HEALTH RESEARCH AT A CROSSROADS –

ARE PUBLIC-PRIVATE PARTNERSHIPS THE WAY FORWARD?

EUROPEAN PARLIAMENT, BRUSSELS – 13 NOVEMBER 2012
ROOM PHS P7C050

17:00 – 20:00

Dear members of the European parliament,

Dear guests,

Dear friends

I am delighted to be with you both as chairman of IMI and as CEO of UCB (nr1 company R&D spend per employee).

I would like to **thank madame amalia sartori**[1] for hosting this event at the European parliament.

I know that in your role of chairwoman of the industry, research and energy committee, the innovation and competitiveness of Europe is close to your heart.

And innovation is precisely the point of my talk.

Never has science given us so many opportunities to innovate and provide better health to European citizens and to citizens around the world. Yet, science is now so complex that no one can harness its potential on its own.

Just 11 years ago, the genome was discovered.

6 years ago, Shinya Yamanaka reprogrammed a mature skin cell to become a stem cell. 3 to 5 years ago, we focused on epigenetics.

Just a few weeks ago the encode project gave us 40,000 new switches to figure out around the genome.

It is only through open innovation, aligning our strengths, our technologies, including it, that we can transform this amazing science into medicines and other solutions for people living with severe diseases.

[1] Italian, Group of the European People's Party (Christian Democrats)

There are four points that i would like to discuss tonight:

- The importance of healthcare innovation in Europe
- What are the challenges around innovation in healthcare, especially in Europe
- How has IMI become a role model for open innovation
- Why public-private partnership should continue, under horizon 2020

1. **Let me start** with the importance of healthcare innovation in Europe.

   Can Europe get out of the current economic crisis without innovation? Has any region in the world been competitive over the long term without innovating?

   Innovation and the related entrepreneurship made the united states who they are. And china is on its way to follow a similar path. China is already the second country in the world in terms of patents and scientific publications.

   By 2025, china will have over 400 million people over 65. And china does not intend to be just an importer of medicines to treat its aging population. They intend to compete with a robust industry of their own in biopharma.

   In Europe, aging of the population is already an issue. As the CEO of UCB but also as someone who is getting older, i think more and more about aging as an opportunity.

   Europe has built its world competitive place based on innovation whether in aerospace, in cars or in pharmaceuticals. In which innovative industry does Europe want to lead? Cars or healthcare? Which one is the most important? Which one should be most cherished and stimulated?

   We all agree here on the importance of innovation for the future of Europe, as we all agree that the biopharmaceutical and health industry is one of the most strategic industries in Europe to cope with the aging of citizens in Europe, the us and in Asia. Anybody disagree?

2. As we all agree on the importance of healthcare innovations, let me move to my **second point** and reflect on the challenges of the innovation in health care.

   2.1. First challenge: the <u>complexity</u> of the new science. The biopharmaceutical industry in Europe and throughout the world has to completely rethink its approach to discovery of new medicines. There is a major paradigm shift ongoing with new leaders emerging and i work hard so that mid-size EU companies such as mine become one of these new leaders. The old model of target discovery and testing chemical leads in cell and animal models is just not working anymore. Over 90% of drugs fail in clinical development and even after poc, chances are not better than flipping a coin. The industry is investing a record 70 billion euro a year in R&D with declining level of new drugs approved over the last 10 years, now around 20. 70 billion euro divided by 20 new medicines is not a great outcome. The old days of each one competing for disease targets on their own are over. The old model gave results but modern medicine has delivered remarkable results over the past decades.

In the united states death rates for cardiovascular disease fell a dramatic 28% between 1997 and 2007. Similarly, heart failure and heart attack death rates following hospital discharge fell by half between
1999 and 2005

For people diagnosed with cancer between 1975 and 1979, the five-year survival rate was 49%. For those diagnosed in 2003 (the most recent year for which five-year survival rates are available), it was 67%.

The biggest improvements have been seen in developing countries.
Between 2000 and 2006, immunization campaigns cut the number of deaths caused by measles by 68 percent worldwide and 91 percent in Africa.

Between 2000 and 2009, infant mortality fell from 77 deaths per 1,000 births to 62 – a reduction of 20 percent.

If we take the entire world into account, a child born in 1955 had an average life expectancy at birth of only 48 years. In 2000, a child could expect to live 66 years. By 2025, life expectancy is predicted to rise to 73 years. That's an increase of more than 50 percent in less than a century – unprecedented in human history. And developing countries are seeing the most rapid gains.

And the pharmaceutical industry keeps adding new innovative medicines every year. In 2011 we saw two new breakthrough medicines against hepatitis c - the first in a decade. We also got two new personalized medicines against melanoma, for which we had no effective treatment before. And recently, just a few weeks ago, the European commission approved the first gene therapy product for a very rare disease.

