Rafael Holdings Announces Completion of Last Patient Last Visit for Pivotal Phase 3 TransportNPC™ Study Evaluating Trappsol® Cyclo™ for the Treatment of Niemann-Pick Disease Type C

Topline results are expected to be announced in second half of calendar 2026

The company has had a pre-NDA meeting with the FDA and expects to submit an NDA in second half of calendar 2026

The company has received acknowledgement from the FDA that our US Expanded Access Program (EAP) can proceed

NEWARK, N.J., June 10, 2026 (GLOBE NEWSWIRE) -- Rafael Holdings, Inc. (NYSE: RFL) today announced the last patient has completed the final 96-week study visit in the pivotal Phase 3 TransportNPC™ study evaluating Trappsol® Cyclo™ for the treatment of Niemann-Pick Disease Type C (NPC). With 94 patients studied across 27 sites in 13 countries, the TransportNPC™ study is the most comprehensive, controlled pivotal study regarding patient size, global footprint, duration and clinical outcomes of an investigational therapy for NPC. Additionally, 10 patients were enrolled in a single-arm sub-study per the adopted Pediatric Investigational Plan (PIP) treating newborns to 3 years of age. Topline data from the main study cohort is expected to be known and announced in the second half of calendar 2026.

Data presented at WORLDSymposium 2026 from the single arm pediatric sub-study in patients ages zero to three, continues to highlight the potential for Trappsol® Cyclo™ as a therapeutic option in NPC with 80% of the sub-study patients' CGI scores showing improvements or remaining stable with no serious adverse events considered to be related to the study drug.

"Reaching the conclusion of the pivotal Phase 3 TransportNPC™ trial is a monumental achievement that belongs, first and foremost, to the patients and families, advocacy organizations, and physicians who stood at the heart of this global effort. Together, we have advanced the clinical understanding of NPC, as well as the lived experience of this devastating disease, as we work to deliver a potentially life changing treatment option for a community with significant unmet need," said Joshua Fine, Chief Operating Officer of Rafael Holdings. "We would also like to recognize the FDA for their long-term continued collaboration, engagement and guidance in advancing this program."

"Having already completed our pre-NDA meeting with the FDA, we believe we have a clear and expedited path forward reflective of the urgency and unmet need in NPC and expect to submit our NDA in the second half of 2026. NPC represents a high-unmet-need market, positioning Rafael Holdings to potentially unlock substantial, long-term value for our shareholders as we work toward transitioning Rafael Holdings into a commercial-stage biotechnology company," said Howard Jonas, Chief Executive Officer, Executive Chairman and Chairman of the Board 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 NPC 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.

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.

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