

Rafael Holdings Announces Patent Licensing Agreement with MIT for "Small Molecules to Improve Myelination in ApoE4 positive Alzheimer's Disease"

License of the MIT patent strengthens Rafael's intellectual property around cyclodextrins as an Active Pharmaceutical Ingredient (API) in the treatment of ApoE4-positive Alzheimer's Disease

NEWARK, N.J., April 22, 2026 (GLOBE NEWSWIRE) -- Rafael Holdings, Inc. (NYSE: RFL) today announced that its subsidiary, Cyclo Therapeutics, has entered into an exclusive licensing agreement with Massachusetts Institute of Technology ("MIT") covering U.S. Patent No. 12285440. This license covers the use of cyclodextrins in patients with Alzheimer's Disease (AD) who carry an ApoE4 genetic mutation, strengthening the company's intellectual property portfolio around Trappsol[®] Cyclo[™] in this important AD patient population.

The ApoE4 gene variant is estimated to be present in 50-70% of patients with AD and is believed to be a key risk factor in the development early onset sporadic AD. Dysregulated cholesterol metabolism is believed to contribute to amyloid and tau pathology, especially in those patients with the ApoE4 gene variant, which has been shown to accelerate AD as it is the key lipids/cholesterol transporter in the brain.

The established mechanism of action (MOA) for Trappsol[®] Cyclo[™] could have a direct impact in ApoE4 positive patients as it has been shown to improve cholesterol transport between central nervous system (CNS) cells. Preclinical data suggest Trappsol[®] Cyclo[™] can improve delivery of cholesterol between CNS cells, reduce tau-pathology and improve learning/memory in mice.

"Securing this exclusive license from MIT represents a transformative milestone for our company and greater potential positive news for the millions of patients and their families affected by Alzheimer's disease. By combining the results of MIT's world-class foundational research with our clinical development expertise, we are well positioned to accelerate a potential breakthrough that addresses the underlying pathology of this devastating condition. This agreement underscores our commitment to pursuing the highest caliber of innovation to deliver life-changing therapies to patients in need," said Howard Jonas, CEO, Executive Chairman and Chairman of the Board of Rafael Holdings.

"With Phase 3 results from Transport NPC due in calendar Q3, 2026, we're excited by Trappsol[®] Cyclo[™]'s potential across multiple disease areas. Our NPC1 phase 3 study is the largest ever conducted in patients with NPC, yielding crucial clinical and safety data needed to advance the treatment paradigm for patients and families dealing with NPC." said Joshua Fine, COO of Rafael Holdings.

About Rafael Holdings, Inc.

Rafael Holdings, Inc. is a biotechnology company that develops pharmaceuticals and holds interests in clinical and early-stage companies that develop pharmaceuticals and medical devices. Our lead candidate is Trappsol[®] Cyclo[™], which is being evaluated in clinical trials for the potential treatment of Niemann-Pick Disease Type C1 (NPC1), a rare, fatal and progressive genetic disorder. We also hold interests in other clinical-stage and early-stage pharmaceutical development companies and an orthopedic-focused medical device company. Our lead candidate, Trappsol[®] Cyclo[™] is the subject of an ongoing pivotal Phase 3 clinical trial.

Forward Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including without limitation statements regarding our expectations surrounding the potential, safety, efficacy, and regulatory and clinical progress of our product candidates; plans regarding the further evaluation of clinical data; and the potential of our pipeline, including our internal cancer metabolism research programs. These statements are neither promises nor guarantees, but involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements, including, but not limited to, those disclosed under the caption "Risk Factors" in our Annual Report on Form 10-K for the year ended July 31, 2025, and our other filings with the SEC. These factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management's estimates as of the date of this press release. While we may elect to update such forward-looking statements at some point in the future, we disclaim any obligation to do so, even if subsequent events cause our views to change.

Contact:

Barbara Ryan
Barbara.ryan@rafaelholdings.com
(203) 274-2825