Society, the Hemophilia Utilization Groups Study, and ATHN and NHF. This was a medical-data-focused conference; no presentations or questions addressed costs and revenue questions.

#### > COTT Operations

COTT's proposal to identify and develop a profile of the one-third of the hemophilia community who do not use Hemophilia Treatment Centers (HTCs) was approved by the Centers for Disease Control this summer, but unfortunately fell victim to the severe budget cuts preoccupying Washington this summer. We are pursuing the idea, and planning to work collaboratively with other organizations as well as seeking support from other private and public funders, including two of the Institutes at the National Institutes of Health (NIH).

#### **BLOOD SAFETY SUMMARY**

Due to the length of this issue of the **Update** and the large number of FDA Enforcement Reports since the last issue, we are omitting what would be a many-page Extracts from Enforcement Reports from this issue.

COTT acknowledges the assistance of Hemophilia Health Services and Factor Support Network in publication of this issue of the COTT Washington Update.

## Attachment #1: COTT Testimony on Informed Consent

# **Committee of Ten Thousand**

*Advocates for Persons with HIV/AIDS and HCV*

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Remarks Concerning Informed Consent  
Advisory Committee on Blood Safety and Availability  
December 5 & 6, 2011

Good afternoon. My name is Dave Cavanaugh, and I am Government Relations staff for the Committee of Ten Thousand, also known by its acronym, COTT.

### Introductory Remarks:

Ladies and gentlemen, I will be only the second person at this two-day meeting to mention the word "chronic," which I would have hoped would be a larger part of the discussion. The entire emphasis has instead been on one-time events; transplants and the like. I understand that if you have a person who needs an eye, and there was a fatal car accident last night at midnight from which an eye could be harvested, you don't have much of an opportunity to check the safety of the organ much less seek informed consent from the family.

However my organization basically grew out of the HIV contamination of the blood supply and its impact on the hemophilia community. At the time, and it wasn't too long after HIV itself was sweeping through more and more communities, let's put it that way, it was thought to be a gay disease until people with hemophilia and infants contracted it. We worked with the folks in the MSM community and worked at getting FDA to improve the processing and approval of AIDS drugs. We did this often outside the buildings, sometimes we got in, and we actually succeeded in getting people on Advisory Committees, as we have two gentlemen here today representing the consumer population.

It's been clear to me from these two days that, in the case of transplants and related procedures, a 42-page Informed Consent form is not paid attention to by the consumer or the provider. The education if given is given verbally or non-verbally by the provider to the consumer and a choice is made -- which has very little chance of being **not** to proceed.

I think the reason for that (this excessively legalistic but in fact quite slipshod way of carrying out Informed Consent requirements) is because there is no feedback loop. There's no organized way that a patient viewpoint can be understood. It occurred to me today that one of the lessons of the first day of this meeting was, 'get the materials down to an eighth-grade level.' Well, in this committee of physicians, I didn't hear any suggestions on how to do that. I don't know what you would take back to your organizations that would get that **done**. Would this mean asking people with fewer and fewer credentials to begin translating the consent forms such that you get closer and closer to that eighth-grade level? I went to an AIDS conference quite a few years ago and bought a T-shirt that said "Patients Aren't Stupid." If you have an average eighth grade level, that mean you have some people with graduate degrees there. Be fair. The holistic treatment of people -- we don't like the term "patient" in my organization; we prefer consumer or end user,

because hemophilia factor can be taken at home because the medical interventions regularly required aren't that great. But definitely 'customer' would work, or 'consumer.' And there needs to be a feedback loop, other than their only recourse at present so often, going into court to seek redress in malpractice suits, which these days are often to the family's chagrin considered frivolous and thrown out.

You need to listen to the people you work on. Otherwise you might as well call them 'husks' or 'shells' -- you're not acknowledging their ability to reason, and to enquire, and to learn. We have heard some mention of internet searches and using Googling, I understand that. But the one-on-one, before the procedure, needs to be higher quality.

