

with contributions from Charles Waller and Bill Speir

THE GLOBAL FINANCIAL CRISIS

and the Plasma Protein Patient Community

AS WE ENTER THE FOURTH YEAR OF THE GLOBAL FINANCIAL CRISIS (GFC),

troubling indications are emerging that the community of patients needing plasma protein therapies (PPTs) are potentially very vulnerable to its effects. Authorities worldwide, faced with collapsing economies and mounting budget deficits, are taking the axe to all aspects of health care spending, and this threatens to engulf patient communities with rare diseases.

The cost-effectiveness pressures

In previous issues of The Source we have described the techniques of health technology assessment (HTA), particularly the use of decision analysis to analyze cost-effectiveness. We have described the role of the quality adjusted life year (QALY) in ranking the cost of health care interventions and assisting payers in ranking and prioritizing resources. These techniques have been developed for ranking interventions involving large populations of patients, given drugs which may need extensive clinical trials to demonstrate benefits. In many instances, these benefits may be marginal and the use of a tool to quantify their cost-effectiveness and allow ranking is understandable.

In the case of PPTs for rare diseases involving deficiencies in e.g. coagulation factors, immunoglobulin etc, the benefits for these treatments are entirely clear and indeed, in many countries clinical trials designed to prove their efficacy are considered unethical; for example, trials comparing prophylactic to on-demand treatment for hemophilia are not performed in Sweden as it is considered unacceptable to subject young children to the risk of bleeding. Therefore, the techniques of HTA are not necessarily applicable to PPTs and, sometimes, may impede access to treatment.

In particular, the use of the QALY as a tool in ranking resource allocation needs to be scrutinized critically. Two elements of the estimation of the QALY are, if applied dogmatically, particularly damaging to patients who need chronic treatment for rare disorders. These include the practice of *discounting benefits*, which leads to the minimization of benefits which accrue over long periods of time. An example of this is the prevention of joint bleeds in hemophilia,

AT A GLANCE

Some examples of Government actions

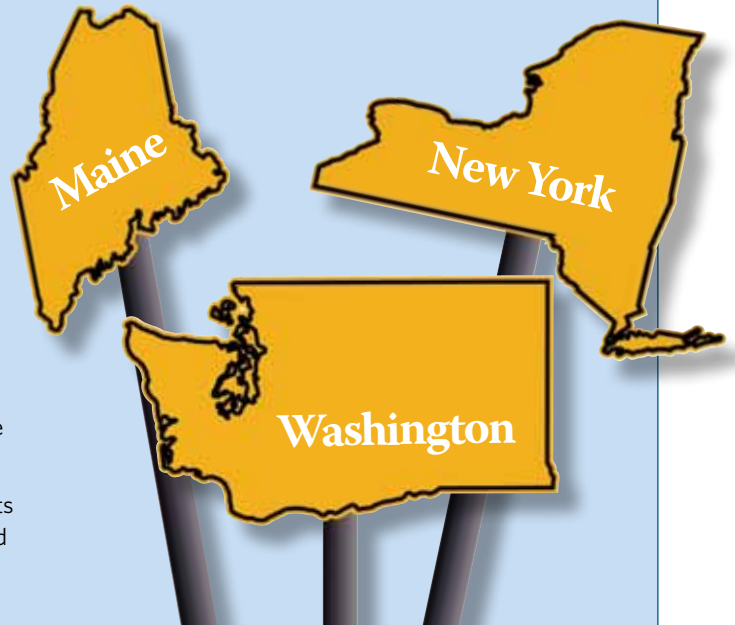
- › France introduces across the board reimbursement price cuts from January 2011 including clotting factors
- › Spain cut pharmaceutical spending by 2.4% in 2010 and introduces reference pricing system with 7.5% cuts to products not in the RPS.
- › Portugal introduces reference pricing system and a new automatic price review system has just been enacted.
- › Turkey introduces reference pricing and in February 2011 implemented a 9.5% discount on all pharmaceuticals.
- › The UK, in 2013, introduces new "value" based pricing in which clinical value in relation to costs and compared to the cheapest product in the same class.
- › Greece announces 30% discounts on generic pharmaceuticals.

STATES TO WATCH

MAINE is developing a request for proposals for a single specialty pharmacy to provide specialty pharmacy products to Medicaid recipients. Since blood clotting factor, IG and alpha-1 proteinase inhibitors are specialty pharmacy products, this could result in a change in pharmacy provider for individuals that use these products. It could also result in an interruption in the continuity of care for these individuals.

