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A roundtable discussion, Slowing The Impact: The Role of Specialty Pharmacy in Managing Progressive and Chronic Diseases, was held on September 29, 2010 in New York to explore and debate the growing role of specialty pharmaceuticals in the U.S. health care system. This white paper is based on the Roundtable proceedings, including an interactive question and answer session among the panelists, moderator and audience.

Executive Summary

Specialty pharmaceuticals represent the single most explosive prescription medications market in terms of growth and high cost. Specialty drugs, many of which are also referred to as biologics, are used to treat serious or chronic medical conditions such as multiple sclerosis, hemophilia, hepatitis and rheumatoid arthritis. They are typically large molecule, injectable drugs and often require refrigeration and other special handling. In addition, adherence is a challenge for almost all drug-based regimens and this is also the case with many specialty products as well. In addition to injectable drugs, the specialty “pipeline” is increasingly filling with newer oral medications, predominantly for cancer treatment, but also for diabetes and other diseases.

There is currently no regulatory pathway for bringing biosimilar versions of specialty drugs to market, although health reform legislation allows for the approval of generic biologics after 12 years of patent exclusivity. While the U.S. Food and Drug Administration (FDA) is actively working to create a regulatory process, experts warn that there will be many challenges along the way from Congress, pharmaceutical manufacturers and others. All of these issues heighten the importance of thoughtfully addressing the delivery and clinical management components of these potentially highly effective, yet most expensive of prescription medications.

Susan Dentzer, Editor-in-Chief of Health Affairs and moderator of the Roundtable, put forth several serious questions to the panelists regarding the role of specialty pharmaceuticals in health care: (1) how do we as a society ensure the right drugs get to the right people? (2) how do we minimize adverse effects and monitor the use of new interventions? and (3) what responsibility do manufacturers, government, insurers, physicians and patients have in ensuring the most efficient use of valuable and finite health resources?

1. The FY 2012 Federal Budget proposal suggests a reduction in the period of exclusivity to seven years.
Roundtable panelists represented the pharmaceutical industry, disease advocacy, pharmacy benefits management, bioethics and the patient perspective. The panel discussion revealed many points of consensus regarding the key issues around specialty pharmaceuticals that need to be addressed, including bringing patients into the decision-making process about treatment and costs, enhancing knowledge about drug efficacy and safety, and questioning the allocation of resources.

The panel proposed the following series of recommendations to address the issues discussed surrounding specialty pharmaceuticals:

- Convene more stakeholder forums.
- Encourage data collection and data sharing.
- Create and support national registries.
- Use and replicate successful models from other fields to convene key stakeholders in making care more accessible, affordable and of higher
- Establish multimedia public information programs to broaden awareness and knowledge of specific diseases and treatment options.
Significant breakthroughs in medical innovation over the last few decades have spurred the development and availability of increasing numbers of specialty drugs for rare diseases. Specialty pharmaceuticals, also known as biologics, provide critically needed treatments for some of the most debilitating conditions, such as multiple sclerosis, some cancers, rheumatoid arthritis, hemophilia and hepatitis, which affect millions of people in the U.S. and around the globe.

The passage of the Patient Protection and Affordable Care Act also paves the way for an increase in ‘biosimilar’ products as the legislation allows the U.S. Food and Drug Administration (FDA) to approve generic versions of biologic drugs, which presumably will be made available at significantly reduced prices.

Specialty drugs offer profound hope and extraordinary promise for patients (and their families) suffering from severe and often life-threatening diseases. However, specialty drugs are often costly, ranging from about $7,200 to $750,000 for a single drug per year of treatment. A recent AARP study reports the average annual cost for a patient taking just one specialty drug is now $34,550. Health care spending in the U.S. continues to grow at an alarming rate; Health Affairs projects that almost 120 percent of the expected increase in the Gross Domestic Product over the next 75 years will go into health care, leaving fewer resources for other vital national priorities, such as education.

As an increasing slice of the overall health care pie, the future costs of specialty drugs – already high to begin with – are of growing concern. The expanding market for these drugs poses serious questions about the best and most cost-effective use of finite health care resources and the roles and responsibilities attached to that use:

- How do we, as a society, ensure that we are getting the right drugs to the right people?
- How do we minimize adverse effects of specialty drugs and monitor the use of new interventions?
- What responsibility do manufacturers, government, insurers, physicians and even patients and their families have in ensuring the most efficient use of valuable and precious health resources?
The term “specialty drugs” is a new one and is used in many different ways, so it is important to remember the true definition. These are products that have the following characteristics:

1. **High complexity**: Specialty drugs include any biotech medication that structurally mimics compounds found in the body, from blood derived factors to customized gene-based therapies.

2. **High touch**: Specialty drugs are high maintenance, typically requiring special preparation and handling, refrigeration and immediate fulfillment. Because of the high-touch component, patients generally need high levels of education and ongoing follow-up care to manage side effects and to ensure compliance.

3. **High cost**: As stated previously, specialty drugs are costly; some oncology treatments run as high as $750,000 per year.

While forming a unique category of pharmaceuticals, specialty drugs are also becoming mainstream. In 2000, only one such drug was on the list of top ten selling drugs. Today, the pharmacy industry’s consensus forecast is that by 2014, six of the top 10 selling drugs will be classified as specialty pharmaceutical products.