#### Prepared Remarks:

Persons in the hemophilia community who were infected with HIV and hepatitis C when these were found to be contaminants in the US blood supply, were given little or no advance information from which to make an informed decision regarding choice of medications. As with many within and outside this group in those years, when HIV tests were given, in many cases both the testing and the results were kept secret from the patient. In the midst of a chaotic time in medical ethics, people with hemophilia learned that many in their community were falling ill and dying. The morbidities and mortality from the hemophilia community's infections with these contaminants continue today, thirty years later. It should come as no surprise to medical practitioners and policymakers that this group, including both affected family members and the infected themselves, remains highly distrustful of medicine in general with regard to such matters.

Some of the key subcategories of the study and practice of Informed Consent have been shared here in the last two days, such as how risks and benefits are portrayed in advance to the patient. Others have not, but in our experience should be of interest to all providers: how the contamination has traumatized our entire community, and how these experiences contribute to depression and affect adherence. It has been suggested that the twin trauma of hemophilia followed by testing positive for HIV and/or HCV through no action of the individual have consequences many acknowledge as elements of Post Traumatic Stress Disorder (PTSD).

The severity of the consequences of improperly administered (or NOT administered) informed consent cannot be ignored. The Institute of Medicine's report on the HIV contamination of the US blood supply, *HIV and the Blood Supply: A Crisis in Decisionmaking* was issued in 1995. It contained a valuable series of recommendations, including the creation of this Committee. Many of the recommendations have been enacted; others are more challenging. For example, one, calling for a widespread change of practice, speaks to many of the points we have heard in presentations and Q&A at several points throughout the agenda: **"When faced with a decision in which all options carry risk, especially if the amount of risk is uncertain, physicians and patients should take extra care to discuss a wide range of options."**

In chronically ill populations especially, Informed consent is an ongoing matter. "All recombinant factor is safe" may be a blanket statement heard these days by proponents of such products, including providers; it informs not at all as to dosing and effectiveness issues and emerging trends concerning inhibitor formation. Glossed-over information from proponents is not patient education, and does not support Informed Consent.

At the core of Informed Consent is the question of the trust of those treated in the medical profession, and physicians who are delivering treatment and care. Medical betrayal, the very opposite of this, is a common theme that has been reported to COTT repeatedly. The alienation and jeopardization of care which this engenders is critically serious, especially in the AIDS/HCV survivors and their families. This ongoing lassitude concerning patient relations, given that in the hemophilia community the use of high-cost medications needed for a lifetime, compounded in too many cases by the need to also treat complex conditions of HIV and HCV, furthers the isolation and alienation from the Hemophilia Treatment Centers (HTCs) in up to a third of the hemophilia community.

As the AIDS/blood and HCV epidemics move farther into history we have seen this marked increase in individuals' sense of isolation and alienation, including from the larger hemophilia community. These individuals perceive the majority, those seeking care regularly at HTCs, as uneducated regarding the devastation that occurred across the hemophilia community in the 1980s and 1990s. The release last year of the movie "Bad Blood" has helped those who went through it find some closure, and helped the many more who have come up since that horrible time to appreciate the constant vigilance needed now and in the future in the area of blood safety by all in our community.

It is more important than ever that providers seek a better understanding of the underlying mechanisms of complications and the potential opportunities for the intervention and prevention of PTSD, trauma and depression to reduce the consequences or impact of complications. Trust is key in doing so.

#### Concluding Remarks

I obviously have limited my remarks to the hemophilia community, which has not otherwise been discussed here, but chronic issues of repeated blood donations -- when a person with hemophilia, as many did about eight years ago, converts from plasma-derived factor to a synthetically manufactured recombinant factor, was there Informed Consent signed? Given and signed? I don't think so.

