TRACON Announces FDA Orphan Drug Designation for TRC102 in Malignant Glioma, Including Glioblastoma

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SAN DIEGO, Oct. 26, 2020 (GLOBE NEWSWIRE) -- TRACON Pharmaceuticals (NASDAQ:TCON), a clinical stage biopharmaceutical company focused on the development and commercialization of novel targeted cancer therapeutics and utilizing a cost efficient, CRO-independent product development platform to partner with ex-U.S. companies to develop and commercialize innovative products in the U.S., today announced that the U.S. Food and Drug Administration (FDA) granted TRC102 orphan drug designation for the treatment of patients with malignant glioma, including glioblastoma (GBM). TRC102 is a small molecule inhibitor of the DNA base excision repair pathway.

TRC102 is being studied in multiple Phase 1 and Phase 2 clinical trials sponsored by the National Cancer Institute through a Cooperative Research and Development Agreement (CRADA). TRC102 was evaluated in a Phase 2 trial in combination with Temodar chemotherapy in 19 patients with progressive or recurrent GBM who progressed following Temodar and external beam radiotherapy. Extended survival was observed in two patients for more than two years, both of whom demonstrated activation of the DNA base excision repair pathway and demonstrated hyperactivation of DNA damage response genes. TRC102 was also evaluated in combination with chemotherapy and external beam radiotherapy in a separate Phase 1 study of 15 patients with newly diagnosed non-squamous cell non-small cell lung cancer that resulted in a response by the Radiographic Evaluation Criteria in Solid Tumors (RECIST) in all patients, including three patients who had a complete response to treatment.

“Orphan drug designation for TRC102 underscores the high level of unmet medical need in patients with malignant glioma and supports its potential in this indication based on the previously reported data demonstrating prolonged survival in patients retreated with Temodar combined with TRC102,” said James Freddo, M.D, Chief Medical Officer of TRACON. “We remain committed to developing TRC102 in collaboration with the National Cancer Institute and believe that the data generated to date provide a rationale to study TRC102 in combination with Temodar and radiotherapy in newly diagnosed patients with malignant glioma.”

The FDA’s Orphan Drug Designation program provides orphan status to drugs and biologics that are being developed to address rare diseases or disorders that affect fewer than 200,000 people in the U.S. With orphan designation, TRACON qualifies for various incentives with respect to TRC102 for the treatment of glioblastoma, including tax credits for qualified clinical trials and seven years of market exclusivity upon receipt of regulatory approval.

About TRC102

TRC102 (methoxyamine) is a novel, clinical-stage small molecule inhibitor of the DNA base excision repair pathway, which is a pathway that causes resistance to alkylating and antimetabolite chemotherapeutics. TRC102 is currently being studied in multiple Phase 1 and Phase 2 clinical trials sponsored by the National Cancer Institute through a Cooperative Research and Development Agreement (CRADA). For more information about the clinical trials, please visit TRACON’s website at www.traconpharma.com/clinical-trials.php.

About Malignant Glioma and GBM

GBM (also called glioblastoma) is a fast-growing malignant glioma that develops from star-shaped glial cells (astrocytes and oligodendrocytes) that support the health of the nerve cells within the brain. GBM is the most invasive type of glial tumors, rapidly growing and commonly spreading into nearby brain tissue. The National Cancer Institute estimates that approximately 22,850 adults (12,630 men and 10,280 women) are diagnosed with brain and other nervous system cancer annually in the U.S. and approximately 15,320 of these diagnoses will result in death. GBM has an incidence of two to three per 100,000 adults per year in the U.S., and accounts for 52 percent of all primary brain tumors.

About TRACON

TRACON develops targeted therapies for cancer utilizing a capital efficient, CRO independent, product development platform. The Company's clinical-stage pipeline includes: Envafolimab, a subcutaneous PD-L1 single-domain antibody being developed for the treatment of sarcoma in a registrational trial in the U.S.; TRC253, a Phase 3 ready small molecule drug candidate for the treatment of prostate cancer; TRC102, a Phase 2 small molecule drug candidate in development for the treatment of lung cancer and glioblastoma; and TJ004309, a Phase 1 CD73 antibody in development for the treatment of advanced solid tumors. TRACON is actively seeking additional corporate partnerships whereby it leads U.S. regulatory and clinical development and shares in the cost and risk of clinical development and leads U.S. commercialization. In these partnerships TRACON believes it can serve as a solution for companies without clinical and commercial capabilities in the U.S. To learn more about TRACON and its product pipeline, visit TRACON's website at www.traconpharma.com.

Forward-Looking Statements

Statements made in this press release regarding matters that are not historical facts are “forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, statements regarding TRACON's and

the National Cancer Institute's plans to further develop product candidates, the potential benefits of orphan drug designation for TRC102, expectations regarding clinical trials, development and regulatory plans, the potential benefits of TRC102, and TRACON's business development strategy and goals. Risks that could cause actual results to differ from those expressed in these forward-looking statements include: risks associated with clinical development; whether TRACON or others will be able to complete or initiate clinical trials on TRACON's expected timelines, if at all, including due to risks associated with the COVID-19 pandemic or other pandemics; the fact that future preclinical studies and clinical trials may not be successful or otherwise consistent with results from prior studies; the fact that TRACON has limited control over whether or when third party collaborators complete on-going trials, initiate additional trials or seek regulatory approval of TRACON's product candidates; the fact that TRACON's collaboration agreements are subject to early termination; whether TRACON will be able to enter into additional collaboration agreements on favorable terms or at all; whether the expected benefits of orphan drug designation will ultimately be realized, including due to potential loss of orphan drug designation or the development of competing products; potential changes in regulatory requirements in the United States and foreign countries; TRACON's reliance on third parties for the development of its product candidates, including the conduct of its clinical trials and manufacture of its product candidates; whether TRACON will be able to obtain additional financing; and other risks described in TRACON's filings with the Securities and Exchange Commission under the heading "Risk Factors". All forward -looking statements contained in this press release speak only as of the date on which they were made and are based on management's assumptions and estimates as of such date. TRACON undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

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