

PriceSpective is pleased to announce the continued growth of its expert staff, with the appointment of Walter Colasante and Sophie Murdoch in London. In addition, Dan Ross has joined the company's new San Diego office and will lead its business development and mergers and acquisition work.

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**PhRMA and the White House:
Risk Sharing in the Donut Hole**

Few doubt that the road to national health reform will occur in the absence of significant industry involvement, or at the very least, a display of ostensible support towards the White House commitment to overhaul how healthcare is financed and delivered in the US. However, it could be argued that President Obama's goal of achieving comprehensive health reform with bipartisan support and, perhaps more importantly, his stated goal of not adding to the ballooning national deficit, will necessitate a strategic, rather than simply cordial, relationship with industry.

In a move aimed to quell a longstanding criticism of Medicare's prescription drug coverage under Part D, the White House formally announced on June 22nd an agreement with the Pharmaceutical Research and Manufacturers of America (PhRMA) to narrow the so-called "donut hole," the gap in coverage requiring enrollees to pay the full cost of their drugs out-of-pocket until qualifying for catastrophic coverage (\$3,454 out of pocket while in the donut hole in calendar year 2009). While the summer's heated town hall debates may have captured much of the media's attention, this agreement could nevertheless play an integral role in health reform. The proposed 10-year deal, brokered by Senate Finance chairman Max Baucus, would

see the industry pledging \$80 billion to reduce patients' drug costs, and essentially would result in the largest-scope risk-sharing (or risk-shifting) agreement ever reached. Part of the industry's "contribution" would pay for up to half of the costs patients face while in the Part D coverage gap. Under the Baucus plan, contributions would also count towards patients' total out-of-pocket spend, allowing them to reach catastrophic coverage much quicker. Though details around the legislation remain ambiguously defined, Medicare patients with incomes up to \$80,000 will benefit from this agreement, presumably in some form of sliding scale manner, and a substantial portion of the savings under the program would go towards national health reform. Despite rumors that the White House has backed away from this deal, President Obama recently reaffirmed that the agreement would remain in place, ensuring that a meaningful, albeit small, source of funding is contributed to the seemingly certain overhaul of the nation's health system.

The proposed deal will have dramatic implications not only for the pharmaceutical industry, but also for the 26 million individuals enrolled in the Part D program, either through stand-alone prescription drug plans (PDPs) or through Medicare Advantage drug plans (MA-PDs). For 2009, the Centers for Medicare and Medicaid Services (CMS) allows Part D plan sponsors to place agents on a specialty tier if the drug's negotiated price exceeds \$600 per month, and the vast majority of beneficiaries are currently enrolled in such plans. Furthermore, most patients currently face a 33% coinsurance for agents on this specialty tier, reflecting a clear trend towards increasing patients' share of total drug spend by CMS.

A segmented view of Part D enrollees, however, reveals that many patients will not be impacted at all by the proposed plan, as roughly 12.5 million beneficiaries are eligible for low-income subsidies (LIS) by virtue of being dually eligible for Medicaid and Medicare, Medicare Savings Program recipients, or Supplemental Security Income (SSI) beneficiaries. While the level of governmental assistance varies, most LIS-eligible patients have nominal cost-sharing

requirements. Additionally, LIS patients account for a significant portion of high-cost biologics dispensed under Part D. For example, roughly 65% of Humira prescriptions under Part D are currently attributable to LIS patients.

For the Part D patient population that will be impacted by this agreement, the implications on access and drug utilization are likely to be significant. Approximately a quarter of this population (an eighth of all Part D beneficiaries) had spending high enough in 2007 to reach the donut hole coverage gap (requiring more than \$2,400 of drugs in the calendar year), while some 15% of those (~2% of all Part D beneficiaries) ultimately had total drug benefit spending high enough to reach catastrophic coverage. Ultimately, this coverage gap has limited access to drugs for a small but non-trivial percentage of patients, especially since many reach the coverage gap early enough in the benefit year to incur significant out-of-pocket costs. For example, among Part D enrollees taking diabetes medications who reached the coverage gap, roughly 10% completely stopped taking their medications and did not switch to an alternative treatment. Overall, it is estimated that 15% of Part D enrollees who fall into the donut hole subsequently stop taking their medications, while 5% switch to an alternative therapy in the class. Such changes in drug use not only involve cost-conscious patient “consumers,” but also involve physicians, who are increasingly cognizant of the out-of-pocket burden facing many of their patients. The current proposal, by significantly reducing the gap in coverage for resource-intensive patients and accelerating the speed at which catastrophic coverage will begin, is likely to lower patient price sensitivity and, by extension, may impact physician prescribing patterns. How and to what extent these dynamics will change should be closely monitored by the industry, and included in forward-looking market scenario analysis.

As competing interests continue to hash out healthcare reform in Congress, it is still possible that the government or industry will change their respective stances on the donut hole agreement. Senator Baucus, in fact, recently staved off efforts by fellow Democrat Senator Bill Nelson to introduce an amendment that would have required drug makers to provide \$106 billion in rebates over 10 years, an act that would have undermined the original agreement. It is also likely that the Part D program will be the target of other components of legislative proposals. For example, the controversial “non-interference” clause of the Medicare Modernization Act of 2003 prohibiting direct negotiations by the federal government with manufacturers will be a major point of contention in the months to come (and one with potentially huge ramifications for value-based pricing). The

Baucus agreement, whether or not ultimately incorporated into the future of this country’s health system, should nevertheless serve as a symbolic milestone of the ever-changing dynamics of our policy environment.

