



PRESS RELEASE

For Immediate Release

This document is intended for members of the press. This product (PAL-222) is an investigational regenerative medical product currently under development and has not yet been approved.

PharmaBio Achieves Primary Endpoint in Phase I/IIa Clinical Trial (PAMyCA Trial) of PAL-222, a Human (Allogeneic) Adipose-Derived Mesenchymal Stromal Cell Sheet, for Myopic Chorioretinal Atrophy

NAGOYA, Japan—October 2nd, 2026—PharmaBio Corporation (Representative Director and CEO: Hitoshi Kusano; hereinafter "the Company") today announced that the Phase I/IIa clinical trial (PAMyCA Trial) of the regenerative medical product PAL-222 for myopic chorioretinal atrophy achieved its primary endpoint, demonstrating the suppression of the expansion of the chorioretinal atrophy area. The top-line results also indicated the potential for maintaining visual function and a favorable safety profile.

Myopic chorioretinal atrophy is a progressive disease in which the retinal pigment epithelium, choroid, and photoreceptors irreversibly degenerate and atrophy due to pathologic myopia. It is considered one of the leading causes of blindness and severe visual impairment. To date, no effective treatment to suppress its progression and maintain or improve visual function has been established, representing a significant unmet medical need.

This exploratory trial was conducted to evaluate the safety and efficacy of PAL-222 transplanted into the subretinal space of patients with myopic chorioretinal atrophy. PAL-222 is a disc-shaped regenerative medical product composed of human (allogeneic) adipose-derived mesenchymal stromal cells (MSCs). This product consists of layered cells and the extracellular matrix (ECM) produced by the cells themselves, featuring a structure that retains the ECM. Through paracrine and other mechanisms, PAL-222 is expected to exert neuroprotective, tissue repair, and anti-inflammatory effects, which are expected to contribute to the protection of retinal and choroidal tissues and the maintenance of visual function.

PAL-222 was transplanted into all 11 subjects enrolled in this trial and evaluations were performed over a 52-week observation period. For the efficacy evaluation, a comparative analysis was conducted by defining the transplanted eye as the study eye and the non-transplanted eye as the control eye.

Primary Endpoint: Efficacy (Changes in Chorioretinal Atrophy Area)

The trial achieved its primary endpoint, demonstrating a statistically significant suppression of chorioretinal atrophy area expansion in the study eyes compared to the control eyes.

- Progression was almost entirely suppressed in the study eyes, whereas the chorioretinal atrophy area in the control eyes expanded over time (Figure 1).
- At week 52 post-transplantation, the mean $\pm$ standard deviation of the log-transformed area ratio was -0.01 ± 0.02 for the study eyes and 0.12 ± 0.07 for the control eyes.
- The point estimate of the difference between the study and the control eyes was -0.13 (95% Confidence Interval (CI): $-0.18, -0.08$), confirming a significant difference (paired t-test: $p<0.001$, permutation test: $p<0.001$) (Table 1).

Figure 1. Time Course of Chorioretinal Atrophy Area (Rate of Change)

