

Company Description:

Demonstrating the unprecedented iPSC (induced Pluripotent Stem Cell) based drug discovery technology for more effective treatment of ALS (Amyotrophic Lateral Sclerosis)



Company Details:

Founded: 2016

No. of employees: 20

Type of Ownership: Public

Stock exchange: 4896 (Tokyo Stock Exchange Growth Market)

Interviewee: MATSUMOTO Masato, CFO

Company Address:

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Introduction:

May 2026:

Specialized in iPSC drug discovery and regenerative medicine, with its expertise in CNS (Central Nervous System) diseases, K Pharma is preparing for Phase III trials for its flagship project: ALS treatment.

Venture Valuation interviewed MATSUMOTO Masato, CFO

Venture Valuation:

Your competitive advantage is the ability to induce the differentiation of iPSC cells into consistent and high-quality nerve cells. This is crucial to screening compounds, evaluating drug efficacy and safety, and developing cell-based therapies.

Matsumoto:

To advance iPSC technology, it is essential to maintain reliable and top-grade differentiation into human cell types such as neurons.

We are working with the research team at Keio University School of Medicine, who have used patient-derived iPSCs to create motor neurons affected by ALS, an incurable and fatal disease. By screening 1,232 existing compounds with expired patents, ropinirole hydrochloride, a drug used for Parkinson's disease patients, was identified as a potentially effective compound for ALS.

The chart below¹ shows the results of the Phase I/IIa trial in Japan: a small-scale (20 participants), double-blind, placebo-controlled feasibility trial. Safety and tolerability were confirmed, and physical decline as measured by the ALSFRS-R (ALS Functional Rating Scale-Revised) was slower in patients receiving ropinirole than in the placebo group.

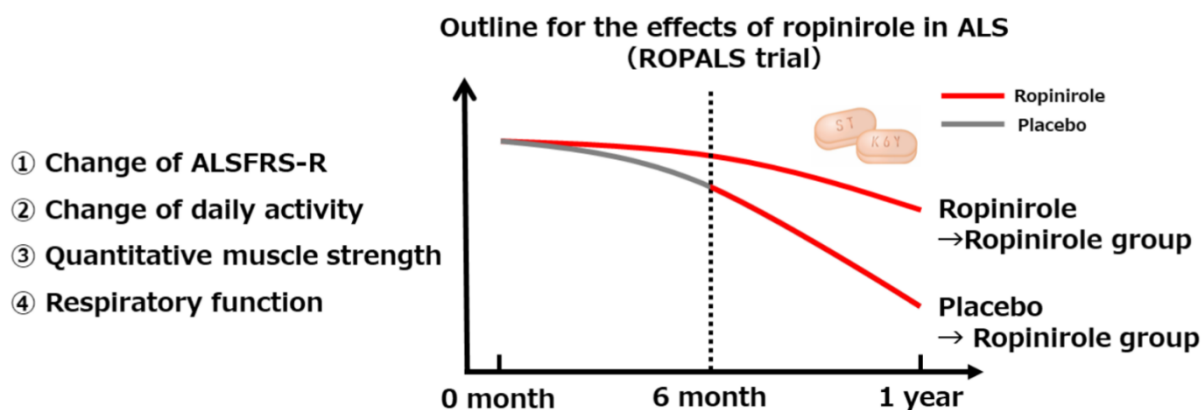


Fig. 2: Outline for the effects of ropinirole in ALS (ROPALS trial).

Based on criteria ① ② ③ and ④, the trial, although limited in size, suggests that ropinirole may slow down the progression of the disease.

The research team reported that "the median survival of the patients receiving ropinirole was 50.3 weeks while it was 22.4 weeks for the patients receiving the placebo."

¹ <https://www.keio.ac.jp/en/press-releases/files/2021/5/20/210520-1.pdf>

As the trial results reveal better efficacy than the currently available treatments, you are preparing for Phase III in Japan and a pre-IND (investigational new drug) meeting with the FDA (Food and Drug Administration) in the U.S.

Phase III trials are expected to involve around 200 patients across multiple centers in Japan for a period of 2 to 2.5 years.

In January of this year, we made a public announcement that the U.S. FDA provided the written response to our request for the pre-IND meeting. The Phase I/IIa data supported the transition to Phase III; therefore, the discussion focused primarily on Phase III results for registration. The pre-IND meeting is a great opportunity to introduce our iPSC technology for treating patients with rare life-threatening diseases.

Along with iPSC drug discovery, you are conducting research on applying regenerative medicine in practical applications. The project for the treatment of subacute spinal cord injury is in progress.

Our research on regenerative medicine applies iPSC cells derived from healthy individuals. In December 2021, an investigator-initiated clinical study was conducted to transplant iPSC-derived neural progenitor cells by injection into four patients in the subacute phase of spinal cord injury. The subacute phase, defined as two to four weeks post-injury, is considered the appropriate window period to maximize neurological recovery.

Since the procedure's safety and tolerability have been confirmed, and encouraging efficacy results have been observed, we are planning a K Pharma-led clinical study with approximately seven to eight patients.

It is vital for a start-up to raise funds to keep projects advancing. Your business strategy is to find right licensing partners for each project within a reasonable timeframe.

We know very well that developing treatments for rare diseases with small patient populations is medically valuable but financially challenging.

The drug discovery program includes six projects: ALS, FTD (frontotemporal dementia), HD (Huntington's disease), hearing impairment, neuroferritinopathy, and Nasu-Hakola disease. Additionally, the regenerative medicine program has five projects: subacute spinal cord injury, chronic spinal cord injury, chronic cerebral infarction, chronic cerebral hemorrhage, and chronic traumatic brain injury.

Some of these projects already have partners. Any suggestions and inquiries on partnership are more than welcome.

VV Comments After the Interview

K Pharma is a spin-off company from Keio University School of Medicine, a top medical school in Japan, led by two prominent professors: Dr OKANO Hideyuki (Research Center for Regenerative Medicine) and Dr NAKAMURA Masaya (Department of Orthopedic Surgery). The company has access to a small sample size of patients affected by rare diseases through the university's tightly connected network of around 9,700 medical alumni² and over 100 related medical institutions. A key strength for K Pharma is to organize clinical trials for rare diseases in a timely manner.

K Pharma's iPSC technology provides versatile applications for neurological diseases, from rare intractable diseases to common diseases. That is the company's "rare to common strategy". Once their ALS treatment is approved, common diseases, for instance, Alzheimer's, could be a target.

The number of ALS cases in the world was estimated to be 222,801 in 2015 and projected to be 376,674 by 2040.³ Meanwhile, the current number of Alzheimer's disease cases is approximately 33 to 38 million worldwide and projected to reach 139 million by 2050.⁴

Contact Details

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Venture Valuation specializes in independent assessment and valuation of technology-driven companies in growth industries, such as the Life Sciences (Biotech, Pharma, and Medtech), ICT, Nanotech, Cleantech and Renewable Energy. In addition to valuation products, Venture Valuation offers high-quality, focused information services like the Global Life Sciences Database, Biotechgate.com and this "*Let's Interview Series*" with companies with interesting technologies and services. We select and interview thriving companies and organizations.

² <https://www.sanshikai.jp>

³ <https://www.nature.com/articles/ncomms12408>

⁴ <https://www.alzint.org/about/dementia-facts-figures>