Rafael Holdings Announces Submission of New Drug Application to FDA for Trappsol® Cyclo™ (remibetadex) for the Treatment of Niemann-Pick Disease Type C

NDA supported by the Phase 3 TransportNPC™ study, data from earlier clinical studies, and an overall survival analysis versus external registry controls

Remibetadex has received Orphan Drug, Fast Track and Rare Pediatric Disease designations from the FDA; approval would make remibetadex eligible for a Rare Pediatric Disease Priority Review Voucher

United States Adopted Names (USAN) Council assigns nonproprietary name "remibetadex" to Trappsol® Cyclo™

NEWARK, N.J., Oct. 29, 2026 (GLOBE NEWSWIRE) -- Rafael Holdings, Inc. (NYSE: RFL) today announced that its subsidiary, Cyclo Therapeutics, LLC, has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) seeking approval of remibetadex (Trappsol® Cyclo™, previously identified as intravenous hydroxypropyl-β-cyclodextrin), for the proposed indication of the treatment of the neurovisceral manifestations of Niemann-Pick Disease Type C (NPC) in adult and pediatric patients from birth. NPC is a rare, fatal and progressive genetic disorder for which there is no known cure. The Company also announced that the United States Adopted Names (USAN) Council has assigned the nonproprietary name "remibetadex" to the compound known until now as intravenous hydroxypropyl-β-cyclodextrin.

Within 60 days of receiving the application, the FDA will determine whether the application is filed and, if filed, whether Priority Review will be granted. If the application is filed, the FDA will assign a Prescription Drug User Fee Act (PDUFA) target action date.

"This submission is an important milestone for Rafael Holdings and, most importantly, for patients living with NPC," said Howard Jonas, Chairman and Chief Executive Officer of Rafael Holdings. "I believe the totality of the data, including the Phase 3 trial results and the overall survival analysis, supports the potential of remibetadex for patients with limited treatment options. Pending FDA approval, we are excited to bring remibetadex to patients and families who urgently need new options to treat NPC."

The NDA includes results from the global, double-blind, placebo-controlled Phase 3 TransportNPC™ study. Although the study did not achieve statistical significance on its primary endpoint, remibetadex showed a directionally favorable 64% relative slowing of disease progression compared with placebo (p=0.19) and in a prespecified analysis of patients receiving background therapy (approximately 83% of the study population), remibetadex showed a 71% relative slowing of disease progression (p=0.046). The NDA also includes data from earlier clinical studies and an overall survival analysis in which remibetadex was associated with an 85% reduction in the risk of mortality in patients with infantile-onset NPC compared with matched registry controls (p=0.044). Remibetadex was generally well tolerated, with no new safety signals observed, although serious adverse events occurred more frequently with remibetadex than with placebo (35.9% vs. 16.7%). Topline results from TransportNPC™ were reported on September 30, 2026.

"The remibetadex NDA is the culmination of more than 15 years of research and investment and, if approved, could help address a significant unmet need in a rare, and fatal disease," said Joshua Fine, Chief Operating Officer of Rafael Holdings. "With the data in the NDA and Orphan Drug, Fast Track and Rare Pediatric Disease designations in place, upon the FDA's filing of the NDA, we believe remibetadex is well positioned for review, and an approval would make it eligible for a Priority Review Voucher, a potential source of non-dilutive capital. With this important milestone completed, we are preparing for a potential commercial launch, and we look forward to working with the FDA as the regulatory review process advances. We are grateful to the investigators, patients, families and advocates who made this milestone possible."

About Remibetadex (Trappsol® Cyclo™)

Remibetadex is an investigational, intravenously administered therapy containing a proprietary formulation of

hydroxypropyl-β-cyclodextrin and is being developed for the treatment of the neurovisceral manifestations of Niemann-Pick Disease Type C (NPC) in adult and pediatric patients from birth. Remibetadex is designed to mobilize cholesterol from late endosomes and lysosomes, facilitating the transport of accumulated cholesterol out of cells so it can be further processed and excreted. Remibetadex has been granted Orphan Drug, Fast Track and Rare Pediatric Disease designations by the FDA for the treatment of NPC.

Remibetadex is an investigational product and has not been approved by the FDA or any other regulatory agency for any indication.

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 remibetadex (Trappsol® Cyclo™, previously identified as intravenous hydroxypropyl-β-cyclodextrin) for which we have completed a pivotal Phase 3 study in Niemann-Pick Disease Type C ("NPC") and submitted an NDA to the FDA in October 2026. NPC is 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; the FDA's review of our NDA, including whether the NDA will be accepted for filing or receive Priority Review; our eligibility for a Priority Review Voucher; our commercial preparations; plans regarding the further evaluation of clinical data; and the potential of our pipeline. 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, the risk that the FDA may not accept for filing or approve an NDA for remibetadex based on the available data; the risk that we may not receive or be able to monetize a Priority Review Voucher; and those disclosed under the caption "Risk Factors" in our Annual Report on Form 10-K for the year ended July 31, 2026, 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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RAFAEL Holdings, Inc.

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