RBI's Phase 1, First-in-Mankind Direct-to-Brain Stem Cells Show Improved CSF Proteomics, Cognition in Alzheimer's

Presented at ISCT 2026 in Dublin May 6-9: intracerebroventricular injections showed sustained CSF, amyloid PET, cognitive improvements in Alzheimer's disease.

NEWPORT BEACH, CA, UNITED STATES, May 12, 2026 /EINPresswire.com/ -- Regeneration Biomedical, Inc. (RBI), a clinical-stage biotechnology company developing direct-to-brain regenerative therapies for neurodegenerative diseases, today announced first-in-mankind cerebrospinal fluid (CSF) proteomic, amyloid PET imaging, and exploratory cognitive findings from its completed FDA-authorized Phase I clinical trial evaluating intracerebroventricular (ICV) administration of its proprietary Wnt-activated autologous adipose-derived stem cell therapy (RB-ADSC) in patients with mild-to-moderate Alzheimer's disease. The company also announced that, based on the Phase I safety and biomarker findings, the U.S. Food and Drug Administration (FDA) has cleared RBI to proceed into a Phase II clinical trial evaluating RB-ADSC in Alzheimer's disease.

> "The persistence of biomarker improvements one year after a single treatment raises the possibility that these cells may be inducing durable regenerative and immunomodulatory effects within the brain"
> *Christopher Duma, MD, FACS*

The findings were presented at the International Society for Cell & Gene Therapy Annual Meeting, one of the world's leading scientific conferences focused on advanced cell and gene therapies.

The trial represents what RBI believes to be the first-in-mankind study evaluating direct intracerebroventricular administration of Wnt-activated autologous adipose-derived stem cells for Alzheimer's disease, with longitudinal CSF proteomic analysis demonstrating broad modulation of neurodegenerative biomarker pathways following a single treatment.

The results demonstrated that a single direct-to-brain injection of RB-ADSCs was associated with substantial reductions and normalization trends in multiple biomarkers associated with Alzheimer's disease pathology, including cerebrospinal fluid amyloid beta, phosphorylated tau (p-Tau), histone-related neurodegenerative signaling markers, and broader inflammatory

proteomic signatures. Favorable changes in cognitive performance and amyloid PET imaging were also observed.

RBI's approach utilizes ex vivo-expanded autologous stem cells activated through a proprietary Wnt-signaling protocol and injected directly into the brain's ventricular system through an Ommaya reservoir, allowing broad CNS exposure through cerebrospinal fluid circulation while bypassing the blood-brain barrier. Unlike anti-amyloid antibody therapies such as Leqembi and Kisunla, which primarily target amyloid plaque burden, RB-ADSC demonstrated broad simultaneous effects across multiple neurodegenerative biomarker systems following a single treatment, including favorable changes in phosphorylated tau, total tau, CSF proteomic markers, amyloid PET imaging, and exploratory cognitive measures. Importantly, these biomarker changes emerged following a single administration without chronic infusion therapy, and no treatment-related adverse events, ARIA events, or serious adverse events were observed through 16 months of follow-up.

"The rapidity of the biologic response was particularly striking," said Christopher Duma, MD, founder and president of Regeneration Biomedical, Inc. and Medical Director of the Stereotactic Radiosurgery Department at Hoag Memorial Presbyterian Hospital. "In most patients, following only a single intracerebroventricular injection, we observed significant changes in CSF proteomic and neurodegenerative biomarker levels within just 12 weeks. What is particularly compelling is both the magnitude and durability of these changes after direct delivery into the cerebrospinal fluid."

"While the precise mechanism of action remains under investigation, the breadth and speed of the biomarker changes suggest that multiple biologic mechanisms may be contributing simultaneously." said Gabriel Nistor, Scientific Advisor to Regeneration Biomedical, Inc. "Potential mechanisms may include paracrine and exosome-mediated suppression of neuroinflammation, modulation of GSK-3β and tau signaling pathways, enhancement of intracellular tau clearance, restoration of neuronal integrity, and shifts in APP processing away from amyloidogenic pathways through suppression of BACE1 and γ-secretase activity. The CSF proteomic findings suggest these cells may be broadly modulating multiple interconnected neurodegenerative pathways simultaneously."

Key Findings from Baseline to 12 Weeks Following Treatment
- Median CSF p-Tau decreased 56.9%
- Median CSF total tau decreased 54.6%
- Median amyloid PET centiloid scores decreased 23.6%
- Median ADAS-Cog-13 cognitive scores improved 13.8%

Biomarker Improvements Persisted Through 52 Weeks Despite No Repeat Dosing
- Median CSF p-Tau remained decreased by 65.4%
- Median CSF total tau remained decreased by 62.8%
- Median amyloid PET centiloid scores remained below baseline at one year

The company additionally reported favorable modulation of CSF proteomic markers associated with neuroinflammation, neuronal injury, cellular stress signaling, and extracellular histone biology, pathways increasingly believed to contribute to progressive neurodegeneration in Alzheimer's disease.
The Phase I study enrolled six participants aged 45–80 years with FAST stage 4–5 Alzheimer's disease who received escalating doses of 2 million or 5 million RB-ADSCs following lipoaspiration and Ommaya reservoir placement.

"The persistence of biomarker improvements one year after a single treatment raises the possibility that these cells may be inducing durable regenerative and immunomodulatory effects within the brain," Dr. Duma added. "We are encouraged that the FDA has now cleared us to advance into Phase II clinical trials, where we will further evaluate efficacy, durability, repeat dosing strategies, and broader proteomic responses in a larger patient population."

RBI is currently preparing to initiate a randomized placebo-controlled Phase II trial of RB-ADSC in Alzheimer's disease and plans to expand evaluation of its direct-to-brain stem cell platform into additional neurodegenerative disorders, including Chronic Traumatic Encephalopathy, Amyotrophic Lateral Sclerosis, Multiple Sclerosis, and Parkinson's Disease, based on the broad neurodegenerative and inflammatory biomarker changes observed in the Phase I study.

About RB-ADSC
RB-ADSC is an investigational autologous adipose-derived stem cell therapy manufactured using RBI's proprietary Wnt-activation protocol designed to enhance regenerative signaling activity. The therapy is administered intracerebroventricularly to bypass the blood-brain barrier and facilitate broad CNS exposure through cerebrospinal fluid circulation.

About Regeneration Biomedical, Inc.
Regeneration Biomedical, Inc. is a clinical-stage biotechnology company developing direct-to-brain regenerative cell therapies for Alzheimer's disease and other neurodegenerative disorders. The company's platform focuses on intracerebroventricular delivery of autologous stem cells designed to broadly modulate neurodegenerative pathways throughout the brain.

Media Contact:
Regeneration Biomedical, Inc.
Newport Beach, California
info@regenerationbiomedical.com

Forward-Looking Statements
This press release contains forward-looking statements regarding investigational therapies and clinical development plans. RB-ADSC is an investigational therapy and has not been approved by the U.S. Food and Drug Administration. Clinical outcomes observed in early-stage trials may not be predictive of results in larger randomized controlled studies.

Robert Lynn
Regeneration Biomedical, Inc.
+1 877-240-1660
ROBERT@REGENERATIONBIOMEDICAL.COM
Visit us on social media:
LinkedIn
Instagram
Facebook
X
