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Tony Alvarez, Chairman of the Japan-Based Executive Committee of PhRMA

Opening Statement (as prepared for delivery)

Good afternoon. My name is Tony Alvarez, and I am the President of MSD Japan and the Chairman of the Japan-Based Executive Committee of PhRMA. Thank you for joining me this afternoon.

As I look at 2015, I see this year as a key turning point for the pharmaceutical industry in Japan in terms of enhancing our contributions to economic growth improvement of health in the nation. The overarching theme I see is promotion of innovation thru economic, science and R&D policies. To understand why 2015 will be the key year for innovation in the pharmaceutical sector in Japan, let's look at some of the major developments in 2014.

In almost every meeting that Ian Read, the global Chairman of PhRMA, had in November last year with ministers, senior Diet members, and other stakeholders, there was a clear consensus that Japan needs to make significant changes in its policies to promote innovation in the pharmaceutical and healthcare area. This was expressed not as just one need among many, but as one of the top priorities of government to ensure a continued high level of healthcare and to stimulate economic growth.

First, the Japan Agency for Medical Research and Development, AMED, the so-called Japan NIH, was created by legislation and will begin formal operation in April under the leadership of Dr. Makoto Suematsu. For the first time, there will be a centralized and strategic approach taken by the government in its funding for R&D in healthcare. This includes a commitment to promote translational science to move discoveries from the laboratory thru the development process and into the market.

Second, the improvements in PMDA's performance continue to be nothing short of remarkable. Standard review time for drug approval has been reduced from 23.2 months to 11 months and priority reviews are now down to 8.5 months. And the lag of NDA submissions has been shortened from 30 months to 17 months, with industry expecting that it will be further reduced to four months within the next few years. PMDA has initiated programs to reach out to universities to help them enter the drug development process and to smaller drug companies to help guide them thru the difficult review process. The professionalism of the staff at PMDA improves every year and

PMDA will continue to expand its review and safety staff numbers, an important effort to deal with the many new and more complex drugs under development now. PMDA has become more open to accepting foreign data and is a leader in the commitment to support multiregional clinical trials. Japan will host the 2015 meeting of ICH, the International Conference on Harmonization, and this will generate new initiatives from PMDA and MHLW on drug development policies and global harmonization.

Third, the impact of the drug price premium rule initiated in 2010 has been significant. In a PhRMA survey a year and a half ago about its impact on the American, Japanese, and European pharmaceutical companies, we saw that it had already led our members to bring their pipelines to Japan more quickly and to increase their drug development staff. As a result, the number of drugs developed in Japan has increased from 231 drugs in 2010 to an expected 395 drugs in 2017. There has been a significant decrease in the submission lag, and the expectation is that lag will continue to diminish. Japan has become part of companies' global development strategies, rather than Japan development occurring years after development elsewhere.

We are currently doing a survey to update those findings. I expect it to show even more significant changes. We will present those findings to Chuikyo later this year as they debate policies on drug pricing, but the conclusion is clear – the drug price premium has led to an increase in new innovative drugs becoming available to Japanese patients promptly.

Fourth, passage of the new Pharmaceuticals and Medical Devices Law included measures that will speed up the development of regenerative medicine, building upon the discoveries of Nobel Prize winner Dr. Yamanaka. This will contribute to new cures and new businesses.

Fifth, MHLW is broadening the definition of orphan drugs to include many other intractable diseases. Our experience in the United States is that a change in the scope of orphan drugs can have a dramatic impact on public health and on investment in R&D. Many think that orphan drugs just relate to small populations. But when you look at the broad societal impact, you reach a different conclusion. For example, there are 7,000 orphan diseases in the United States affecting ten percent of the population – or 30 million people. Changes in US laws and rules have resulted in hundreds of orphan drugs being developed, approved, and available to patients. The opportunities in Japan with these regulatory changes are enormous.

Sixth, the government's Health and Medical Strategy includes goals and commitments in a number of areas of drug development, including commitments to improve translational research.

I was educated as a pharmacist and have spent over thirty years in the pharmaceutical industry, including the past nine years in Japan, and I have never seen the opportunities that I see in Japan this year and into the future. But, of course, progress and success depends on the right mix of public policy, support and encouragement of the industry, involvement of all stakeholders in decision-making, and cooperation among all those stakeholders.