However, when the health care community sees a new health intervention that offers enormous hope in addressing serious health concerns but at an expensive cost, it is critical to ask how the collective community wants to address this. The health care industry is becoming increasingly polarized on this issue, but will not be able to develop meaningful answers and solutions unless broad perspectives are brought to the table and honored.
From the pharmacy benefit management (PBM) perspective, it is critical to ask whether incentives are aligned appropriately between the patient and the payer. For example, should there be lower co-pays for more effective medications and, importantly, vice versa? Should there be strict step therapy that requires patients to try less expensive, but largely equally effective and perhaps less complicated, treatments before moving to more expensive ones? Should specialty medications be submitted to a rigorous cost benefit analysis before PBMs put these drugs in a preferred position on formularies?

Should drug price reflect value, the amount of research and development effort, or the R&D effort invested in similar drug development that is not brought to fruition? In some cases, PBMs are approached by pharmaceutical companies with the statement that money will be refunded “if drugs aren’t cost effective or if they’re not therapeutically effective.” The critical questions are whether this process is administratively possible and if so, is it actually engendering a desired relationship?

As a larger society, we must ask if we are doing a good enough job rewarding drug manufacturers for investing in the development of curative drugs for diseases most in need of attention. At the same time, it is critical to incentivize people to live healthy lives and engage in preventive health where possible to help avoid or reduce the need to take medications. The rate of smoking has fallen over the past decades, but not necessarily in the adolescent population. Perhaps a more striking example of the critical need for preventive health is the impending obesity epidemic the U.S. is facing, which affects both younger and older individuals. It is important to question the role society plays, or should play, in incentivizing people to engage in healthier behaviors.

Government plays a critical role in the specialty pharmacy arena. Government is, and has been for some time, the largest payer in health care. The new health care reform bill grants 12 years of exclusivity to biologic drugs, after which biosimilars and competitive manufacturers of brand-name biologics can enter the market. However, perhaps government should grant longer periods of exclusivity for drugs that make significant gains in controlling symptoms and that are curative; perhaps a “one-size-fits-all” law is not the best approach.

Providers, specifically physicians, also play an important role in this discussion. Perhaps providers should require that all patients given specialty drugs enroll in a registry to monitor and track use. This is especially important when

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2. The FY 2012 Federal Budget proposal suggests a reduction in the period of exclusivity to seven years.
patients are given drugs for off-label indication – that is, for use not approved by the FDA – which may even warrant official and scientifically sound clinical trial enrollment. Registries and clinical trials help provide information on which drugs work well, under what circumstances, and for what cost; they also demonstrate which drug regimens do not work well. A successful example of this approach can be found in pediatric oncology: physicians in the field have developed excellent treatment regimens by organizing themselves and placing patients in coordinated registries and clinical trials.

Lastly, patients themselves have a responsibility in how they use specialty drugs. Adherence is an enormous problem in this country, not just for specialty drugs but even for small molecule drugs. However, if a patient is receiving $750,000 worth of a specialty drug, should he or she have an obligation to be adherent? Perhaps there should be consequences for non-adherence, with the responsibility shared by patients, physicians, PBMs and health plans.

Education is an important part of the equation, but it is important to understand how best to utilize education and what types of education are needed. Perhaps before beginning a treatment regimen, patients should receive objective educational programming explaining their condition, the goal of the regimen, the expected treatment consequences (including consequences for not adhering), expected side effects and anticipated co-payments.

Patients’ families must also understand the full picture of specialty drug regimens. For example, patients may face drug therapies costing $200,000 that add two- to three- months of life but with a co-payment price tag of as much as 20 percent, or $40,000. This information may significantly impact families’ decision-making processes.

Exploring the questions and considerations above is essential to collectively addressing the challenges posed by specialty pharmacy, and to maximizing the benefits of a new and exciting era of medical development. Prescription Solutions and its sister company UnitedHealthcare deal with these issues on a daily basis as facilitators of access to quality care and as stewards of health care dollars.
Specialty Pharmaceuticals and the Importance of Evidence-Based Medicine

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Specialty drugs have made some major scientific breakthroughs. In cancer, for example, three drugs have been transformational: Gleevec, a pill that is easy to take, has extended the survival of chronic myelogenous leukemia patients by many years, and even offers the potential for a cure; trastuzumab, also known as Herceptin, reduces the chances of relapse and death in women who have an over-expressed HER2 gene by half—a very significant gain; and rituximab has been lifesaving and curative for a large percentage of patients who have non-Hodgkin’s lymphoma.

These drugs are transformative, but they have also focused the spotlight on some major issues within the field of medical care and health policy. The first involves understanding the limitations of drugs.

When Avastin, a colorectal cancer therapy, was released and shown to extend the lifespan of patients with relapsed disease, there was enormous enthusiasm about the drug’s colon potential. Oncologists began using the drug in ways that testing had not yet proven, such as administering it immediately after surgery as a curative drug. Clinical trials now show that the drug has no value in this setting. Moreover, prior to the completion of clinical trials, enthusiastic physicians combined Avastin with another biologic, cetuximab, which also had shown good activity in colorectal cancer. The subsequent clinical trial showed that while effective when administered alone, the drugs in combination actually worsened the prognosis. The logic was good—it made sense to combine these drugs, but we have to complete the trials to understand the limitations of these medications.
These examples highlight a “habit” in U.S. health care of using promising new technologies and expanding their applications without undertaking the appropriate scientific studies to evaluate the effectiveness of other uses. This practice is costly, both financially and in terms of patient toxicity. There is a need to address how to manage “off-evidence” use of potentially great ideas that have not yet undergone clinical trials. This happens every single day in the clinical setting, and again, it is very costly and wastes very precious resources. As my colleague, Jackie pointed out, registries and clinical trials offer some solutions.

