



Medical Innovation and Public Policy: At the Crossroads

U.S. Food & Drug Administration and the
Centers for Medicare & Medicaid Services Summit
December 11, 2012 • Washington, D.C.

Keynote Speech

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“**Medical innovation** is a crown jewel of America. It has contributed so greatly to the **economic success** of our country over the last 50 years and has made such a meaningful difference in the **quality** and **length of our lives.**”

– *Robert J. Hugin*



The following speech was presented by Robert J. Hugin, Chairman & Chief Executive Officer of Celgene Corporate at the FDA/CMS Summit in December 2012.

think it is important, certainly for me, and for all of us, to take the opportunity to step back and really think about the key issues that face us in the coming weeks and months.

For those of us who don't live and work in Washington, we are numbed by all of the information that comes out on a regular basis through the media, and the issues that we're facing – the fiscal cliff, the long-term issues of healthcare reform, and more. We get somewhat distracted by the news reports, and it gets portrayed as a political contest. It really undermines people's understanding of the profound, significant, long-term consequences to our economic future and our social future of America as to how these issues are going to be resolved. We are going to deal with them. I'm here today to really make sure that I do everything I can to implore you that we all work together to ensure that we preserve the culture of scientific and medical innovation in America.

Medical innovation is a crown jewel of America. It has contributed so greatly to the economic success of our country over the last 50 years¹ and has made such a meaningful difference in the quality and length of our lives.² It is paramount that we work together to ensure that policymakers all around the world, and especially here in the coming weeks and months in Washington, that we recognize that a positive policy environment is actually a critical part of the solution in dealing with these many challenging issues that we all face.

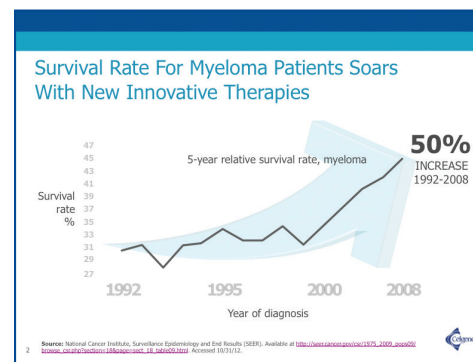
At Celgene, every day, we recognize the value and the benefit of establishing and operating

a science-based business in a country where a positive policy environment, that drives a culture of innovation, is valued. This is truly fundamental to the success of Celgene. The fact that we have risk-takers who recognize opportunities that make a difference in the lives of patients and work collaboratively with a strong and bold FDA recognizing the need for patients, that thalidomide could be brought back to the market, and through thoughtful planning and diligent execution of restricted-access management programs, ensure safe access, to the maximum extent possible, in the interest of public safety. The FDA collaboratively worked with Celgene to establish a risk-mitigation system that allowed access to THALOMID®³, but at the

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same time, preserved the public health and safety of society. Without that kind of collaborative, risk-taking spirit, our company would certainly not exist in the form that it exists today.

Additionally, if we didn't have the Medicare Modernization Act of 2003, and the opportunity for senior citizens to have access to oral prescription drugs, so many seniors today would lack access to life-saving cancer therapies and more. Our company would certainly be very different, and we would not be having the impact on patients' lives that we are having today.



At Celgene, we have witnessed a dramatic increase in five-year survival rates for patients with multiple myeloma.⁴ I have to tell you, the information that we share with you today so understates the economic and social impact of the changes in that one blood cancer, multiple myeloma, because it reflects the five-year survival rate up to 2008. In the last four years, from 2008 to 2012, we have seen nothing but an increased survival rate as a result of multiple innovative therapies that have been approved. Very, very positive impacts on this disease, starting with thalidomide, but now with multiple new novel therapies from Celgene and other companies. So the impact on patients, because of the existing policy environment, has been very clear.

As a result of these positive policy actions and a culture of innovation, we have

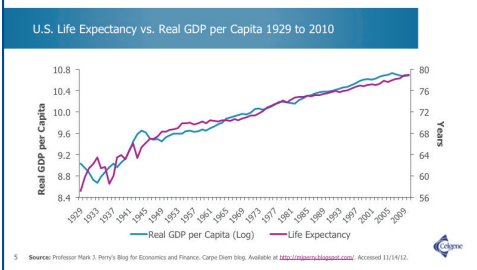
leveraged that success and reinvested it to ensure that we can capitalize on the breakthrough therapies that we now have. All of this has enormous impact on our economy, locally in New Jersey, and the other states where we operate in the United States. It also allows us to really capitalize on the revolution in molecular biology and information technology that is accelerating at an exponential rate today.