NEW YORK is proposing a change in their Medicaid reimbursement for clotting factor to one based on average acquisition cost. This change could result in a number of the specialty pharmacies leaving the market. This would disrupt care for individuals with bleeding disorders in New York.

WASHINGTON is considering a policy that would require recipients in their public health programs to have their clotting factor supplied by a 340B Hemophilia Treatment Center.



Lumping all the different immunoglobulin products into one group and then deciding to reimburse the cheapest is scientifically unjustifiable and medically dangerous for patients who react and tolerate different products differently.

which leads to greatly decreased medical costs when treatment is started early. Another example is the prevention of long term lung damage from immunoglobulin deficiency. All these treatments avoid long term disease sequelae and unquestionably decrease medical costs, yet this benefit is minimized, and often totally disappears, in conventional QALY estimates.

The other element which proves problematic in the calculation of the QALY is the use of *utility estimates*, which are derived from questioning individuals such as patients and the general public about their perceived benefits from interventions. For example, such individuals may be asked to rank their preference for prophylactic treatment for hemophilia compared to on-demand treatment. It is well understood that patients with chronic disorders tend to be cautious when faced with a change of treatment. This caution translates into a relatively modest benefit in the cost of a QALY for e.g., prophylaxis compared to on-demand treatment, despite the significant clinical benefits which are acquired.

These difficulties with using the QALY are partly the reason why cost-effectiveness analysis has not been applied to many plasma protein

deficiencies and treatments. However, with payers facing increasing costs in a deteriorating economic climate, some authorities are proposing to use these techniques to prioritize interventions and “ration” care. The danger to patients, exposed to the possibility of being “ranked” below what payers are willing to reimburse, is clear.

Similar or different – pressures on choice

Other cost-containment pressures are evident when payers attempt to group all therapies – e.g., all Factor VIII concentrates, all immunoglobulin products, all albumin solutions – into one category as “similar” or “generic” drugs. Payment is then restricted to the cheapest product in the group. The concept of similarity/genericity is well accepted in mainstream pharmaceutical drugs which are the products of the chemical synthesis of small molecules. This makes such drugs from different manufacturers very similar and comparable in a medical setting. PPTs are made from biological sources including human plasma and mammalian cell lines and are very complex molecules whose overall product composition is very dependent on the manufacturing processes which vary from producer to producer. Hence,



for example, lumping all the different immunoglobulin products into one group and then deciding to reimburse the cheapest is scientifically unjustifiable and medically dangerous for patients who react and tolerate different products differently. Deciding that PPTs such as albumin are classifiable as generic drugs, as was attempted in an economically challenged European country recently, fails to recognize the differences between products which may well affect patient care.

The way forward

So, as a historical figure once said “What is to be done?” It behooves all those responsible for the treatment of patients with rare disorders, including those dependent on PPTs, to advocate for very careful application of these measures described in this article. If measures such as cost-effectiveness and biosimilarity are really about the best use of the health care dollar, then recognition is needed for the special place of rare and chronic disorders. Otherwise patients will be harmed, and quite likely health care costs will increase. And that is something which everybody seeks to avoid. ☞

ALBERT FARRUGIA is PPTA's Vice President, Global Access

EVERETT CROSLAND is PPTA's Manager, Federal Affairs

CHARLES WALLER is PPTA Europe's Vice President

BILL SPEIR is PPTA's Director, State Affairs

DEFICIT REDUCTION EFFORTS TARGET MEDICARE

BY EVERETT CROSLAND

ON NOVEMBER 21, 2011, the members of the Congressional Joint Select Committee on Deficit Reduction—commonly known as the Super Committee—announced that it had failed to reach an agreement to cut the federal deficit by the statutorily required minimum \$1.2 trillion. The Super Committee's failure triggers a statutory procedure known as sequestration, resulting in indiscriminate across-the-board cuts to the federal budget equaling \$1.2 trillion beginning January 1, 2013.

Largest among the many government programs facing across-the-board reductions are defense and Medicare, with cuts to Medicare capped at 2 percent of total Medicare spending. However, even with the 2 percent cap, Medicare sequestration would result in cuts of as much as \$123 billion over 10 years. Motivated by the blunt force of sequestration, it is likely that Congress will take up the charge of the failed Super Committee and seek targeted savings. While the magnitude and indiscriminate nature of sequestration is daunting, it pales in comparison to the likely prospect that Congress, unrestricted by statutory limits, will focus much of its deficit reduction attention on finding savings in Medicare.