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“Cell therapy for kidney diseases”

Rege Nephro Co., Ltd. was established in 2019 by Prof. Osafune in Kyoto University. He is developing various solutions for renal diseases aiming to improve QOL among patients.

The problem that we want to resolve is CKD “chronic kidney disease” because there is no effective treatment against it while the number of patients have been increasing to more than 13 million in Japan. Our solution is iNPC cell therapy to transplant iNPC into the renal sub capsular space.

[What is iNPC?]

iNPCs are nephron progenitor cells, which are designed to differentiate into glomeruli and renal tubules, and were firstly identified in the embryonic kidney by Prof. Osafune. iNPC is a nephron progenitor cell derived from human allergenic iPS cells. Renal sub-capsular transplantation of iNPCs exerts not only serum biochemical efficacy, like BUN and Creatinine but also histological efficacy. Because iNPCs exert a paracrine effect by secreting neurotrophic factors. Recently, we also confirmed the therapeutic effects of iNPC in CKD models. Identified one of the main factors responsible for the therapeutic effects and succeeded in developing iNPC expansion culture method which can significantly reduce cost of manufacturing.

[Production]

As for production, we are developing a scale up manufacturing process using clinical iPS cells in collaboration with Kirin Holdings, Nikkiso, ABI, and SHIMADZU which can save time and cost by a lot.

[Development Strategy]

As for development strategy, we plan to conduct first in human study on CKD with transplanted kidney aiming to obtain early conditional approval. Then move on to a larger indication CKD in the general population, hoping to conduct non clinical safety studies in 2022. And enter clinical trials in 2023. We expect to obtain NDA approval in 2027.

[Sales Forecast]

As for the sales forecast, we expect NHI sales could reach 100 billion yen in 2035. There is a large untapped market for renal diseases.

We have a further product pipeline using NPCs such as reconstruction of kidney, organoids for toxicity test, high throughput screening for ADPKD-specific iPS cells, and next generation NPCs. ADPKD is the most frequent, monogenic disorder, but no curative treatment is available. The next key milestone is a series A. In the fourth quarter this year we hope to raise funds of more than 1 billion YEN for further development activities. We are discussing with several Pharma companies about how to co-develop iNPC therapies and how to collaborate HT screening of good drugs.

[Q & A]

Q.

Why don't you develop the one of the main factors you identified as a drug that is simple and cheaper, I guess.

A.

Cell therapy is the best solution approach, because effective factors are not one, but also maybe 3 or 4 factors. And also, the factor is administered via vein, but it's not easy to reach the kidney, and this factor may affect badly to other organ. So, because we think the cell therapy would be the best solution and CKD caused by many factors. So therefore, cell therapy can effect on multi aspect. So cell therapy would be the best solution for kidney disease.

Q.

Do you need license from Kyoto University to induce iNPC from iPS cells?

A.

Yes, of course, we have already been granted the patent with iPS cell. Use of iPS cell and differentiation method of iNPC from iPS cell.

During the production development, we identified excellent technology, which can be filed as a patent, but not only by ourselves, but also collaborative patent application with Kyoto University.

Q.

You said that first indication is CKD of transplanted kidney. Why did you choose this as a first indication?

A.

Because the patient with a transplanted kidney has already been administrated by immunosuppressant. We are going to use allergenic iPS cells. So no additional suppressant administration could be acquired. And also, transplanted kidney is located very near to skin. Therefore, it is easy to transplant iPS cell into sub capsular space and finally, the number of the population of CKD with transplanted kidney is a few in Japan. Therefore, we will not be required to conduct double blind study or confirmative study. So maybe we can apply early conditional approval.

Q.

I am wondering the transplanted kidney has the GVHD or other complication may happen and also the effect of the transplantation of the cell could be not only the regeneration of the nephronal so other type of effect. Is that the Proof of Concept study, or just the study to salvage those kinds of very severe patients?

A.

It could not be POC study, but we can confirm some efficacy against the CKD.

Q.

In that case, is that regeneration of nephron or other type of the stem cell effect? What is the mechanism?

A.

Mechanism of mode of action is paracrine effect by secreting the renotrophic factors and we confirmed one factors, and we are analyzing how to exert an efficacy. So then, we could explain the model reaction in case confirming some kind of efficacy.