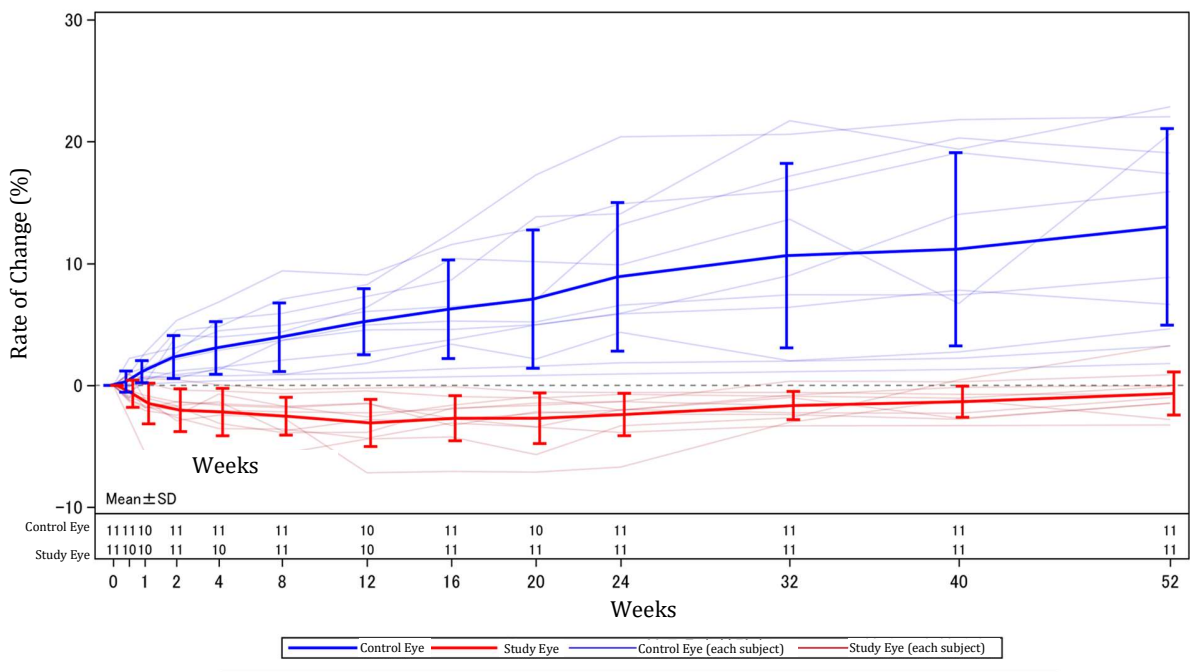


Table 1. Changes in Chorioretinal Atrophy Area Before and After Transplantation

Timepoint	Statistic	Study Eye	Control Eye	Difference (95% CI)	p-value
52 Weeks Post-transplantation	Number of Cases	11	11		
	Mean (SD)	-0.01 (0.02)	0.12 (0.07)	-0.13 (-0.18, -0.08)	p<0.001

Secondary Endpoints: Efficacy (Maintenance of Visual Function)

- At week 52 post-transplantation, no statistically significant differences were detected between the study and control eyes regarding the secondary endpoints of retinal sensitivity and best-corrected visual acuity.
- Meanwhile, both parameters in the study eyes (with best-corrected visual acuity measured by LogMAR) were generally maintained at baseline levels through week 52 post-transplantation, suggesting a potential association of the suppression of chorioretinal atrophy area expansion with the maintenance of visual function (suppression of disease progression).

Safety Evaluation

No significant new safety concerns were identified in this trial.

- No serious adverse events attributed to PAL-222 were observed.
- No findings suggestive of graft rejection, tumor formation, ectopic tissue formation, or severe immunological reactions—which are known concerns associated with MSC products—were confirmed.
- No serious surgery-related complications that could severely impact visual function were observed.

The overall results suggest that PAL-222 suppresses the progression of chorioretinal atrophy, potentially leading to the maintenance of visual function. The results also suggest the potential of PAL222 as a novel regenerative medicine option that targets disease progression through cytoprotection and tissue repair.

Comment from Professor Tsutomu Yasukawa, Nagoya City University Hospital (Medical Expert for this Trial):

"Myopic chorioretinal atrophy is a severe disease for which there is currently no effective treatment, leaving patients with progressive vision loss and expanding central scotoma without any adequate treatment options. The trial results not only confirmed the favorable safety profile of PAL-222 but also demonstrated its potential to maintain visual function and exert tissue-protective effects by suppressing the expansion of the atrophy area. I believe this represents a promising result—a new therapeutic approach that directly targets and suppresses the underlying pathology itself to prevent the visual impairment caused by atrophy expansion."

