Theriva Biologics Announces Orphan Medicinal Product Designation Granted by the European Commission to VCN-01 for the Treatment of Retinoblastoma

Rockville, MD, October 16, 2024 – Theriva Biologics (NYSE American: TOVX), ("Theriva" or the "Company"), a clinical-stage company developing therapeutics designed to treat cancer and related diseases in areas of high unmet need, today announced that the European Commission has adopted the European Medicines Agency (EMA) recommendation to grant orphan medicinal product designation to lead clinical candidate VCN-01, Theriva's systemic, selective, stroma-degrading oncolytic adenovirus, for the treatment of retinoblastoma. The United States Food and Drug Administration (FDA) has previously granted orphan drug designation and rare pediatric disease designation to VCN-01 for the treatment of retinoblastoma.

"We are very pleased with the European Commission's grant of orphan medicinal product designation to VCN-01, emphasizing the urgent need for new treatment options for retinoblastoma," said Steven A. Shallcross, Chief Executive Officer of Theriva Biologics. "We have previously reported encouraging results from an investigator sponsored Phase 1 trial evaluating the safety and activity of intravitreal VCN-01 in pediatric patients with refractory retinoblastoma, and we are working closely with leading physicians and regulatory agencies worldwide to refine our clinical strategy for VCN-01 as an adjunct to chemotherapy in childen with this challenging disease."

The EMA recommends orphan designation for products intended to treat, prevent or diagnose a disease that is life-threatening or chronically debilitating and either the prevalence of the condition in the European Union (EU) does not exceed 5 in 10,000 or it is unlikely that marketing of the product would generate sufficient returns to justify the investment needed for its development. Additionally, there should be no authorizable method of diagnosis, prevention or treatment of the condition, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition. Orphan designation is designed to provide drug developers with various benefits to support the development of novel therapies, including 10-years of market exclusivity once they receive marketing authorization in the EU, protocol assistance, administrative and procedural assistance, and reduced fees for regulatory activities.

About Retinoblastoma

Retinoblastoma is a tumor that originates in the retina and is the most common type of eye cancer in children. It occurs in approximately 1/14,000 - 1/18,000 live newborns and accounts for 15% of the tumors in the pediatric population < 1 year old. The average age of pediatric patients at diagnosis is 2, and it rarely occurs in children older than 6. In Europe, retinoblastoma has an estimated incidence rate of 1 per 13,844 live births (14.1 per million children under the age of 5) with approximately 300 children diagnosed per year (Stacey et al. 2021). Preserving life and preventing the loss of an eye, blindness and other serious effects of treatment that reduce the patient's life span or the quality of life, remains a challenge. In addition, children with retinoblastoma have been more likely to lose their eye and die of metastatic disease in low-resource countries.

About VCN-01

VCN-01 is a systemically administered oncolytic adenovirus designed to selectively and aggressively replicate within tumor cells and degrade the tumor stroma that serves as a significant physical and immunosuppressive barrier to cancer treatment. This unique mode-of-action enables VCN-01 to exert multiple antitumor effects by (i) selectively infecting and lysing tumor cells; (ii) enhancing the access and perfusion of co-administered chemotherapy products; and (iii) increasing tumor immunogenicity and exposing the tumor to the patient's immune system and co-administered immunotherapy products. Systemic administration enables VCN-01 to exert its actions on both the primary tumor and metastases. VCN-01 has been administered to over 140 patients to date in clinical trials of different cancers, including PDAC (in combination with chemotherapy), head and neck squamous cell carcinoma (with an immune checkpoint inhibitor), ovarian cancer (with CAR-T cell therapy), colorectal cancer, and retinoblastoma (by intravitreal injection). More information on these clinical trials is available at Clinicaltrials.gov.

About Theriva Biologics, Inc.

Theriva Biologics (NYSE American: TOVX), is a diversified clinical-stage company developing therapeutics designed to treat cancer and related diseases in areas of high unmet need. The Company's wholly-owned Spanish subsidiary Theriva Biologics, S.L., has been developing a new oncolytic adenovirus platform designed for intravenous (IV), intravitreal and antitumoral delivery to trigger tumor cell death, improve access of co-administered cancer therapies to the tumor, and promote a robust and sustained anti-tumor response by the patient's immune system. In addition to VCN-01, the Company's clinical-stage candidates include (1) SYN-004 (ribaxamase) which is designed to degrade certain commonly used IV beta-lactam antibiotics within the gastrointestinal (GI) tract to prevent microbiome damage, thereby limiting overgrowth of pathogenic organisms such as VRE (vancomycin resistant Enterococci) and reducing the incidence and severity of acute graft-versus-host-disease (aGVHD) in allogeneic hematopoietic cell transplant (HCT) recipients); and (2) SYN-020, a recombinant oral formulation of the enzyme intestinal alkaline phosphatase (IAP) produced under cGMP conditions and intended to treat both local GI and systemic diseases. For more information, please visit Theriva Biologics' website at www.therivabio.com.

Forward-Looking Statement

This release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. In some cases forward-looking statements can be identified by terminology such as "may," "should," "potential," "continue," "expects," "anticipates," "intends," "plans," "believes," "estimates," and similar expressions, and include statements regarding continuing to work closely with leading physicians and regulatory agencies to refine the Company's clinical strategy for VCN-01 as an adjunct to chemotherapy in pediatric patients with advanced retinoblastoma and the potential benefits acheivable from the grant of orphan driug designation. These forward-looking statements are based on management's expectations and assumptions as of the date of this press release and are subject to a number of risks and uncertainties, many of which are difficult to predict that could cause actual results to differ materially from current expectations and assumptions from those set forth or implied by any forward-looking statements. Important factors that could cause actual results to differ materially from

current expectations include, among others, the Company's ability to address the unmet medical needs for treatment of pediatrc retinoblastoma, the Company's ability to take advantage of the potential benefits of orphan drug designation, the Company's ability to reach clinical milestones when anticipated, the Company's product candidates demonstrating safety and effectiveness, as well as results that are consistent with prior results; the ability to complete clinical trials on time and achieve the desired results and benefits, continuing clinical trial enrollment as expected; the ability to obtain regulatory approval for commercialization of product candidates or to comply with ongoing regulatory requirements, regulatory limitations relating to the Company's ability to promote or commercialize their product candidates for the specific indications, acceptance of product candidates in the marketplace and the successful development, marketing or sale of the Company's products, developments by competitors that render such products obsolete or non-competitive, the Company's ability to maintain license agreements, the continued maintenance and growth of the Company's patent estate, the ability to continue to remain well financed and other factors described in the Company's Annual Report on Form 10-K for the year ended December 31, 2023 and its other filings with the SEC, including subsequent periodic reports on Forms 10-Q and current reports on Form 8-K. The information in this release is provided only as of the date of this release, and Theriva Biologics undertakes no obligation to update any forward-looking statements contained in this release on account of new information, future events, or otherwise, except as required by law.