A second health policy issue centers on the cost of technologies. Specialty pharmaceuticals are expensive, but also serve as examples for other new technologies. Perhaps the big-picture question is, how much can the U.S. afford to pay for an added year of life or an extra year of significant quality of life? Instead of answering this question, this country has essentially avoided the issue by just paying for technologies, which has been followed by insurers raising premiums. Insurers cannot realistically be expected to absorb the rising costs and remain viable participants in the health care system. The rise in premiums leads to more and more people dropping out of the health insurance system, resulting in inadequate or no coverage.

With 51 million uninsured individuals in this country, it is critical that we examine the health care affordability issue. There are wonderful breakthroughs and advances in medicine, yet specialty pharmaceuticals highlight the larger problems that challenge the health care system and can serve as a model for understanding and answering questions of cost and affordable health care access.
Patients as People: The Cornerstone of Biopharmaceutical Innovation

Ross Maclean, MD, MBA

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A key piece of this health care conversation includes the people suffering from progressive and chronic diseases. The biopharmaceutical industry is one of the leading agents driving innovation in health care and health care delivery; this innovation exists to serve patients who, before they became patients, were people and remain people throughout the entire health care experience. The U.S. leads the world in discovering and developing innovative medical therapies. Innovators with true leadership must be sensitive to the environment in which they operate and also to the needs of customers.

Biopharmaceutical innovators must have a fundamental understanding of the needs of customers beyond regulatory agencies, including patients, health care professionals and those who bear financial risk.

Patients, or more accurately people, are the cornerstone of the biopharmaceutical industry. These patients have families, jobs, histories, expectations and behavior patterns, all of which influence their health behavior and their health outcomes. Biopharmaceutical innovation needs to be anchored in an understanding of patients that is deeper and richer and more insightful than at any time in the past. Understanding these needs and priorities can help innovators design for value and bring to market the products people really need.

For the biopharmaceutical industry, key questions include how medicines are likely to be used in day-to-day care, especially for patients struggling with co-
morbid medical conditions. Similarly, how can medical care shift from focusing on one treatment for one disease when patients have multiple diseases and conditions? How will taking medicines deliver value to a patient? What does that value look like? For example, in one person’s family, value could represent missing fewer days from work or school, resulting in an ability to care for a failing elderly relative. Value may be having a sense of reassurance that new medicines for a specific cancer are a match to an inherited genetic code, or being comfortable that the out-of-pocket investment for occupational therapy for a family member has been studied and proven to work.

These issues are both challenges and opportunities for biopharmaceutical innovators. Innovators who truly understand the needs of patients and the importance of value will be able to embed that value into the R&D process. Those who understand the dynamics of the health care quality and performance measures environment will be able to align the value of their portfolio with what society believes is worth delivering and paying for. Those who understand the dynamics of the comparative effectiveness research environment will be able to generate scientific and medical information that is available in a timely manner, so it can be embedded into independent synthesis of available evidence. Lastly, and most importantly, innovators who realize that patients put their trust in medical innovation will ensure that they demonstrate scientific integrity at all times to sustain that trust and to build respect as innovators.

Specialty biopharmaceuticals represent highly advanced science. Familiar statistics about these drugs include cancer patients living an average of three years longer, with 83 percent of those survival gains attributed to new treatments. Heart failure and heart attack deaths fell by nearly half from 1999 to 2005 because of these drugs.

However, the cost of developing these drugs is higher than for traditional small molecules, and now costs about $1.2 billion on average. Furthermore, specialty biopharmaceuticals frequently address diseases affecting fewer patients than traditional medicines.

Importantly, these advances are driven by a U.S.-based medical innovation machine. According to the Tufts Center for Drug Development, 75 percent of all new drugs approved worldwide between 2005 and 2007 were first introduced in the U.S. This homegrown innovation not only saves lives, but also

Innovators who truly understand the needs of patients and the importance of value will be able to embed that value into the R&D process.
employs millions of Americans and pumps billions of dollars into the nation’s economy. Unlocking the genetic code may bring even more promise in finding biomarkers for specific diseases. The prospect that diagnostic products will accompany medical products is an exciting forefront that may bring even greater value to a focused subset of patients.

Today, treatment of chronic conditions accounts for nearly 75 percent of all health care spending. Prescription medications represent the best value for money in today’s health care system, and they play an important role in disease prevention. Unfortunately, though, medicines are often evaluated based on cost rather than on savings, even though they can help reduce unnecessary hospitalizations, avoid costly medical procedures, increase productivity and even slow the growth of health care costs. But, appropriate preventive action can reduce the need for costly health care services. For example, recent research estimates that delaying the onset or slowing the progression of Alzheimer’s disease by about five years could save Medicare and Medicaid as much as $100 billion by the end of this decade...
The opportunities around innovation and collaboration are all around us. Innovative stakeholder collaborations can further drive the safe, effective, and appropriate use of (and value of) pharmaceuticals. For proof, witness recent announcements by insurers and health information technology organizations collaborating to maximize the value of data; government regulators and insurers exploring the use of health care administrative data to support safety monitoring; and IT agencies and biopharmaceutical companies aligning around what an R&D program should look like to facilitate a future successful health technology assessment. An additional example is health insurers and biopharmaceutical organizations coming together to co-develop medicines, sharing the risk and reward, and optimizing development of medicines in populations that will ultimately be likely users. Throughout all of these collaborations and endeavors, it is critical to keep the focus on the patient.
Key ethical issues arise from the growth of specialty pharmaceuticals. Ethical concerns can guide the choice of one course of action over another, with reference to values and principles. Ethical dilemmas arise when values are competing with one another.

