Makoto Suematsu – President, Japan Agency for Medical Research and Development (AMED)

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Makoto Suematsu, president of the Japan Agency for Medical Research and Development (AMED), discusses AMED's successful unification of Japan's previously disparate systems for medical research funding; the strategic significance of the country's strong footprint in regenerative medicine and rare diseases; and the organization's internationalization strategy, propelled by the push to publish its major projects in English.

How was AMED founded and what is its mission?

AMED was born 2.5 years ago. We are a baby, standing on two legs, but still wearing a diaper! Our foundation was as a result of Prime Minister Shinzo Abe's brave decision to reform the funding systems for medical research. We used to have three completely separate funding systems, emanating from three separate Ministries, which generated a very complicated and chaotic situation, akin to attempting to play basketball, baseball and football in the same ground at the same time. Prime Minister Abe asked me to unify the three systems into one and that is how AMED was born.

In our organization's first year, I therefore spent most of my energy on reforming the funding systems. Bureaucrats from all three Ministries were actually very helpful in this process of

unification and changing the rules very rapidly. We still need to expand reforms to each individual university – it takes time – but so far the process has been successful.

We aim to stimulate the biomedical industry and our main mission is to fast-track medical R&D and improve three different types of lives: lifespan, daily life, and quality of life. If any of our reforms adversely impact any of these three types of life, we do not proceed with it.

Aside from the necessity to unify funding sources, what else was behind the creation of AMED- what other deficiencies are being improved, or what existing assets are being enhanced by the agency?

AMED does not hold any laboratories or institutes itself, but the alteration in the funding system means that we have more control of research in the country and can ask the researchers to produce something specific for the patients. Right now, there are 360 full-time employees in our organization – not a huge number – but we also have many consultants and other staff coming from medical and pharmaceutical schools as well as from industry.

In evaluating research proposals, we always consider how the medical schools that we are funding can help other medical schools. We have 80 medical schools here in Japan and many companies, but before AMED, all of these activities were not particularly organized. However, now, when we determine next year’s funding size, we always check how one funded institute can help the others.

One example of this is the Initiative on Rare and Undiagnosed Diseases (IRUD). I have devoted myself to empowering the network system to facilitate the diagnosis for rare and undiagnosed diseases in this country; something which has never been done before. Within 1.5 years, we succeeded in forming a good network of 420 different regional core hospitals, together with the university hospitals in which we can share data well.

Among these hospitals, we are sharing the data of the *phenotype* rather than genotype so that if we see similar patients within this database, we can immediately try to make a candidate case match and then go to the genome analysis. If there is exactly the same gene disrupted, now we can say that this gene is responsible for the generation of that disease. By so doing, within two years we are happy to have discovered 12 different diseases which affect both children and adults. This speed has never been seen before in other countries.

Will AMED focus on funding in areas where Japan can excel or on filling gaps in areas where Japanese researchers are not necessary very involved today? Why do rare diseases and regenerative medicines have such a high budget allocation?

One important field is how to eliminate the balkanization [the process of fragmentation or division into separate parts that are often hostile or uncooperative with one another – Ed.] among Japanese physicians and scientists. I used to be a scientist and we never released our data before publication. But if we hide all of our important data, this affects outcomes for the patient and is a real problem. We need to alter this behavior.

Regenerative medicine is very strong in Japan and so is allocated more and more money. However, due to low patient numbers, very few companies allocate money to rare disease R&D. Therefore, in order to break through this way of thinking, we need to expand the size of our patient database by taking it international. That is my dream. If we can expand the number of countries involved, then we can increase the number of patients, so that ‘rare’ is no longer ‘rare’. I am always thinking about global communication.

What incentives is AMED putting in place to incentivize researchers to share their work more?

IRUD, for example, always asks researchers to facilitate the diagnosis. That accordingly reduces the cost of the research and is why we check, one by one, how and how quickly the individual patient can be diagnosed. There are actually many patients who have suffered for 20-30 years undiagnosed. I am proud to say that almost 500 previously undiagnosed patients now have a diagnosis thanks to the IRUD program. Shortening and speeding up the processes saves money.

For a country with the second or third largest healthcare expenditure in the world; would it not be natural to also focus on other less niche areas of R&D than rare diseases or regenerative medicines?

If we can make a breakthrough in rare disease, then we can move on to common diseases. If we ask the people working in common diseases to do something similar, they always ask for more money. Pediatricians and those working in human genetics or imaging diagnosis, for example, are devoted to saving patients and never ask for more money from the government.

However, researchers in particular fields sometimes ask for budget for things like latest generation technology; they spend a huge amount of money and, in the end, produce only papers. We cannot save the lives of patients using these papers. Those who really diagnose cancer are the pathologists, endoscopists and diagnostic radiologists. These specialists are never funded by the government but seek funding from the private sector. I am interested in unifying and integrating these three communities in order to switch on cancer genomics.