Based on all of these investments in R&D, today we have built one of the most promising innovative pipelines in the industry that we believe will have a sustained, long-term impact on the lives of patients in the United States and throughout the world. It is because of a positive policy environment that we will continue to make a difference, and have such a meaningful impact on patients' lives.

Celgene is but one small part of that ecosystem of innovation in America that has contributed to the economic growth in our society, and the quality and length of lives. There are so many other parts that have contributed so greatly to the dramatic extension of life in our country. Some of you may be thinking: "oh, the U.S. doesn't do that great as far as life expectancy as other

Increasing Life Expectancy Sustains Economic Growth



countries.” When you take out violent deaths, automobile deaths, things that have nothing to do with healthcare, our life expectancy is absolutely at the top of the entire world due in large part to the medical advances that the U.S. has delivered to the world over the last 30 and 40 years.⁵

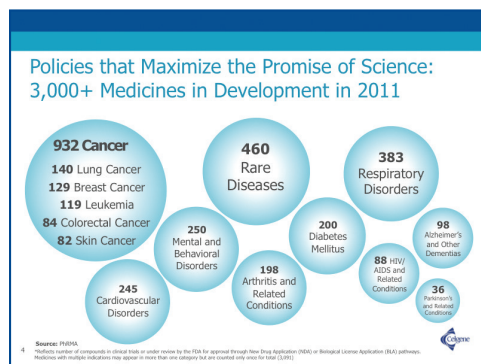
So few people today appreciate the improvements to the quality and length of life that medicines and devices and medical innovations⁶ have on our society. I think about my own college-aged children. They don’t have

We have built one of the most promising innovative pipelines in the industry that will have a sustained, long-term impact on patients.

any real concept of the AIDS epidemic. They don’t realize that there has been 90 percent reduction in death rates, for AIDS patients, as a result of medical innovation. They don’t understand that from 1999 to 2006, we had a 45 percent reduction in death due to heart

attacks and heart failure. Forty-five percent reduction because of approved medicines and medical intervention. People don’t understand that you’re four times more likely to be institutionalized with Alzheimer’s if you’re untreated – very important changes in our society that have an economic impact and social impact because of the innovation culture and the benefit of medicines.

Today, what is most exciting to me is that it is just the beginning. We are only at the early stages of being able to capitalize on the revolution in molecular biology and information technology. There was great fanfare more than a decade ago when the human genome was mapped. But the reality of that expectation of approximately 120,000 genes in the human genome turned out to be not much



different than a mouse, at about 30,000 genes.⁷ It just meant that the complication, the puzzle was more complex. We have restructured how we do research and drug discovery over the past decade, creating a greater understanding of the proteome and the importance of downstream intracellular signaling. The promise is incredible. Exceptional by any measure. Today, we have more than 3,000 new compounds in development;

a thousand compounds looking to change cancer from a terminal, incurable disease to one that becomes a chronic disease. We are making great progress. In the next ten years, we’re going to see multiple cancers turn into manageable diseases, from terminal diseases today. Importantly, there are approximately 100 new Alzheimer’s drugs that are under significant development today.⁸

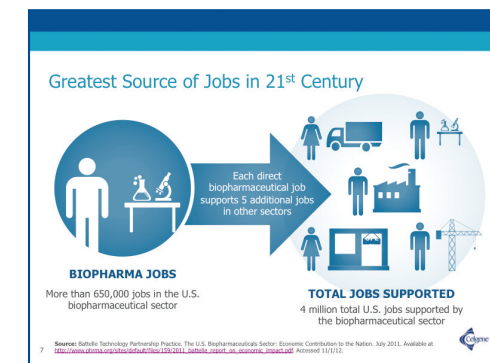


We are making great progress – and the promise of the future of the next decade is incredibly bright. If we are successful in just a few of these areas – and we will be – we will have an increasingly positive impact on economic benefit to our society, important quality of life and length of life. Just turning one or two cancers into manageable diseases has tremendous benefits.

Think about what the economic impact would be if we could delay the onset of Alzheimer’s by five years. It is projected over the next 20-25 years – if we change the course of this disease – we can have a \$100 billion reduction in healthcare expenditures on an annual basis, based on the projections of what is going to happen to Alzheimer’s disease prevalence over the next 20 years.

I think all of us, including policymakers, now understand more about the importance of societal benefits and importance of scientific and medical innovation to our economy. Medical innovation is a critical part of the growth engine. It is one of the few manufacturing sectors that is projected to increase the number of jobs over the next 10 years.