Health care professionals are obligated to act in the best interests of their patients, but it is not always clear how this works in practice, nor is it always clear how to resolve competing interests. While there are a large number of ethical issues that are germane to the pharmaceutical discussion, perhaps the three most important questions are: Is the drug effective? Is it safe? Is it equitable?

With regard to effectiveness, it is easy and yet hazardous to fall back on metaphors – the pipeline, the breakthrough, the innovation – when talking about what biologics and other newer therapies can do for patients with serious illnesses. However, these metaphors do not convey to the patient, or his or her loved ones, what the goal of the particular treatment is. When a progressive or chronic disease is potentially treatable but not curable, the goal may be the stabilization of a disease process, the relief of symptoms, the prevention of recurrence, or something else entirely. Good ethics requires clarity concerning the goals of treatment, enabling a patient to make an informed choice among treatment options. It also requires clarity concerning a treatment’s potential for a physiological benefit relative to the burdens of treatment.
It must be made clear to patients and other stakeholders that the availability of a drug or its coverage by insurance is not what makes the patient’s condition medically manageable. Health professionals must constantly evaluate the quality of evidence, which demonstrates whether a drug’s effectiveness is better compared to other interventions with the same goal. Reversals of professional and regulatory opinion are hard on patients living with advanced cancer and other life-threatening conditions. Therefore, it is essential to be clear about what constitutes evidence of effectiveness.

Ethical safety, and preventing harm from medication-related errors, is integral to good care. Specialty pharmacy faces unique challenges because these regimens may require a high degree of patient self-management. Self-administration of treatment can be experienced by the patient as a benefit – more privacy, more control over personal time – but also as a burden. Patients who no longer travel to clinics for treatment may feel unsupported or apprehensive, particularly when a new treatment is being introduced. Ethically, supporting patient self-management should never be reduced to mere compliance or to transferring responsibility of safety entirely to the patient.

Chronic disease management is a form of social ethics in that it aims to provide structures and conditions that promote positive outcomes and reduce the risk of negative outcomes. The common chronic disease paradigm includes the obligation of physicians to collaborate with patients and help them integrate treatment and treatment decisions into their everyday lives. It also calls upon nurses and other health care professionals to ensure patients are taught the skills needed for self-management, including the ability to identify problems in their care and to participate in finding solutions.

However, paradigms for managing complex progressive or chronic diseases that involve specialty care are still taking shape. In fact, there may be no clear pathway to helping patients manage chronic illness or symptoms. Within the hospital culture, the outpatient setting is less visible than the inpatient setting. Because of this, hospital staff may believe that convenience of treatment is what is most valued by a patient, while the patient may place a higher value on access to support. Therefore, safety in the self-management paradigm requires specialty pharmacy to provide, and invest in, structures that demonstrably serve the best interests of the patient.

Other factors central to the issue of health care equity are cost and access. The intersection of access and cost involves hard questions about reimbursement,
out-of-pocket costs, and how these are addressed when treatment options are presented to patients. Health care professionals should avoid creating economic hardships for patients and loved ones whenever possible. Medical specialists should be prepared to talk about the cost of specialty pharmaceuticals when discussing treatment options and making recommendations, even when an expensive treatment is covered by a patient’s current insurer.

Research suggests that physicians need help in learning how to discuss the issue of cost with patients. The results of a national survey of oncologists, which appeared in *Health Affairs* earlier this year, found that oncologists are influenced by their knowledge of treatment costs but also reluctant to discuss these concerns directly with patients. Though 84 percent of respondents said treatment recommendations accounted for patients’ out-of-pocket expenses, only 43 percent reported actually talking with patients themselves.

Health outcomes relative to socio-economic status raise further ethical concerns, particularly in the U.S., where an estimated 200,000 patients with cancer are uninsured. Spending on this group of patients is lower, correlating with lower-income individuals having inadequate or no health insurance, and therefore, more likely receiving an initial diagnosis of advanced disease. It’s time to ask the complex question about whether or not uninsured or underinsured patients have equitable access to treatment. It is critical for all stakeholders to recognize and evaluate the key ethical issues arising from the growth of specialty pharmaceuticals.
The availability of specialty pharmaceuticals has had a major impact on multiple sclerosis (MS). MS is a chronic, and often disabling, disease that attacks the central nervous system, which is composed of the brain, the spinal cord and the optic nerves. Symptoms can be mild, such as numbness in the limbs, or severe, such as paralysis or loss of vision. The progress, severity and specific symptoms of MS are unpredictable and vary within each individual and from one individual to another.

MS is thought to be an autoimmune disease where the body’s own defense system attacks myelin, the fatty substance that covers the nerve fibers in the central nervous system. There is no cure for MS and it is not yet known what causes it, but fortunately there are now several medications used to slow the progression of this disease.

The first MS disease-modifying therapy was introduced in 1993. The medication, Betaseron, is manufactured by a biotechnological process from a naturally occurring interferon, a protein released by the immune system. Betaseron is administered as an injection taken every other day. Since its introduction, seven other MS disease-modifying therapies have been produced: three injectable medications, one intravenous (IV) infusion treatment, and as of October 2010, the first oral treatment specifically for multiple sclerosis. Additionally, there is a form of chemotherapy used to treat people with a more aggressive form of MS.
These medications have significant side effects for many patients, causing flu-like symptoms immediately after drug administration, though these usually subside over time. The chemotherapeutic agent causes many of the side effects often associated with chemotherapy, including susceptibility to disease, nausea, hair thinning and mouth sores. The IV-infused medication can cause headache, fatigue, urinary tract infections, depression, lower respiratory tract infections, joint pain and chest discomfort. In some rare cases, people have developed a very serious disease called progressive multifocal leukoencephalopathy (PML), which can lead to death.

