



Arrowhead Pharmaceuticals Announces ARO-MAPT Interim Phase 1/2a Clinical Data Demonstrating Deep and Durable Tau Reductions in Healthy Volunteers

October 14, 2026

- Arrowhead's TRiM™ platform achieves clinical validation for delivery of siRNA that crosses the blood-brain barrier after subcutaneous injection
- ARO-MAPT demonstrates mean cerebrospinal fluid total tau reduction of 73% on Day 105, with a maximum individual reduction of 85%, supporting the potential for quarterly dosing
- Arrowhead will host a webinar today, October 14, at 2:30 PM ET/11:30 am PT to review additional interim results

PASADENA, Calif.--(BUSINESS WIRE)--Oct. 14, 2026-- [Arrowhead Pharmaceuticals, Inc.](#) (NASDAQ: ARWR) today announced interim results from an ongoing Phase 1/2a study of ARO-MAPT, the company's investigational RNA interference (RNAi) therapeutic being developed as a potential treatment for Alzheimer's disease and other tauopathies. These results represent the first successful clinical validation of a platform to deliver siRNA across the blood-brain barrier and achieve substantial target gene knockdown in the brain. In healthy volunteers, subcutaneous administration of ARO-MAPT achieved deep and durable reductions in cerebrospinal fluid (CSF) total tau, with a mean reduction of up to 73% at Day 105 and a maximum reduction of 85%.

Arrowhead will review additional results during its webinar today at 2:30 pm ET/11:30 a.m. PT, featuring presentations from Arrowhead management and Adam Boxer, M.D., Ph.D., Endowed Professor in Memory and Aging in the Department of Neurology at the University of California, San Francisco. A live webcast with Q&A and replay will be available on the [Events and Presentations](#) page under the Investors section of the Arrowhead [website](#).

"These early clinical data are exciting because they demonstrate that ARO-MAPT can achieve deep and durable reductions of tau expression in the brain following a simple subcutaneous administration, representing an important step toward developing a potential treatment for Alzheimer's disease and other primary tauopathies," said Christopher Anzalone, Ph.D., President and Chief Executive Officer of Arrowhead. "Abnormal tau accumulation is a key feature of multiple devastating diseases, and we believe reducing tau production has the potential to offer a meaningful new approach for patients. Equally important, these results provide clinical validation of our proprietary Targeted RNAi Molecule (TRiM™) delivery platform to potentially address multiple diseases in the central nervous system (CNS). This major scientific breakthrough further solidifies Arrowhead's position as the leading innovator in siRNA therapies."

ARO-MAPT is designed to silence expression of the microtubule-associated protein tau (*MAPT*) gene in the central nervous system, reducing production of all six isoforms of the tau protein. Pathological aggregation of tau is implicated in a group of neurodegenerative diseases called tauopathies, which include Alzheimer's disease, MAPT-related frontotemporal dementia, and Progressive Supranuclear Palsy.

Interim Healthy Volunteer Results

- **CSF total tau:** ARO-MAPT administered at 2.1 mg/kg and 6.4 mg/kg on Days 1, 8, and 15 achieved mean reductions of 40% and 69%, respectively, at Day 105. ARO-MAPT administered at 12.8 mg/kg on Days 1 and 15 achieved a mean reduction of 73% at Day 105 with a maximum reduction of 85%.
- **Durability:** Day 105 represents 90 days following the final dose, with the sustained reduction in CSF total tau supporting the potential for dosing once every three months or less frequently.

The reported biomarker results are from healthy volunteers. Effects on tau protein tangles in the brain, cognition, function, and disease progression must be investigated in patients with Alzheimer's disease and other tauopathies. These patients are being studied in Part 2 of the current Phase 1/2a study and in additional planned studies.

Safety and Tolerability

ARO-MAPT was generally well tolerated. Most treatment-emergent adverse events (TEAEs) were Grade 1 or mild. No serious TEAEs, deaths, or TEAEs leading to discontinuation of study treatment or participation in the study were reported. No persistent anemia or neurobehavioral adverse events were reported. The most common reported TEAEs were injection-site reactions, vessel puncture-site bruising, upper respiratory tract infection, back pain, and headache.