Building Value Around Regenerative Medicine

Over the last decade celebrities such as Michael J Fox and the late Christopher Reeve have helped draw attention to the enormous potential advances stem cell research can bring. The energy that these Hollywood stars and countless other advocates in both the US and Europe have put into raising awareness reflects the level of hope and promise that rests with regenerative medicines.

Recent political changes in the US (namely, the presidential order re-instating funding for stem cell research) and the approval of the first two clinical trials testing regenerative medicine have boosted the hope and expectations for stem cell research. As these products enter Phase I clinical trials, it will be imperative for companies to undertake the potentially ground-breaking task of accurately assessing their value and preparing effective communication to healthcare systems. Even new setbacks, such as the FDA’s recent hold on one of these trials, should not stop industry from the critical task of long-term, proactive value assessment.

The Basis for Hope

Cell replacement has enormous untapped potential to treat multiple diseases such as diabetes, spinal cord injuries, stroke, macular degeneration, and Parkinson’s disease with both clinical and economic implications. Stem cell research may also result in tissue engineered products with therapeutic application in heart disease and skin and cartilage replacement. For some, the ultimate goal is to use tissue engineering to replace failing organs. The WHO estimates that only 1 in 10 patients who need a kidney transplant – the organ most in demand – receives one. Given the breadth of diseases and the benefit that regenerative medicines may offer, it is easy to understand the proactive passion behind stem cell research.

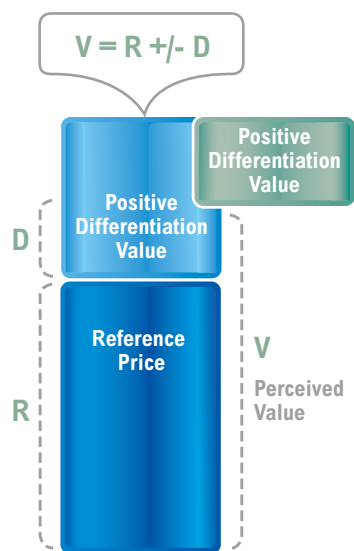
Moving Beyond Theory

Regenerative medicines have moved significantly beyond scientific speculation. Earlier this year, clinical trials were given the green light in both the US and the UK. In January 2009 the FDA granted permission for Geron Corp to initiate a Phase I multi-centre trial for its embryonic stem cell based therapy for spinal cord injuries (GRNOPC1). Meanwhile, in

the UK ReNeuron is moving forward with testing a stem cell therapy for stroke (ReN001).

The Geron trials have experienced a regulatory set back. On 17 August, the FDA put the trials on hold because of information that the mice in animal trials developed cysts. Geron shares fell by 10%. However, because these cysts are non-proliferative and not the type that develop into teratoma tumors, financial analysts believe that the hold will be temporary.

Clinical trial approval is only one of many hurdles regenerative medicine manufacturers will face. It will be vital for these companies—even those with setbacks like Geron—to be prepared for the pricing, reimbursement and market access hurdles that lie ahead. Companies will be faced with a challenge of identifying which products hold the greatest promise and comprehensively capturing the value of those products.



Conventional value-based assessment uses the equation $V = R \pm D$, where V is the value, R is the reference and D is the differential value that the product brings. As these products will be the first to market there will be no viable references available. Therefore, the value is based purely on the strength of the clinical outcomes. Indirect references may be used, e.g., the cost of transplants or other surgical interventions that can help to provide indicative guidance. However, the greatest challenge will be communicating the value to those who make decisions about what treatments are paid for by insurance or healthcare systems. Building a strong economic argument will help funding bodies realise the potential positive impact regenerative medicine therapies have on the burden of the disease and to reduce the total health care costs that are associated with debilitating diseases, e.g.,

long-term care, hospitalisation, morbidity etc. These types of costs are often overlooked or not fully understood during value assessments and, if not fully captured, can negatively impact the overall product value.

It is likely to take many years before successful outputs of stem cell research are realised, but the work on capturing the value of these products should begin today, to lay the foundation for the significant investments that have been made. The doors have now been opened to encourage global scientific advancement in the regenerative medicine area—making patients the greatest success story of the stem cell research debate.

Please visit PriceSpective's booth at ISPOR's 12th Annual European Conference from 25 to 27 October 2009 in Paris. PriceSpective is pleased to also be presenting three posters at the conference:

- **Applying Value-Based Pricing to Regenerative Medicine Based Pharmaceuticals**
- **Biosimilars: HGH to TNFs, How Will Payers Respond?**
- **Mind the Gap: Assessing the Different Data Requirements Between Regulatory Approval and Health Technology Assessment**

PriceSpective produces this quarterly bulletin to assess current issues with relevance to value strategy in the biopharmaceutical industry. We accept suggestions for coverage as well as articles from external parties. Should you be interested in participating in an upcoming PriceSpective Bulletin, or if you have questions or comments about current articles, please contact:

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