Even the recently approved oral medication has been linked to side effects including flu, diarrhea, back pain, liver enzyme elevations and cough. Additionally, its label carries warnings about potentially serious risks, including decreased heart rate and/or other heart effects after the first dose. Other potential risks include infections, macular edema, decrease in lung function, slight increase in blood pressure, increases of liver enzymes, and harm to fetus.

After reviewing the side effects, it is clear why treatment decisions are often challenging for patients. And yet people are faced with these choices frequently and do make these decisions. Moreover, many people taking these medications have experienced years without a relapse in symptoms and doctors have seen a reduction in the evidence of MS for these individuals.

As with other specialty drugs, MS medications are expensive. The injectable medications cost between $2,200 and $2,600 each month, and the IV-infused medications similarly cost an average of $2,300 to $2,400 monthly. Therefore, it is no surprise that the cost of these medications acts as a barrier to access.

People with MS regularly experience difficulty affording the medications, even though most people with MS do have some form of health insurance, whether public or private. In fact, about 94 percent of people with MS have health insurance. Many health insurers do include all of the MS disease modifying therapies on their drug formularies and some individuals have very reasonable co-pays for their treatments, such as $25 to $50 for injectable medications. Despite this insurance coverage, many people with MS are dramatically underinsured and face significant out-of-pocket expenses.

One of the most common problems experienced is within the Medicare Prescription Drug Program, particularly with the addition of a specialty tier
People with MS face enormous difficulty in paying for medications, a challenge exacerbated by all of the other health care costs need to just maintain their disease.
Specialty pharmacy products are the present and future of treating MS and other chronic illnesses and conditions. These medications are not inexpensive to research, develop and produce, and their high costs create an obstacle to care. Health insurance plans are responding to these costs, and to the demands of employers who are looking for lower premiums in their health insurance products, by creating mechanisms in which enrollees are responsible for increasing shares of the cost.

The current costing model does not seem sustainable, so it is critical for all stakeholders to come together and agree upon steps for moving forward. Forums such as this Roundtable are a vital first step – and only the beginning – to finding workable solutions to these complex issues.
I’m here to talk about my experience of living with a chronic disease – one that has no known cure yet. I have MS, multiple sclerosis. I was first diagnosed 20 years ago and it was a total shock to me. At that time, very little was known about the disease and medications were just beginning to go into trial phases. My neurologist, who confirmed my MS with an MRI, told me in April 1990 that MS comes in many forms and affects individuals differently. People may experience a wide variety of symptoms, and in my case, my symptoms were very mild. The doctor also said my condition could change at any time and could actually worsen.

I felt numbness in my feet and hands constantly. Sometimes that sensation would spread through my body and then recede and weeks later come back again. But that essentially was, and is, my main symptom even today. My only awareness of MS 20 years ago was my aunt, who had suffered from MS for many years and already was in a wheelchair.

To learn more, I visited the National MS Society to gather as many brochures and reading materials as available, and I attended MS meetings. But I also decided 20 years ago not to tell anybody except my wife, parents and sister that I had MS. I did not even reveal my diagnosis when I went to MS meetings; I would say I was there because of my aunt. If my MS was indeed a mild case and it was not clear to others that I had this disease, why should I bother to reveal the condition? The fact is that most people with MS – certainly those who are still working – do keep it a secret and do not tell their employers for a variety of reasons.
After the third annual visit with my neurologist who found no change at all in my condition, I decided then to not return to my doctor and to stop attending MS meetings. I decided that whatever this chronic disease was for me, it was stable and unlikely to ever interfere in my daily life. I focused on my work and my family. But things do happen – it does change. I learned seven years ago that MS truly is totally unpredictable. In 2003, there were new sensations in my body, and I experienced bouts of incredible fatigue, enough concern to lead me back to the original neurologist. After another MRI, the first in 13 years, my doctor informed me that my MS was very active, there were more lesions in the brain. My disease had, to my surprise, advanced. I needed to find a neurologist – in fact, a whole medical team – that specialized in MS.

“You look a lot better than you should,” the head of the Mount Sinai MS Center told me in April 2003 after examining me and reviewing the original and new MRIs. Shortly thereafter, I began a regimen of self-injecting medication three times a week, knowing that it cannot cure my condition, cannot reverse it, but is essential to managing this chronic disease. I have asked on occasion, even a few months ago, what would happen if I don’t do these injections. The answer always is, “Ken, your condition likely will get worse. You really have no choice. Just keep going with the injections.”

As one can see from my participation in this panel, I am very public today about my MS. This was not easy to decide at all. During the first year of taking drug therapy, I agonized over whether and how to tell my boss about my MS. I have been the public relations director for the past 12 years for a global advocacy organization, often working around the clock. But telling my employer was the right thing to do. It alleviated a tremendous burden on my mind, and since I express myself best in writing, I then was able to write and publish articles about my MS experience.

Over the past six years I’ve come to terms with the reality of my medical condition and how it affects my daily routine. However, I do have very real concerns, some of which are shared by individuals with any chronic disease. A key example of a shared concern is the constant worry about the economics of disease management. Specialty drugs for MS are expensive and their costs have grown significantly in even the past few years. For some, this means suspending or even forgoing medication altogether to meet other daily demands of life. I am aware that it costs a lot of money to do the research and testing of new drugs, but there must be a greater effort to ensure that they are affordable for the patients who, out of necessity, must use them.
I happen to be very fortunate in that I have decent health insurance with manageable premiums and co-pays for doctors and medications (which I do obtain from a specialty pharmacy). But somewhere in the back of my mind, and even sometimes more top of mind, is the concern that my unpredictable MS might change again, requiring a different kind of therapy or medical treatment with all the attending costs. Even if my condition remains stable, suppose there are changes in my health insurance coverage or the cost of medication continues to rise and I need to find new ways to pay.