I want to give you one example that really highlights the power of innovation on an economic perspective. The mapping of the human genome was about a 13-year project, and it was about a \$3.8 billion governmental investment. In 2010 alone, the United States government collected \$3.7 billion in federal taxes related to economic activity, only related to human genome-specific activity. A tremendous multiplier effect of when you advance the science, and then you are able to extrapolate.

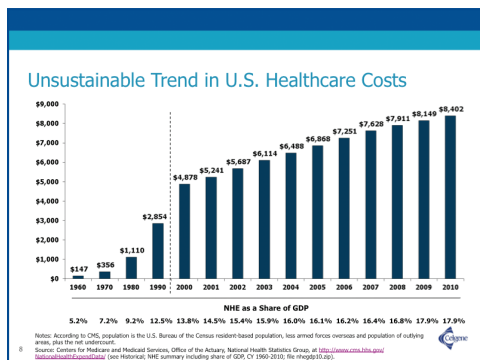


It’s not just the quality of the jobs, but the impact it has on local communities and on the academic environments. We have to recognize that this is potentially at risk. Emerging economies recognize the value to the development of their societies’ economies, and to the quality of life of their people. These countries are looking to incentivize and to draw, what is today, one of the crown

jewels of the American economy – our life sciences sector – away from the United States. If we do not maintain forward-looking policies to encourage risk-taking, to encourage medical innovation, our life sciences sector will not only be drawn away by emerging economies, it will be forced away and driven to other markets. It is an important crown jewel that we ensure that we fight to retain in our country.

Biopharmaceuticals is one of the few manufacturing sectors that is projected to increase the number of jobs over the next 10 years.

We do have significant challenges, and certainly you can understand the magnitude of the fiscal cliff issue that affects all of us in so many different ways. We have to make sure that the policy implications of these decisions do not change that entrepreneurial and innovation-driven culture that will be part of the solution.



It is not just the near-term fiscal issues that we have to deal with. We have an unsustainable expenditure, growing expenditure, in healthcare

in America. There is no question that we need to reform healthcare in America. We have begun healthcare reform, and there are a lot of positive events every day, in local hospitals, and in local doctors' communities, and companies around the world, around the country, and in government agencies to stimulate changes to improve quality and address rising healthcare costs. It is not a question of if we have to – we have to. We must do it even faster. We have to accelerate the change. We have to work on payment delivery changes in the healthcare system. We have to ensure that we continue to spur innovation with accountable care organizations focused on quality and outcomes. We must demand best practices to ensure that hospitals and practitioners provide services to patients in a cost-effective, value-producing manner that meets today's standards. Equally important, we must establish easy-to-access data-based systems that make hospital and practitioner performance data more transparent. Together, we can make better decisions on how to do that.

Pharmaceuticals are not the root cause of the problem. Branded pharmaceuticals make up only about nine percent of healthcare expenditures.⁹ And in fact, in 2010, branded pharmaceutical expenditures in the United States were less than branded pharmaceutical expenditures in 2009. The growth rate has not been significant. In fact, each of the last two years, the Congressional Budget Office has reduced its ten-year forecast for prescription drug costs by over \$100 billion. I believe many of us saw a week ago, for the first time, the Congressional Budget Office is now, in its future projections, going to actually score the benefit of cost savings of increased prescription drug usage on other medical services within the Medicare framework – another positive part of the solution.

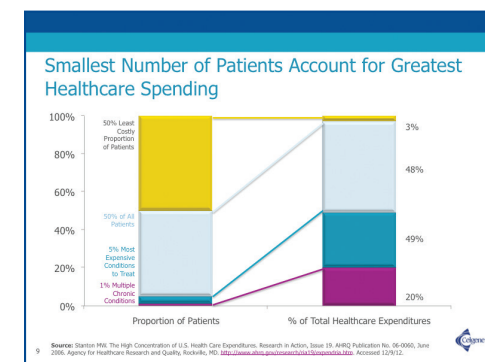
It doesn't mean that we don't have to focus on value for patients when looking at the cost of healthcare. We all have to be part of the solution in dealing with the healthcare crisis. Otherwise, we will not have a sustained and strong economy in America. I think we have a very focused approach. The changes that we make will not be revolutionary, but we will live with a changing healthcare system for the next 30 years. That is the reality we will face every day.

Branded pharmaceuticals make up only 9% of healthcare expenditures.

The situation is not going to get any easier with the changing demographics of an aging population in the United States; it is forecast that in the next 20 to 25 years, we will witness a doubling of the population over the age of 65 in America.¹⁰ Unfortunately, elderly people tend to consume more healthcare in America, at higher rates, than younger people do.

That is all the more incentive to accelerate and take revolutionary steps to change the way we develop anti-cancer drugs, and the way we address neurodegenerative diseases. We must accelerate the promise of those drugs, because it's absolutely an imperative.