The benefits of innovation in the pharmaceutical area are of incredible importance to everyone. This included to improvement in the quality of life of all citizens, prevention of debilitating disease, amelioration of the effects of disease, an easier aging where we can lead a healthy life for a longer period allowing us to be part of a productive workforce, and contributing to the growth of the nation's economy.

PhRMA has produced several studies that we have discussed with you before that demonstrate conclusively the contributions of innovation in the pharmaceutical sector to society.

One study looked at five of the 176 drugs approved over a five year period and demonstrated that the economic value of those five drugs was 1.3-1.5 trillion yen, divided into 800-950 billion yen in saved medical costs and 500-550 billion yen in productivity gains for the society.

Another study analyzed drugs approved in 12 disease areas over a six year period, looking at how patients and healthcare providers viewed those drugs and how important they were in helping to save and improve lives.

These studies demonstrate clearly how important innovation is to a healthy society and to economic growth.

Last year, together with EFPIA, our sister association representing the European research-based pharmaceutical companies, we issued a joint report on the state of clinical research in Japan. This report, entitled "Research in Your Backyard", looked at

clinical development in all 47 prefectures in 16 disease areas, including cancer, allergy, cardiology, gastroenterology, and rare diseases. Our companies conducted a total of 682 clinical trials in 11,653 sites throughout the country. Total clinical-related spending by PhRMA and EFPIA companies in Japan was 88 billion yen, while total clinical-related spending by all pharmaceutical companies in Japan was 247 billion yen.

These trials benefit patients and the nation in various ways.

First, medical facilities conducting clinical trials are able to provide advanced medical treatments to their patients, improving their quality of life. .

Second, spending on these clinical trials goes to hospitals and clinics nationwide and supports the economy in every prefecture.

Third, the clinical trials create jobs throughout Japan as local medical facilities, pharmaceutical companies, SMOs or site management organizations and CROs or clinical research organizations employ local staff to support the trials. And, importantly, most of those jobs are high skilled, high paid, high value-added jobs.
These clinical trials represent the intellectual base of pharmaceutical industry and are at the core of what we do to bring benefits to patients and to the economy. The value added of this industry is in the intellectual property we put into drug development.

However, as I said earlier, to sustain these investments and ensure that patients and citizens can enjoy the enormous benefits that come from pharmaceutical innovation, it is necessary to have supportive government policies in the areas of pricing and reimbursement, drug approval, vaccine development, and translational research.

Let me start with pharmaceutical pricing and reimbursement. The decision to extend the price premium system for another two years was an important one, demonstrating the government's commitment to promote innovation.

However, drug development is a long, expensive, and risky venture. Especially in the recent years, drug development is taking longer and costing more with decreased rate of success as the research focus has shifted to those therapeutic areas with more complexity and fewer patients.

Making long-term decisions on drug development demands confidence that rules will not change that will impinge on those investment decisions. That is why we continue to believe that making the price premium system a regular part of the NHI system with no changes is critical to promote innovation in our sector.

We were pleased that the government last spring postponed discussion of changing the current biennial repricing system to an annual one. This is a complex issue, fraught with threats to the success of Prime Minister Abe's economic growth plans. We are deeply concerned about continuing proposals by some to make that change and also to implement a price revision at the time of the next consumption tax hike in 2017. We are strongly opposed to both of these ideas.

Drug prices are lowered every two years with the aggregate saving of 500 billion yen under the current biennial price revision system. Further price cuts during the patent period will discourage investment in Japan, which could have negative results on clinical development projects and employment. This would, inevitably, widen the so-called drug lag again and possibly threaten a stable supply of drugs to Japanese patients.

The existing drug pricing rules are designed based on the premise that the government biennially revises drug prices along with the schedules of physicians and hospital fees in an integrated manner. Without taking into account the consistency of various technical rules in drug pricing and the balance between drug pricing and the physician/hospital fee schedule, the government should not just consider the change of frequency of drug price revisions.

Drug price revisions require an appropriate business environment for a fair market price formulation and the collection of precise data of market prices of individual products. However, performing an accurate survey of market prices in a short period will be extremely difficult given the current business practices and business conditions in the drug distribution sector.