As a final point, I want to comment on national registries. National registries for chronic disease patients are very important, as they facilitate data collection and positively contribute to research endeavors. It is imperative that the U.S. Congress adopt legislation to establish a national registry for MS and Parkinson’s diseases.
[Addressed to panel]

What are the challenges in developing metrics to determine what therapies work? Specifically, which metrics and research do insurers use to determine if a specialty drug will be covered?

Jacqueline Kosecoff: Metrics are not necessarily financial; for example, one can ask, is this therapy curative? If it’s not curative, does it reduce symptoms? If it does, to what degree does it do that without introducing new problems? You can collect data from the people who are experiencing the disease. There are sometimes physiological markers that neurologists or others will point to that indicate whether there is disease progression or not and what will be predictors of future problems.

If we bring together a group of people, they can collectively come up with metrics that are important – important to patients, important to the scientists and physicians who manage these diseases. And then we can figure out how much it costs. But I look at cost differently. I think that dollars are fungible. The question is whether these drugs really are important and they actually are achieving the goals we want. Let’s first start with knowing what’s important, and are we achieving those goals.

Lee Newcomer: One thing to add is that what we measure must be important to a patient. In the world of oncology there is a lot of measurement of “time to progression,” or sometimes just simply, “did the tumor shrink on an x-ray?” Some drugs do have dramatic effects on shrinking the tumor in the x-ray, but the patient does not notice a single clinical benefit and, unfortunately, does not live a day longer. That is not value.
Those metrics are not as important as the simple question, “How is the patient’s life affected?” If we can do something positive, those are the sorts of measures we should seek and then use those measures to assess the value of whatever therapy we’re examining. We should not worry about how the x-ray changed or how a blood test changed, but rather than how did the patient’s life change? Quite frankly, the second set is harder to measure and it takes a little longer. But they really are the ones that are important.

[Addressed to Ross Maclean]

You talked earlier about the importance of scientific integrity for the biopharmaceutical sector, yet the sector has not always been at one with everybody else on this issue of what should be measured and what metrics are meaningful. Shed some light from your perspective on this question.

Ross Maclean: Looking more to the future than the past, I’d like to look at this registry concept that we’ve been discussing and reiterate the comment that whether we’re talking about what should we measure, or we’re talking about a registry for a disease of interest, I think it offers great opportunities. One is to bring the stakeholders together to align on what the questions are that we’re trying to answer; and secondly, can we use recognized tangible incentives to drive the behavior that we seek? We can collect appropriate data and answer priority questions and use those answers as a base to shape what care we are willing to pay for.

[Addressed to Nancy Berlinger]

What do you think would be the value 10 years from now of being able to point to a model, such as an MS registry, where important information was gathered, particularly on the issue of what really helped patients experience fewer symptoms or have fewer relapses? Could we envision a model like that coming about and, if so, what would need to happen for that to succeed in terms of stakeholder collaboration?

Nancy Berlinger: One of the diseases this happened with was cystic fibrosis, where the rather dramatic increases in length of life came about because clinicians at treatment centers, not just individual doctors, started sharing what worked and what didn’t work. And not all of these were medical interventions. Some of them were other types of therapies.

For other conditions, such as cancer, we think of oncologists focusing on the treatment, but we have to broaden that focus out to the patient who lives with, and faces the consequences of, the treatment. What we’re looking at is the care structure: specialties and professions beyond clinical oncology, such as pulmonologists or hematologists who work in that context. We are often working with nurses and social workers who are the ones who may be in the most direct contact with patients. And those centers can be very, very siloed.

It is necessary, but very difficult, to forge new pathways that are not just scientific and clinical, but also organizational. There are some efforts to put navigators in place, as in the National Cancer Institute (NCI) in Washington, D.C. New efforts such as the Patient Navigator Research Program at the NCI offer a way of saying, let’s look at this from the patient’s point of view in every respect, and that includes looking at the science and how it is presented to patients.
Can you fathom such a model as described above actually coming about and making a difference?

Jacqueline Kosecoff: Yes, in fact, there are examples already today. For example, UnitedHealthcare has a special insurance program for people with diabetes. If you sign up for it, the costs of care are oriented to help people with diabetes get easy access to their medications. But there’s a carrot and a stick: if you are not adherent then you cannot continue in the program.

The patients are being very active in this process, assertively choosing to go into a program like this. If they do, they receive all sorts of help in terms of disease management, care coordination, more affordable drugs and access to specialists, so that they feel like they’re in a community of care giving. There are also examples of pharmacy benefits, where patients with particular conditions pay no co-pay or very low co-pays in order to encourage adherence. I have great hope for these programs, which are small examples in a huge sea of how care is delivered today.

Let’s say it was considered the norm that if you were diagnosed with MS, you had to undergo a patient education program, immediately upon diagnosis, about the disease, drug treatments, etc. before anything happened to you. Would patients accept that?

Kenneth Bandler: They would. It’s an excellent idea. If there were resistance, then patients should be actively encouraged to participate in such a program. I’m fortunately engaged with my doctors and they say, “Call any time if the symptoms change.” The specialty pharmacy that I deal with always says, “Do you want to speak to a nurse? Do you want to speak to somebody else?” That kind of extra care – and I just know about my experience with MS – if that applies to others with a disease like diabetes, then it’s fine. But the question with the diabetes program is how do you track that? How do you know that the patient is really adhering to the program?