Additionally, if we look at the challenges that we have in healthcare in America today, it is a very concentrated problem. One percent of the population consumes 20 percent of the healthcare expenditures. Five percent of the population consumes almost 50 percent of the healthcare expenditures.¹¹ Chronic disease



represents nearly three-quarters of our healthcare expenditures. We have serious issues that we have to deal with – issues of obesity, smoking, the lack of preventative care and access to preventative care. These are tough societal decisions, but the problems are concentrated. If we can attack the root cause of the problem represented by a small part of the population that consumes such an incredibly high percentage of health care expenditures, then the opportunity for meaningful change in bending the healthcare curve is there for us. We must direct our medical innovation and creative thinking to

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the areas where the problems exist. There are great solutions to these challenges and they will be available to us.

Clearly, when we think about the future, we need a risk-taking culture to be sustained and enhanced. It is people who are willing to take risks and challenge the conventional wisdom to find new solutions that will be the problem-solvers of the future. Medical innovators cannot assume enormous risk if there is no intellectual property protection that enables them to benefit from the accomplishments and achievements that they develop. We have to support a pro-innovation regulatory environment, as with the positive steps with the implementation of FDASIA. We have to ensure that our society has access to the revolutionary and positive changes that we foresee over the coming decade. What a tragedy it would be, knowing the enormous potential for medical progress that we can deliver to all elements of our society if access to those innovations were denied.

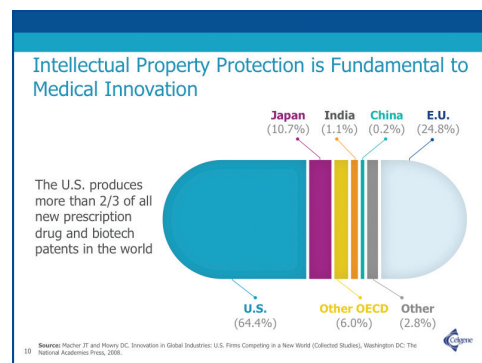
Intellectual property is really an interesting issue to look at because it is sort of a misunderstood and arcane issue for a lot of people. Intellectual property is fundamental to our economic growth and our prosperity.

We must support a pro-innovation regulatory environment and ensure that our society has access to the revolutionary medical progress that we can deliver.

It is important where intellectual property is created and resides. It is where the economic benefit of intellectual property and innovation exists, and that is ultimately where the tax receipts go, where the intellectual property is

owned, and therefore the economic benefit and the tax benefit are also there.

Thirty years ago, Europe produced more than 50 percent of the intellectual property around new medical compounds. Thirty years later, with policies that do not support medical



innovation in Europe, the policy environment has pressured their life-science industry, in terms of research and development, where today, more than 2/3 of the global medical innovation, the intellectual property around new chemical entities, is produced here in the United States. Europe now represents less than 25 percent.¹² That said, this is not a given. If we do not sustain an environment of pro-innovation policies and laws, medical innovation will leave America as sure as it left Europe. It will go to emerging markets in other places, along with progress and prosperity. They will have the full economic benefit of that intellectual property and the ownership. So, albeit arcane, it is critical that we all understand the value of intellectual property and the broader importance that immediate access to novel therapies has on economic and societal benefit.

I mentioned earlier about the positive implementations of FDASIA. This is a great example of great progress in terms of having bipartisan support ensuring that our regulatory agencies in the United States and around the world embrace 21st century regulatory science, that we work together to find new ways of doing things. I think as we implement FDASIA, if we do, we ensure that it becomes best practices, and is implemented as part of the solution of ensuring access, more rapidly, to new innovative therapies to patients in the United States.

We have to continue the acceleration of the changes that we are having in the healthcare system. Already, we are talking about some

As we implement FDASIA, we must ensure that it becomes a best practice, and is implemented as part of the solution of ensuring access to new innovative therapies.

of the innovation of accountable care organizations, and looking at implementing bundled payments in ways to ensure that value propositions are recognized. I was really pleased by the introductory comments about the biopharmaceutical industry in general. I do think it has changed. We recognize there is no good business proposition if your therapeutic solutions do not have a value proposition for patients and payers. The 1990s or the 1980s of low-hanging fruit and me-too products is yesterday's story. There is no good business model that does not focus on value-creation, meeting significant unmet

medical needs, and that is fundamental to all of healthcare. We must accelerate those changes. The biopharmaceutical industry is ready. That is part of the future; it is part of the present, the reality that has existed because of very tough economic conditions all around the world. It is promising in terms of the changes that we will see, increasing and accelerating over the next 10 years.