That sort of thing. I think we all learned to pay more attention to it today. I'm not sure but I appreciate the fact that it was selected as the primary topic for this meeting, but there's a job here too: take it home and make it work.

Attachment #2:  
COTT Testimony  
Affordable Care Act Consumer Advocate Input Session  
October 2011

The Committee of Ten Thousand (COTT) is a community-based organization representing persons with hemophilia, with particular commitment to those infected or affected by the HIV and HCV contaminations of the US Blood Supply. The greatest impact of this contamination in the hemophilia community occurred in the 1980s and 1990s, when we lost thousands in this small community. Those not yet gone, and those affected by such losses in their families and beyond, still -- in the second decade of the twenty-first century -- live lives rooted in this disaster, and need specialty care for their multiple conditions.

COTT's first and foremost concern, growing out of this history, is for the safety of the US Blood Supply. Fortunately science has provided an alternative to human plasma derived blood products for the treatment of hemophilia, but vigilance is required more than ever: there will be a "next one."

Our concerns today come as representatives of those with a chronic -- and also very costly -- rare condition. We have worked with the American Plasma Users Coalition (A-PLUS) throughout the period of regulatory development on the Affordable Care Act (ACA), and you have received input from our coalition. We wish today to underscore several vital points.

A set of minimum Essential Health Benefits published for state Exchanges cannot be built solely around the needs of "typical" consumers. Our medication routinely costs insurers covering individuals in our community \$10,000 - \$25,000 per month. For a normal life span. The conjunction we are indicating that describes our needs -- **very high cost AND chronic** -- means that no normal payment plan based on what a small employer may offer can possibly address our needs.

As individual members of our community embark on coverage under Qualified Health Plans through the state Exchanges individually, we see several areas in which they will need protection:

- It is vital that people with hemophilia are provided with open access, to all medications (different brands of Anti-Hemophilic Factor, although they all address the lack thereof in our bodies, work slightly differently -- they are complex, biologic proteins; one may work effectively for one consumer but not for another), all specialty providers, in- and out-of-network as may be needed (including for our HIV and HCV needs), at all appropriate sites.
- Utilization Management controls under Qualified Health Plans cannot include the increasingly widespread (and ethically challenging) tools such as Step Therapy, Prior Authorization, or Preferred Drug Lists lest excruciating pain and orthopedic damage occur repeatedly for those who thought they were adequately insured. A prior authorization requirement when a child is brought to a Emergency Department with a cranial bleed can cause the death of the child.

- Our members with very high cost chronic conditions must be protected from specialty pharmacy "Tiering" policies, currently only seen in Part D while our needs are met under Part B, of raising the co-pay on the highest cost medications well beyond Medicare's 20%, to 30, 40, and apparently soon to 50% of cost. Even at 20%, few persons with hemophilia, even if well employed, can afford \$5,000 each month for the co-pay on public program support for care of a condition they happen to have been born with.

As a final note we underscore the importance of adequate federal oversight of state Exchange development, Qualified Health Plan certification, and regulations and procedures developed for award of "waivers" from federal program requirements -- such as Essential Health Benefits.

Thank you for the opportunity to share these concerns with you. We are optimistic that the ACA will bring about improvements in care and a turnaround in the steadily increasing cost of that care, in time, but are concerned about the plans that will actually be what our members face. We hope you understand.

## Attachment #3: A-PLUS Coalition Principles for the Essential Health Benefits Package

### **American Plasma Users Coalition (A-PLUS) Principles For the Essential Health Benefits 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-1 Antitrypsin Deficiency, Guillain-Barre Syndrome/ Chronic Inflammatory Demyelinating Polyneuropathy; Hemophilia, Primary Immunodeficiency Diseases (PIDD) and Platelet Disorders. As evidenced by their diagnosis, these patients represent the rarest of rare disorders and need life-long access to expensive, life-saving treatments. With continued access to these extremely effective treatments and therapies, as well as specialized medical professionals, our patients are afforded the opportunity to lead healthy productive lives.

