



# CDB SEMINAR

Speaker:

**Tsuneo Kido**

< National Institutes of Health, NINDS, LMB >

Title: "What's next? -From bench to bedside -"

Date:	<b>Monday, August 2</b>
Time:	<b>16:00 - 17:00</b>
Place:	<b>7F Conference Room of Building A, CDB</b>

## Summary:

We (including you) have already succeeded to make many cell types like dopaminergic cells, beta cells, hepatocyte etc from embryonic stem (ES) cells. These technologies will give us the future treatments to the patient with Parkinson disease, Diabetes Mellitus, Liver cirrhosis and so on. But the gap between basic research and clinics are still big.

Fortunately, I had a chance to participate in developing the cell product for the treatment of neurological deficit before the discovery of the cells. It's still in a pre-clinical stage and we are preparing the pre-IND package, but there are many things to know for designing the basic experiments, too. And I found that the knowledge of this process is very important for all researchers, who are working especially on regenerative medicine, even they don't intend to do clinical trials in near future. I could improve my basic research because of this knowledge. I want to show this process to you because I believe that it will be helpful for you in understanding your basic research deeply.

There are many things to consider. First, ethical and political problems are still very important to discuss about. What source is good for cell therapy? ES cells? Fetal cells? Adult cells? ..... They are quite different in character, function and efficacy. But we can't always choose the best one without discussing ethical and political issue. Unfortunately, Japan government had not decided anything about this issue when we decide to do the clinical trial. Fortunately, US government doesn't have any regulation and we can use any sources. Therefore, we decide to do clinical trial in US.

Second, process design, which begins after the discovery phase, is very important for the success of clinical trial and should start in R&D. Although some people think that such early planning inhibits creativity, the ultimate goal is to produce a biotherapeutic that will be administered to human patients. Safety is the first to be considered in process design. And the present main concern of FDA is inter-species transmissible disease like bovine spongiform encephalopathy (BSE). If you can't find safe reagents, you will be never allowed to use your product for human.

That's the reason why you need to begin process design just after the discovery phase. You also need to think about the source, assay documentation in this phase.

Third, during preclinical studies we need to evaluate the toxicology and preclinical pharmacokinetics of a potential product and prepare for pre-IND meetings with regulatory authorities. For this safety studies, GLP regulations apply and may be painful to you. You also need to prepare documents for a pre-IND meeting, make plans for production of clinical batches and begin training personnel. And SOPs must be in place for the process and cleaning, documentation, and growing.

Then, you can prepare for the IND meeting to start phase I studies. There are some more steps before product approval, but the initial goal must be here.

In the seminar, I want to talk about several issues as above: ethical problems (source of tissue), political problems, data and documentations (efficacy study, safety study, clinical protocol, reagents and materials), manufacturing (SOPs, facility, quality control) and business plan. My experience is in US but the process should be similar in Japan. Even you need a lot of documents and data, you will be encouraged in front of clinical studies just as me. Many patients are waiting for you. You may help many patients by your findings if you learn this process. Why don't you try? There are many steps before clinical, but bedside is not so far from your bench.

**Host: Shin-Ichi Nishikawa <Stem Cell Biology, CDB>**

E-mail: nishikawa@cdb.riken.jp Tel: 078-306-1893 (ext:5301) RIKEN Center for developmental Biology <http://www.cdb.riken.go.jp/>