I find it interesting that so many stakeholders in the healthcare system – the Japan Medical Association, the Federation of Japan Pharmaceutical Wholesalers Association, the Japan Pharmaceutical Manufacturers Association, and the Japan Pharmacy Association – all strongly oppose such a change. We join them in this opposition.

I hope this message is understood by government leaders looking for ways to

incentivize and promote innovation and make Japan a world-leading investment destination for medical innovation. We will be bringing this message to those leaders in the coming months and request that they reject any such proposal that would have these negative and unacceptable consequences.

The rule on repricing for market expansion and the 14-day limitation on prescriptions during the first year after approval are also anti-innovation measures that should either be scrapped or significantly altered as part of the Abe Administration efforts to promote innovation in our sector.

On drug approvals, as I said earlier, we are very pleased and impressed by the recent performance of PMDA and its efforts to turn the agency into a world-class group. We look forward to continuing to work together with PMDA in this regard in areas such as revising MRCT guidelines on Phase I data, enhancing the priority review process and introducing rolling submissions, minimizing any gaps in the Risk Management Plan process between PMDA and industry, and aligning risk minimization measures with global practices. Our cooperation with PMDA is at an all-time high, and we expect that to continue.

As for the so-called “SAKIGAKE designation system,” we strongly urge flexible application of the system. Just like the “Breakthrough Therapy” designation in the U.S., we believe “SAKIGAKE designation system” should be applied equally to non-Japanese pharmaceutical companies who are pursuing global and simultaneous drug development and NDA filing, so that the world’s newest medicines can be delivered to Japanese patients and greater investment in drug development in Japan can be promoted.

On vaccines, we welcome additional approvals of foreign-origin vaccines and funding for those vaccines. As most of you in this room know, vaccines, after clean water and sanitation, is the single most cost effective disease prevention measure. Unfortunately, the awareness of the importance of vaccines is still too low among the general public and policymakers. More education and clear public health policies are needed regarding vaccine-preventable diseases such as cervical cancer and rubella.

PhRMA held a joint symposium last year with the Japan Medical Association on this topic – promoting awareness of the importance of vaccines. PhRMA sponsored a visit by top vaccine policy makers in the United States from the CDC – Centers for Disease

Control and Prevention – and the US Department of Health and Human Services where they exchanged best practices with their counterparts in Japan. A major topic of that visit was how to help the public understand how important vaccination is to the health of their children, themselves, and of society. We will have another such visit by these experts later this year.

PhRMA published its second Vaccine Fact Book last year that focused on vaccine development in Japan and the benefits of vaccines, and we disseminated this information as widely as possible.

In addition to public awareness, another vital issue related to vaccines is to ensure that decisions about approval and funding be science-based. All too often, not only in Japan but also in the United States and other countries, misleading and unscientific information prevents the public from getting the benefits of vaccinations, with dire consequences for public health. It is critical that all decisions be based on science.

Finally, let me conclude with a few comments about translational research. This was a topic that came up in almost all the meetings our global Chairman had with government leaders in November. Creation of AMED is one effort to close the gap where drug discoveries in Japan are then developed and commercialized elsewhere, but it is not enough.

Basic science is done in industry, university, and research institute laboratories. For patients and society to benefit from these discoveries, the science must move from the laboratory thru clinical development and, finally, into the market. This is the gap in Japan, and eliminating this gap will require many changes.

PhRMA is trying to help in this area.

Each year we send eight young scientists to the United States for two weeks to learn about how translational research is done in America. This program is designed to help them develop contacts and a network with people who are leading the way in the United States. Our hope is that this small group, growing every year, will bring these new ideas, concepts, and processes back to Japan and help transform how translational research is being done here. Our third group will travel to the United States in September.

Also, last month we held a large symposium with Chiba University with the goal of making younger researchers in Japan aware of the importance of translational research and helping them refocus their efforts to meet this challenge.

I cannot exaggerate the importance of developing translational science to make Japan a pharmaceutical global powerhouse.

In conclusion, PhRMA supports the Abe Administration's policies to promote healthcare innovation. Our entire agenda is based on this goal in the pharmaceutical sector.

Thank you for your attention. I will be happy to answer your questions.