We applaud the recent efforts of the Center for Consumer Information and Insurance Oversight (OCCIO) as it has implemented provisions set forth by the Patient Protection and Affordable Care Act (PPACA). As the next step in that process, A-PLUS believes the Essential Health Benefit Package (EHB) must provide affordable, quality coverage for all Americans and must take into consideration and account for those patients who suffer from rare and chronic diseases. These individuals present unique challenges within the health care system, as many **rare diseases** require expensive, complex and specialized treatments.

The patient protections below, endorsed by A-PLUS, can be achieved through a balanced benefit design that offers affordable choices without compromising the quality or transparency of the benefit. The following principles highlight our unique needs and concerns regarding the EHB Package:

#### **General Recognition of Rare Diseases**

We call upon all policymakers engaged in the current health care reform process to ensure that the EHB Package fully addresses the needs of all Americans including the specialized needs of individuals with **rare diseases**. For individuals with these conditions, “one size does not fit all.”

#### **Access to Specialists**

Requirements for qualified health plans must ensure that minimum patient protections are met in every state’s Exchange plans. Individuals with rare diseases require services and care from members of the medical profession who have specialized knowledge of the diagnosis, treatment and management of their disorders. Qualified health plans utilizing a provider network must provide an adequate number of in-network providers in various specialties corresponding to the EHB categories of health services. Streamlined access to specialists is critical for people with rare diseases. Qualified health plans should also have a mechanism in place for individuals with extremely rare diseases to receive services out of network without incurring astronomical additional costs.

#### **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.

#### **Access to all Sites of Care**

Patients with rare and chronic conditions must have access to the site of care that is determined by the patient and his/her physician. Because our patient populations have the need for life-long treatment and not episodic care, it is important to take into consideration the site of care that works best for the patient – whether that be in the

hospital, hospital out-patient, a physician's office or in the home. Restricting treatment sites is a barrier to access to care.

### **Cost-Sharing**

It is vital for patients with rare diseases to choose a health insurance policy that will meet their unique needs. Therefore, plans must be required to disclose to all prospective and current members information about the deductible, co-payment and co-insurance amounts that are applicable to in-network and out-of-network covered services as well as any limitations on services. States should be provided with oversight mechanisms allowing them to review plan benefit design ensuring that cost-sharing does not discriminate or unfairly target any patients or rare disease groups.

### **Continuity of Care**

Patients who may find they need to switch enrollment between qualified health plans or Medicaid plans must have protections in place so they do not have to seek reauthorization of services or treatments. Comprehensive educational programs must be offered through state Navigator programs that will provide information about the potential implications of switching between plans.

### **Medical Necessity Determinations, Appeals, and Grievances Processes**

A-PLUS strongly agrees with the following recommendations previously provided by the National Health Council (NHC): Requirements for plans to use medical necessity criteria should be objective, clinically valid, and compatible with generally accepted principles of care. Furthermore, plan denials, based on lack of medical necessity, should explain in clear language the criteria used to make the determination. Uniform exceptions and appeals processes and requirements for states to perform plan oversight must also be included in any regulation. Easy-to-access plan grievances processes and a system to track grievances and oversee plan responses to grievances filed.

### **Utilization Management (UM) without Discrimination**

Health plans' UM practices should not impose unfair nor discriminatory requirements for plans to disclose to all prospective and current members all utilization management techniques. States must be provided with specific oversight mechanisms allowing them to review plan UM policies and to guarantee that plans are meeting federal requirements.

### **Summary**

A-PLUS asks for the opportunity to meet and work with the Department of Health and Human Services to assure that patients with rare diseases will be able to receive the care they need, in the time they need, and in the setting that is the most appropriate.

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

For further information, contact Larry La Motte, Immune Deficiency Foundation,  
llamotte@primaryimmune.org or 443-632-2552.