A prominent management expert once wrote that there are two kinds of problems: problems one can solve and problems one can only manage; the art is deciding which ones are solvable and which ones you just have to manage. From your own perspective, can each of you highlight the number one issue you wish you could solve and the number one problem you wish you could just manage?

Jacqueline Kosecoff: The key problem to solve is helping Americans coalesce around the fact that although medicine is delivered on an individual basis, its foundation is rooted in science. As a country, we need to appreciate and respect that science, and ensure that people who can benefit the most get appropriate access to therapies, even when there are hard decisions. Our society hasn’t done a very good job in loving science, respecting it or adhering to it. Non-adherence to medical advice illustrates this. Education is important in understanding evidence-based medicine, removing costs, figuring out programs to get the right people the right treatments. Even learning about drugs that do not work well can help open the research agenda.
A “wish list” issue to manage is in delivering care and organizing that delivery profoundly better. For example, in patient adherence, it is not just getting the drug to the person, but getting him or her to follow the regimen, understand the treatment and deal with it. We waste a lot of money by going only halfway in this process. Instead, we need to bring together and engage the physician community, the provider community, the payer community and, very importantly, patients in what are called disease management programs. There is a need to help people with chronic and serious diseases to follow through and experience the value that can come from receiving not just the specialty medication but all the wrap-around care involved in trying to treat a serious disease.

Lee Newcomer: An issue to solve is establishing, as a society, what we can afford. We need a forum determining that we are willing to spend X number of dollars for an additional year of quality of life in any therapy evaluated.

Once that number is established, we must collect the information to help make those decisions. This information includes what the patient experiences in terms of his or her illness, what he or she experiences in terms of toxicities suffered for that gain in quality adjusted life years, the financial toxicities of a particular therapy, and the impact of therapy on daily life. Having this information laid out in a way that everyone could evaluate and consider will lead to better decisions. People need the right information to decide what that choice will be.

Ross Maclean: Minimizing variation in health care delivery is an easy thing to state but very difficult to deliver. We can, however, make important steps in this direction. Mark McClellan, Dana Goldman and many others have published work showing we need to do more of what is already known to work, such as the case with lipid lowering therapy. The central question, then, is how do we promote what we know already works? Answering that question could have a significant impact at a national and societal level.

As a solvable issue, we need to understand why we have a patient population that seems largely disengaged in its own health. Patient investment in health management from all aspects – drug adherence to general health and wellbeing – has proven to drive better health outcomes. Understanding patient behavior is critical.

Nancy Berlinger: A solvable issue is piecing together a fragmented system so that there is an articulated model for managing different complex diseases. Primary care models demonstrate that this can be achieved and may lead to such things as the medical home for specialty care. However, this is a huge country with different states and different reimbursement streams, and sometimes people are resistant to learning from one another. Sometimes people are amenable to replicating successful models and others are persistent in creating a new one.

An issue to manage is how we, as a community, talk about cost, price and value, and how we use those terms. We talk a lot about “patient choice,” but a patient doesn’t choose to have some of these diseases. I am not talking about “lifestyle” diseases, but rather diseases that arise from complex causes. As an example, when we read reports on certain drugs adding a few days of life at a very high cost, we are not talking about chronic diseases or slowly progressing but manageable diseases. Using metaphors such as “pipeline,” “promise,” “next great hope,” or “the last ditch” clouds the actual content and makes it more difficult to understand what the value of intervention truly means.
P.J. Weiner: Finding a cure for MS is the issue we would like to solve. Short of that, the most pressing problem is affordable access to medications. There are so many issues playing into why there is not access for everyone to safe and effective treatments.

A manageable issue is bringing together stakeholders to engage in a dialogue about all of the challenges and concerns posed by the panel. The medical community, the pharmaceutical community, the patient advocacy community and government must all come together now and decide how to move forward, because these issues are only going to propagate with the expansion of new drugs and advances in science.

Kenneth Bandler: As a patient, I am very much a fan of patient assertiveness, be it in a chronic disease context or otherwise. The patient is the customer, and so the patient should be asking more questions of the doctor than the doctor is asking the patient.

This is the attitude we need: to encourage patients to step up and be more assertive in asking questions about their condition, making sure they deal with doctors and other health care practitioners who will openly communicate about different available therapies and explain the risks and potential benefits that can emerge from those therapies. This is an achievable goal but one that requires that patients and doctors work together.

The issue to manage is related to this: more open and coordinated information about chronic diseases is needed to educate and inform people. It is striking how long it takes to develop a drug. Perhaps it is up to the media to take us behind the scenes. Why does it take so long? What are the different elements? Who is involved? Everything from the scientists, the doctors, to the funding that’s needed to make it available to patients should be better communicated to the public.

A Call for National Patient Registries

The idea of creating national, coordinated patient registries was supported by all the panelists, although there was some debate over the key details of implementing them. Detailed responses from the panelists are provided below.

Jackie Kosecoff: I am widely in favor of registries, and I am even more in favor of clinical trials, because we learn from these and they serve all of us. But if we’re going to spend the money to collect the registries, then we have to analyze the data so that we learn from the information we’re collecting. And so I don’t want to just see Congress promote collection of a registry; I would be even happier if physicians decided on their own to have a registry, and I would be happiest if we commit to using the information.