The old new models exist, based on genetic understanding or exquisite disease biology understanding. These new models require shared technology and collaboration. No one can do it on their own.

2.2 the second biggest challenge is the disconnect in Europe between innovation and access to innovation. Governments want European companies to innovate but who wants to pay for innovation? The current trends are extremely concerning and need to be addressed urgently if we want an innovative biopharmaceutical European industry. It is understandable that the governments of Greece, Portugal and others, must take measures to achieve sustainable budgets. But these measures should target off patent medicines which provide significant savings opportunities in these markets. They should not target innovative medicine.

Of even greater concern are the new German reimbursement systems and iqwig which is impeding access for German citizens to innovative medicines, including some discovered in Germany. I understand this is not the topic of today, but all the progress in open innovation can be wiped out if the issue of access of innovation for European citizens is not addressed in parallel. European governments have to stop looking at the cost of medicines and focus on the value for their patients and citizens.

2.3 the third big challenge is on the education front.

Innovation cannot happen without strong universities and we should be concerned about the European universities dropping in the world ranking. We need more science students and science excellence from universities to enterprises. What do we think the effect will be 20 years from now on the fact that biology is the third most common university degree in china?

To overcome these challenges and tap into our innovation potential, the European union has many strengths. Our intellectual leadership and the richness of our diversity of thinking are unique assets. Is it just coincidence that of the 10 biggest pharma companies in the world, 9 have a head of research of European origin and the 10th is of asian background.

And now, one of our strengths in Europe is IMI.

Let me transition to my **third topic** and how IMI has become a role model for open innovation?

- The challenges that we are addressing today cannot be resolved by one company or one industry alone. The biopharma industry has opened, and most companies have adopted an open innovation approach. Less is done in house, more in open collaboration set up with other biopharma (in both precompetitive and competitive space), with it companies and with academia. More than that, the collaboration needs are extending to both regulators and payers so as to create a common understanding of what new sciences, 3not just life sciences"? Can deliver to improve health of citizens.
- There is a lot of discussion on open innovation and for the last 5 years we have been piloting: open innovation in a large scale platform, the innovative medicines initiative.
- IMI, or the innovative medicine initiative, may be a role model for open innovation through large public private collaborations.

  In fact, IMI is now the largest public-private partnership in healthcare around the world. It may be, together with IP, the most important initiative for EFPIA and a key one for the European commission. IMI was the first of its kind and its scale, budget and pace of progress continues to be unprecedented and raises interest across the world from the us, japan and china – Europe can be proud of this achievement.
- Thanks to the catalyst role of the commission, academic researchers, researchers in enterprises, regulators, patients and other key stakeholders cooperate to solve the bottleneck of discovery of new medicines.

Here is how the open innovation works and IMI:

- Competitors together identify and address common challenges, with input and stimulus from academia and regulators
- Imi then seeks a real collaboration with public partners and just outsourcing – the best potential and intellectual input throughout Europe is combined
- The neutral platform, and honest brokerage secured by an autonomous office makes it possible for all partners including public authorities to work together without conflict of interest problems.
- Intellectual property rules make it possible to protect, where necessary, assets of companies, smes, and academia. This is essential, as the pre-competitive character of research is fluid – what is precompetitive for a big company, may be competitive for an sme or for a potential spin off initiative.

IMI also allows the dialogue and alignment of objectives between fundamental and applied research, healthcare decision makers, regulators and patients.

And IMI delivers results

IMI results are being achieved much faster than any other funding scheme, with direct application in the innovation cycle and therefore also direct research and economic impact.

37 IMI projects have been launched to date and all 37 address the following issues:

- Knowledge fragmentation by pooling data, samples and knowhow from the different participants.
- Understanding complex diseases by gaining mechanistic knowledge, phenotyping and stratifying patients as well as validating *in vitro*, *in vivo* and *in silico* models.
- Development of predictive tools for efficacy and safety in alignment with regulatory requirements, improving clinical designs, training scientists and actively involving patients.