Comment from Hitoshi Kusano, Representative Director and CEO, PharmaBio Corporation:

"I would like to express my sincere gratitude to the patients and their families who participated in this trial, as well as the medical institutions and researchers for their dedicated efforts in conducting this study. We consider the trial results to be a significant milestone demonstrating the clinical potential of PAL-222. We consider these findings highly meaningful, as they highlight the potential to bring a new treatment option to patients suffering from myopic chorioretinal atrophy—a disease area where no effective treatments currently exist. Moving forward, the Company will proceed with consultations with regulatory authorities towards filing for approval, while aiming to bring this therapy to patients as soon as possible."

Future Developments

Based on the data obtained from this trial, the Company is committed to steadily advancing the development of PAL-222, with the immediate objective of securing conditional and time-limited approval. Concurrently, we are exploring potential global business expansion while considering our long-term development strategies. Furthermore, we intend to present the results of this trial at upcoming medical conferences and submit them to peer-reviewed journals, actively disseminating our findings to both scientific and medical communities.

About PAL-222

PAL-222 is a regenerative medicine product manufactured from human (allogeneic) adipose-derived mesenchymal stromal cells using proprietary cell processing technology. It comprises layered cells and a self-generated extracellular matrix (ECM), featuring a unique structure that preserves the intact ECM. Mediated by paracrine and other mechanisms, PAL-222 is expected to exert neuroprotective, tissue repairing, and anti-inflammatory effects, thereby protecting retinal and choroidal tissues and maintaining visual function.

About Myopic Chorioretinal Atrophy

Myopic chorioretinal atrophy is a disease characterized by the progressive atrophy of the retinal pigment epithelium and choroid, occurring secondary to axial length elongation induced by pathologic myopia. As lesions progress, central visual acuity declines markedly, potentially leading to blindness in severe cases. Currently, no approved drugs or therapeutic modalities exist to treat the advanced atrophic lesions themselves, establishing a global demand for the development of novel therapeutic approaches.

About the PAMyCA Trial

- Trial Name: Phase I/IIa Clinical Trial of PAL-222 for Patients with Myopic Chorioretinal Atrophy
- jRCT Number: jRCT2043220105
- Target Disease: Myopic Chorioretinal Atrophy
- Trial Phase: Phase I/IIa
- Objective of the Trial: To exploratorily evaluate the safety and efficacy of transplanting the regenerative medicine product PAL-222 into patients with myopic chorioretinal atrophy without active choroidal neovascularization
- Trial Design: Non-randomized, open-label, untreated fellow eye controlled comparative study
- Treatment for Study Eye: A single sheet of PAL-222 is transplanted into the subretinal space via pars plana vitrectomy
- This clinical trial is a Phase I/IIa study in regenerative medicine, designed to evaluate the safety and therapeutic potential of subretinal PAL-222 transplantation in suppressing the progression of myopic chorioretinal atrophy. The main inclusion criteria are as follows:
 - Age 20 years or older
 - High myopia (refractive error of -6.0 D or worse) or an axial length equivalent to -6.0 D or greater
 - Best-corrected visual acuity (BCVA) letter score of less than 60 letters in the study eye
 - Bilateral myopic chorioretinal atrophy with an atrophic lesion of a specified size or greater located near the fovea in the study eye
 - Absence of active choroidal neovascularization

The primary endpoint is the rate of change (%) in the chorioretinal atrophy area at week 52 post-transplantation, which is designed to evaluate the efficacy of PAL-222 in suppressing atrophic lesion progression. Long-term safety data collection is currently ongoing, with observation up to 104 weeks.

Forward-Looking Statements

This press release contains forward-looking statements regarding the Company's intentions, beliefs, current expectations, or future events. These forward-looking statements are based on information currently available to the Company as of the date hereof and are subject to certain assumptions. Accordingly, such forward-looking statements involve inherent risks and uncertainties, and actual results may differ materially from those expressed or implied by these forward-looking statements. Readers are cautioned not to place undue reliance on these forward-looking statements.

Contact Information

PharmaBio Corporation

Life Innovation Center, 3-25-22 Tonomachi, Kawasaki-ku, Kawasaki, Kanagawa

Email: press@pharmabio.co.jp