I was in Singapore a few years ago and it was right after the Competitive Effective Research budget was announced, but before health reform was passed. The Singapore Minister of Health stood up and said, “I want to thank the American government, because they’re going to do the work, but they won’t pay any attention to the results. We will.” And that really struck me. I want us to show the Minister of Health in Singapore that we can – and will – lead in not only collecting the data, but using them for the benefit of our own citizens.
Ross MacLean: Registries are a great idea. However, a registry shouldn’t be viewed only as an act of collecting data and analyzing the information, but should be used as an act to build collaboration among all the stakeholders. A registry is only as good as the questions which it’s setting out to answer. It is an opportunity to bring the patients, advocates and the people we have represented at this roundtable to begin to share the questions that they would like an answer to, to agree on the priority questions; we all have different questions, but what are the top five or ten priority questions? And then we must embed those in the registry approach. Otherwise, it will be less credible in the eyes of the entity that will generate answers three, four or five years later.

Lee Newcomer: I have been surprised about the resistance that does occur to registries. About three years ago, UnitedHealthcare asked physicians to voluntarily submit some clinical information about their cancer patients. All we asked for was the clinical stage and the gene test, if one was conducted. We combined that information with claims data and then ran all of that against the set of guidelines published by an external party called the National Cancer Center Network. It was very successful. We had 61 percent participation, and quickly accrued information on 14,000 patients. And then all of a sudden, people came out of the woodwork; the professional society wanted to own the registry and not share the information unless they decided the questions. The American College of Surgeons actually keeps cancer registry information and has done so for now almost 30 years, but won’t let it be released publicly, because they don’t want anyone but the hospital to understand who is doing good work and bad work, or to answer any further questions from the registry.

Unfortunately, there is still some fear about bringing things to light. To me, the value of a registry is it allows us to start answering questions. We may not like what we find there. We may discover that we’re not happy with the results, but I think that serves us all better than living in ignorance. So, I don’t want us to be naïve that creating a registry is going to keep people from still having some fear-based thinking about whatever problem we’re trying to solve. I still would push for it as hard as we can, because I think having that information only enlightens us. If we look at a registry as an opportunity to find out how we can be better, it is a wonderful thing.

P.J. Weiner: My organization has definitely encountered people with MS who are terrified of the idea of there being a registry, that somehow, their names are on this list of people with MS that’s “out there” – and the effects that can happen if someone discloses their disease to their employer. There are people with MS who have lived with the disease for 20 plus years and still have family members who don’t know that they were diagnosed. So there can be a lot of secrecy and fear around the diagnosis.

I think it’s the fear of the unknown. We have been trying to do as much education as we can possibly do. Our vice president of research programs at the MS Society, Dr. Nick LaRocca, has done a lot of education for our members about what the registry would be and that it would be a confidential source. We don’t have an official national registry at this point, but there is a New York State registry that has been set up by neurologists and another national registry as well, but only has certain states participate in it. It’s not able to collect a wide variety of data because of the optional setup of the structure.

That said, I do think that there is plenty of support. We don’t even know exactly how many people are living with MS. We use the figure of 400,000 people that’s based on studies that were done in the 1970s and have been
inflated over time. There are people who think there could be as many as a million people with MS. So it’s important, but there are definitely some reservations. I think that you’ll always have people who are worried about what the impact will be of anyone knowing about their diagnosis, and I don’t know if we’ll ever be able to assuage that small percentage of the population. I do think that the evidence is there that if we have this information it will help lead us to better treatments, and hopefully, eventually even to a cure.

Kenneth Bandler: One of the main reasons I’m supportive of establishing a registry is to really – once and for all – get an accurate number of how many people in this country have MS. I don’t believe it’s 400,000. Just based on anecdotal communications I have with people, I think it’s more. And if people are encouraged to sign up for the registry, then the scientists and the researchers can figure out where people are clustered and what their backgrounds are. It will help in research to develop other therapies.

There is an innate level of suspicion among us about processes requiring private data – that is our society. But, I think ongoing education among MS patients would encourage people about what the benefits are of signing up on the registry.

Nancy Berlinger: Jackie had mentioned a note about registries for off-label drugs. While I have not studied this in particular, it seems as though registries have the potential to support informed decision-making because they would clarify the status of off-label drugs relative to both approved therapies and to clinical trials. Without the data, it is hard for the patient to discern differences between an off-label drug and an approved therapy. Registries might be another safety check there for patients.
The panel discussion revealed many points of consensus regarding the key issues around specialty pharmaceuticals that need to be addressed. These included:

- The health care industry must bring patients and patient groups fully into the process of making decisions about treatments and costs.

- Knowledge about drug efficacy and safety – from manufacturer to patient – can help inform decisions and improve the quality of those decisions.

- The growth of specialty pharmaceuticals raises questions of value and the most appropriate allocation and use of resources.

- The costs of specialty pharmaceuticals, and who pays for them, can no longer be ignored.
The panel proposed a series of recommendations to address these issues of specialty pharmaceuticals including:

1. Convene more stakeholder forums such as the roundtable, and encourage stakeholders to overcome traditional barriers and trust each other.

2. Encourage health care providers to engage in data collection and data sharing (within appropriate privacy laws, rules and regulations).

3. Create and support national registries for patients with diseases requiring specialty pharmaceuticals. These registries will enable us as a society to develop a growing understanding of when and for whom these drugs are effective; what if any are the risks of adverse drug events; and what are the risk-versus-benefit tradeoffs.

4. Use and replicate successful models from other fields that convene insurers, leaders from prominent disease groups, and patient representatives to jointly review data and identify opportunities to make care more accessible, affordable and higher-quality.

5. Establish multimedia public information programs to help people learn about specific diseases and motivate deeper understanding of, and engagement in, their own treatment and health.