Next Steps in Development

All healthy volunteer cohorts are fully enrolled. Arrowhead plans to complete dosing in the multiple-ascending-dose healthy-volunteer cohorts and continue enrollment in the early Alzheimer's disease cohorts, with data updates expected in 2027. The company has also initiated regulatory discussions in preparation for planned studies in MAPT-associated frontotemporal dementia and Progressive Supranuclear Palsy.

About ARO-MAPT

ARO-MAPT is designed to silence CNS expression of the microtubule associated protein tau (*MAPT*) gene, which encodes the tau protein. Aggregation of abnormal tau is believed to be a key driver in multiple tauopathies, including Alzheimer's disease, and primary tauopathies including MAPT-related frontotemporal dementia, and Progressive Supranuclear Palsy. By preventing or potentially reversing tau protein accumulation, ARO-MAPT has potential to prevent or slow disease progression.

About the AROMAPT-SC-1001 Phase 1/2 Study

AROMAPT-SC-1001 ([NCT07221344](#)) a Phase 1/2a placebo-controlled dose-escalating study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of ARO-MAPT in approximately 64 normal healthy volunteers (NHV) and up to 48 subjects with early Alzheimer's disease (AD), defined as mild cognitive impairment due to Alzheimer's disease and mild Alzheimer's disease dementia. In Part 1a of the study, NHVs will

receive between one and three doses of ARO-MAPT or placebo by subcutaneous injection. In Parts 1b and 2a, NHVs and subjects with early AD will receive between two and three doses followed by one to two additional doses, for a total of up to five subcutaneous doses of ARO-MAPT or placebo.

About Arrowhead Pharmaceuticals

Arrowhead Pharmaceuticals (NASDAQ: ARWR) is a commercial-stage pharmaceutical company developing medicines that treat intractable diseases by silencing the genes that cause them, harnessing the natural RNA interference (RNAi) mechanism. The company has built a broad portfolio of clinical and commercial RNAi therapeutics through its industry-leading targeted RNAi molecule (TRiM™) platform, which can precisely silence genes in a wide range of cell types, including liver, lung, muscle, adipose, and central nervous system tissue. At Arrowhead, we rapidly advance potential best- and first-in-class RNAi treatments for diseases with significant unmet medical need, because every day matters to the patients we serve.

For more information, please visit www.arrowheadpharma.com, or follow us on X (formerly Twitter) at [@ArrowheadPharma](https://twitter.com/ArrowheadPharma), [LinkedIn](https://www.linkedin.com/company/arrowheadpharma), [Facebook](https://www.facebook.com/arrowheadpharma), and [Instagram](https://www.instagram.com/arrowheadpharma). To be added to the Company's email list and receive news directly, please visit <http://ir.arrowheadpharma.com/email-alerts>.

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This news release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Any statements contained in this release except for historical information may be deemed to be forward-looking statements. Without limiting the generality of the foregoing, words such as "may," "will," "expect," "believe," "anticipate," "hope," "intend," "plan," "project," "could," "estimate," "continue," "target," "forecast" or "continue" or the negative of these words or other variations thereof or comparable terminology are intended to identify such forward-looking statements. In addition, any statements that refer to projections of our future financial performance, trends in our business, expectations for our product pipeline, products or product candidate or other characterizations of future events or circumstances are forward-looking statements. These forward-looking statements include, but are not limited to, statements about our beliefs and expectations regarding the long-term impacts on patient health and the health care system; our beliefs and expectations regarding the pricing, value, or expected timing for availability of our drugs and drug candidates; and our beliefs and expectations around the potential uses and value of the TRiM™ platform. These statements are based upon our current expectations and speak only as of the date hereof. Actual results or outcomes may differ materially and adversely from those expressed in any forward-looking statements as a result of numerous factors and uncertainties the safety and efficacy of our products and product candidates, pricing and reimbursement decisions related to our products, demand for our products, decisions of regulatory authorities and the timing thereof, the duration and impact of regulatory delays in our clinical programs, our ability to finance our operations, the likelihood and timing of the receipt of future milestone and licensing fees, the future success of our scientific studies, the timing for starting and completing clinical trials, rapid technological change in our markets, the enforcement of our intellectual property rights, and the other risks and uncertainties described in our most recent Annual Report on Form 10-K, subsequent Quarterly Reports on Form 10-Q and other documents filed with the Securities and Exchange Commission from time to time. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

Source: Arrowhead Pharmaceuticals, Inc.

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Source: Arrowhead Pharmaceuticals, Inc.