Utilization of tag information in the hospital, such as how the patient was admitted, how they were diagnosed and how the CTI or MRI was conducted is very important and can provide the key to finding the cure when integrated with the genome information of the cancer. If we go into detail only on the genomics side of the cancer, we cannot answer and optimize which treatment is the best. Therefore, in that sense, the integration of these three sectors are very important and will produce a huge, unified database.

AMED decided to use its money to help these specialists to unify together to get funding for this database. Once the patient signs up, we can use all of this data together. By so doing, we can eliminate unnecessary processes and inefficiencies in the system.

You are looking for points where the impact of the science can be fully quantified into products that improves patients' lives. Globally, only the USA and Switzerland seem reasonably satisfied with their translational research into products. Where does Japan stand?

The speeding up of translational research is another important field to which AMED is fully committed. I am very proud of the iD3 booster, a unique, unprecedented approach operated by the Department of Innovative Drug Discovery and Development (iD3) for accelerating the translation of promising basic research into innovative new medicines. There are around 30 people who have retired from pharmaceutical companies, often at a young age, to join us. They are very good at disseminating to the laboratories how they can help drug development in academia. We are opening up this opportunity to use the high throughput screening system and drug libraries to academia. If there are any good seed products, then AMED can allocate them money.

One KPI that the government has asked us to reach in the last few years is an open drug library system for Japanese companies to participate in. Almost 25 companies have provided a portion of their own drug libraries to be usable for other companies. If there is any hit compound, the company takes the right to improve on the seeds inside the company. A company can hold the

right to improve these compounds until the end. This produces a benefit. I am expecting to see us have a successful product in the next couple of years.

However, for the last decade we have seen no Japanese blockbusters globally, how do you explain this?

Japan is very strong in regenerative medicine, the budget for which mainly comes from the Ministry of Education before AMED. In rare diseases, almost 97 percent of patients have a very small chance to receive therapeutic care. Because of this, many Big Pharma companies have invested in rare disease R&D; 25 percent of this R&D money goes to the rare disease field. In the case of rare diseases, not only the small molecule drug, but also the chemotherapy or antibodies. These are important products. We are thinking about the conjunction between the regenerative medicine and rare diseases budgets together, which never happened before AMED, which never happened before AMED existence.

The iPS technology helps and the chemotherapy can also help such patients. Regenerative medicine is our strength and we have a huge amount of legacy data in rare diseases because the Ministry of Health and Welfare established a law which supported rare disease research 45 years ago. Unfortunately, all of this data is written on paper, so therefore, we need to make a digital database and integrate both things. Hopefully, Japan can contribute to rare disease therapeutics rather than create a blockbuster.

You have started to expand your network abroad from Singapore to India, and the UK among others; why is an international network relevant for AMED and where will your focus geographies be in the next few years?

We have three branch offices, in Washington DC, London and Singapore. This year, AMED provided USD 0.55 billion to be used by pharmaceutical companies for R&D in the next ten years as they desire here in Japan. We ask these companies to facilitate drugs and devices for children, rare diseases, and antimicrobial resistance (AMR). In 2050, more human beings might die of infectious diseases than cancer. Every country knows the critical importance of AMR, including tuberculosis, which is now having an outbreak in India. Japanese companies are very good at developing AMR platforms and I am expecting a good product within a couple of years thanks to our support. This could be very useful to think about the huge market in Asia. The weak point of Japan is that phase I

and phase II of clinical trials is too slow.

Recently, we have learned a lot from Australia, in 2010, they produced a centralized institutional review board(IRB). They have a central committee on the east coast which determines yes or no for each individual proposal of clinical research. That is why, after 2010, many Japanese companies only paid attention to what was happening on the east coast of Australia. Right now, I am determined that Japan and Australia build trust between each other and facilitate R&D, particularly for the phase I and II fast-track system.

I will make up a MOU with the National Health and Medical Research Council (NHMRC), the most important funding agency in Australia. Actually, there are many Japanese companies which have already invested in Australia. If Australia and Japan shake hands with each other, this is a very geographically strategic partnership.

We are interested in the UK because of the history of medicine there, as well as the experience and the level of innovation. Irrespective of Brexit, we remain interested in the UK and the large basis of basic medical science there. That is why we made an MOU with Medical Research Council(MRC) and the British network of clinical research. We have a lot to learn.

AMED is pushing for its projects to be written in English as well as Japanese. Will these open doors for Japan globally?

This is a challenge and there are huge opponents inside the organization, but I will make sure that the major projects of AMED will be written in English by 2020, the end of my first term. We have 2,300 different accepted proposals, all written in Japanese. Now, this year, we are opening up 20 open calls which should be written in English. There are multinational review systems and these are all operated in English. This is a small pool, but good enough to sink the old vessel. It is a challenge of course, but necessary. If we change the use of language, there are no obstacles on the side of academia.

What is your final message to our international readership on AMED and partnership opportunities?

I am very interested in opening the opportunity for young investors, not only from academia but from industry. AMED will make sure to use English as our official language within a couple of years. That is the one way to facilitate the fast-track of medical R&D. Our community is very keen to

contribute to global health and improving people’s quality of life, together.

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