There is now a constant review of on-going projects and 5 key outputs have been identified as having a significant impact on pharmaceutical discovery:

- **1st key output: establishment of robust validated models for drug development and elimination of inefficient pre-clinical models.**

  Existing and novel animal models are being comprehensively evaluated to identify the most efficient ones. This will reduce the number of animals needed in pre-clinical studies, lower the cost and time required for drug development, better validated animal models will increase the chance of development of safer and more effective medicines for patients. First impressive results were achieved for example in the pharmacog consortium which addresses alzheimer disease (ad), the European consortium looking at chronic pain mechanisms, and imidia addressing understanding of diabetic disease

- **2nd key output: more effective approaches to predict adverse drug effects and prevent late attrition.**

  Clinical safety concerns and toxicological findings at late stage development represent about 32% of the most common factors responsible for drug discovery project failure. More reliable and robust methods for early prediction and detection of

adverse drug effects are developed and discussed with regulators. Examples of such new tools from IMI include an *in silico* model for predicting cardiac toxicity developed by the e-tox consortium. The safe-t consortium, in close collaboration with regulatory agencies, has made significant progress towards qualifying new translational safety biomarkers for diagnosis and monitoring of drug-induced injury of the liver, kidney and vascular system .

- **3rd key output: pooling and exploitation of existing data from various sources for novel analysis.**

  Pharmaceutical companies hold massive amounts of legacy clinical trial data from programs that have been stopped for reasons of safety or efficacy. This data can be utilized to help identify safety signal or help understand disease processes better. IMI projects provide a platform allowing the data to be pooled and enabling novel and more powerful analyses. First results in NewMeds resulted in a proposal for reduction in the length and size of schizophrenia clinical trials. When accepted by regulators this could have a profound effect on future clinical trials in this area.

- **4th key output: joint development and regulatory submission of key standards for drug development**

  Multiple IMI projects have invested significant efforts to harmonize procedures and generate international standards to implement best practices across the industry. This will help ensure that the data submitted for regulatory approval is more appropriate to address the regulatory concerns. The ubiopred consortium has established a set of diagnostic criteria on severe asthma providing a stepwise algorithm for diagnosing the disease. The summit consortium has developed non-invasive methods of measuring diabetic macroangiopathy.

- **5th key output: more efficient patient enrolment in clinical trials.** One of the ways of speeding up drug development is to make clinical trial enrolment more efficient and include better characterized patients. By facilitating the creation of clinical investigator networks (nd4bb), involving patients in clinical trial design and beyond (ubiopred, proactive, eupati), and by identification of clinical sites through electronic health records (ehr4cr) the projects highlighted here are addressing this crucial need.

Beyond these early wins and achievements, the most important and long lasting achievement of IMI may be building bridges, so many bridges between academia and industry and destroying so many walls. I was delighted to see many critics of the early days becoming active participants in many IMI initiatives, both in well-known academic centers and in big pharma. This newly created momentum cannot stop. It is estimated that 1500 jobs have already been created in the last 3 years.

And this is my last point. Horizon 2020 has to take the relay of IMI and move it to the next level. IMI exists for only 5 years / and 5 years is nothing in our world of science and discovery. I mentioned the Nobel prize on stem cell. It took 34 years between Sir John Gordon's experiment in oxford to reset the biological clock of a cell and Shinya's publication. In my company, UCB, our scientists were the first ones in the world to bring an anti-TNF antibody to the clinic, back in the early 90s. We just got approval for our unique differentiated anti-TNF four years ago.

IMI has to continue. Horizon 2020, the new public-private partnership, has to be created and build on IMI's achievements and lessons learned.

For horizon 2020, we need to set big airy goals. I like the challenge of creating a virtual human brain. We need bold goals on neuro- degenerative diseases, on cancer, on immunology disorders and on metabolic disorders. All of which are rising rapidly in europe and people who suffer are waiting. We are aiming at redefining lupus and Parkinson's diseases to discover medicines adapted to homogeneous subpopulations. We need also to simplify the rules and the bureaucracy to ease the collaboration for academics and for the industry. And we need to continue to pay special attention to sme. It was encouraging that in the last IMI call, 60% of the funding from industry was from smes.

With IMI, Europe has taken the lead, engaging all our brain power across academia and industry, to harness the complexity of new sciences and conquer the rising severe diseases, to make aging European citizens live longer and healthier and to export these future new medicines and solutions to Asia and Americas.

In the us, NIH cites IMI as an example. US FDA and Japan PMDA engage with IMI. IMI is a role model throughout the world.

It is therefore of utmost importance to maintain adequate level of funding for research and in particular health research in horizon 2020. It has been unequivocally demonstrated that "health is wealth". More and better health will only be delivered through more investments in research. The type of research that is needed requires many actors to work together in a concerted way.

The rumors about planned 50% cut of the horizon 2020 budget from 80 to 40 bn are therefore very worrying.

An ambitious and long term program like horizon 2020, free of political short-termism is essential if Europe wants to effectively address health challenges ahead of us.

Let us build on this amazing momentum and align all stakeholders to make horizon 2020 a reality, and to ensure access to innovation for all European citizens living with severe diseases.

I thank you for sharing this passion!

November 13, 2012