Medicare Part D is a Model for Patient Access Through Competition and Choice

- Cost is 43 percent below initial projections
- Premiums still average \$30 month
- Over 30 million seniors have coverage through Part D
- Satisfaction rate is 88 percent
- Part D is reducing cost of other Medicare services

Source: PRIMA, CBO. Source: PRIMA analysis of data from CMS and HHS. Available at <http://prima.org/sites/default/files/1516-prima-governmental-claim-2012-2013-final.pdf>. Accessed 12/10/12. Prescription Drugs and Medicare Part D: A Report on Access, Satisfaction, and Cost. AARP. Available at <http://assets.aarp.org/presscenter/health/medicare.pdf>. November 2007. Accessed on 12/10/12. Spreading the Word on Medicare Part D. PRIMA. Available at <http://content.prima.org/wordspreading-the-word-on-medicare-part-d/>. Accessed on 12/10/12.

Medicare Part D, I really do believe, is a good example of positive public policy. Great access to medical innovation for senior citizens. It's hard to believe that it was only six years ago that senior citizens under Medicare did not have access to oral prescription drug coverage. I find it offensive for anyone who does not believe that Medicare Part D represents great public policy and is an important social advance for America. You think about what Part D has done. Even in the first year, we saw a \$14 billion reduction in other medical services expenditures with just the introduction of Medicare Part D – and costs 43 percent below budget forecasts. Even when the bill was being negotiated, there were naysayers that wanted to put a cap on the amount of premiums that a senior

citizen could pay. The premiums have never hit the proposed cap and continue to average \$30 per month. Today, senior citizens enjoy broad access to important oral prescription therapies as a result of competition and choice. This was a government paid-for, privately executed program with almost 90 percent approval ratings by the users of the program, and significantly lower cost than ever expected, with positive offsets on other parts of the medical services system.

Today, despite all the wonderful things that have happened and the great changes in the promise of the future, we have tremendous challenges, and we have to live in the real world. The challenges we face in Europe from a financial perspective are many; the compulsory licensing challenges in India; and the lack of respect of intellectual property in other markets, and more. Over the course of the past five years, and as a result of tremendous challenges and tough economic times, important benefits have emerged.



One is a real tangible feeling and recognition that collaboration is central and fundamental to success for all parts of the healthcare ecosystem. No longer can the NIH be successful, or life science companies be successful, or

academic medical research be successful, without having a collaborative relationship with other parts of the healthcare ecosystem.

Collaboration is central and fundamental to success for all parts of the healthcare ecosystem.

It requires integrated solutions, pre-competitive research collaborations between academia, the government, small and large companies, small and large companies working together, governments and companies working together for solutions. It is a reality today that we will not capture the promise in the potential of medical innovation for the future, and we will not provide the solutions for the financial problems we have, if we don't have integrated collaborative solutions. Every day, I am more encouraged and optimistic about that realization, that the adversarial nature is really the wrong approach. We have to have competition. We want competition in markets and market-based solutions, but among components of the ecosystem of healthcare, we have to collaborate together and provide integrated solutions.

It is an incredibly challenging time. It is in fact a perilous time for many of us with the challenges we have with the technological risk, the challenge of science, the tough regulatory environments, and the economic environments we face. It is a perilous time. But I assure you, it is not a time for us to shy away or step back from bold and courageous changes and embracing the future. It is not a time for retrenchment in rationing. That is not the solution. We need to step forward with a

bold agenda for the future and set aspirational goals for the future. Like Celgene, we have to believe that in the next 10 or 15 years we will change the face of cancer, curing some and turning others into manageable diseases. We have to have bold goals. We have no chance for success if we don't set aspirational objectives.

We need to take action. We need to revolutionize the way we do drug discovery. We cannot afford new drug development costs of \$1.4 billion, or taking 12 to 13 years to go from concept to approval. It is not sustainable. This is a fundamental reason for the decline in research and development productivity. You have seen the radical change in drug discovery technologies over the last decade. The recognition of that revolutionary change and the way we do clinical trials, the way we look at translational medicine and its impact on biomarkers and regulatory science, are just signs of the positive changes that are coming. We have to accelerate that innovation and challenge the conventional wisdom in the way we do

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things. It's absolutely imperative. For those of us who believe that medical innovation in a culture of change and science and medicine will be part of the solution, we have to stand up and advocate for public policies and laws that will support a positive environment and positive solutions to the challenges we face.

Certainly here in the United States, as we address the issues of the fiscal cliff and of healthcare reform, and all around the world, we have to believe in a positive future, and be bold and courageous as we tackle it.

Thank you.

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