vasopharm completes patient enrollment for Phase III Traumatic Brain Injury Trial (NOSTRA)

Top-line results expected end of 2020

Würzburg, Germany – January 29th, 2020: vasopharm GmbH, a privately held biopharmaceutical company focusing on novel therapeutics for the treatment of cerebrovascular diseases, is pleased to announce that enrolment into the pivotal European NOSTRA III (**NO S**ynthase in **TRA**umatic Brain Injury) clinical trial is completed. vasopharm remains on track to deliver headline data by end of 2020.

NOSTRA III is a 220 patient Phase III clinical trial assessing efficacy and safety of Ronopternin (VAS203) for the treatment of moderately to severely injured closed head traumatic brain (TBI) injury patients. The study is being conducted in 35 leading European trauma centres. In accordance with the study protocol which requires an evaluation of the extended Glasgow Outcome Scale (eGOS) six months post-trauma, the company anticipates last patient – last visit in June 2020 and full clinical data analysis by end of 2020. The full study protocol has been published in the peer-reviewed journal 'Trials'*.

Frank Tegtmeier, PhD, Chief Scientific Officer of vasopharm, commented; "We are extremely pleased to have completed enrolment into our pivotal study, NOSTRA III. Throughout the trial we have insisted on adherence to extremely rigorous inclusion-exclusion criteria which has resulted in slower recruitment than initially projected. I would like to thank the investigators and their teams in all of our study sites, for their huge commitment and dedication in helping us to reach this important milestone."

Christian Wandersee, Chief Executive Officer of vasopharm, added: "We are delighted to have reached such a significant milestone. We look forward to delivering top line results which we hope will confirm the highly significant data seen in our NOSTRA II trial. This will allow us to deliver an essential therapeutic option to both physicians and patients who experience the devastating and life-long consequences of TBI, for which there is no existing drug treatment."

Traumatic Brain Injury is the leading cause of death and disability among young adults in the developed world. Annually, within the US alone, head trauma is the cause of about two million emergency room visits, roughly 475,000 hospital admissions, nearly 52,000 deaths and approximately 80,000 cases of severe long-term disability (e.g. functional and cognitive disorders, learning disabilities). Direct costs attributed to the treatment of TBI exceed $10bn p.a. in the US alone.

**Efficacy of Ronopterin (VAS203) in patients with moderate and severe traumatic brain injury (NOSTRA phase III trial): Study protocol of a confirmatory, placebo controlled, randomised, double-blind multi-centre study.* *https://trialsjournal.biomedcentral.com/articles/10.1186/s13063-019-3965-4*

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NOTES TO EDITORS

About vasopharm GmbH:
vasopharm is a drug development company focused on small molecule therapeutics for treatment of Traumatic Brain Injury (TBI). vasopharm's lead drug candidate VAS203 (Ronopterin) is currently in the Phase III NOSTRA trial assessing efficacy and safety of VAS203 for the treatment of moderately to severely injured closed head traumatic brain injury patients.

Current approaches to the treatment of acute TBI focus on short term patient mortality and have no significant impact on reducing long-term physical and cognitive deficits. VAS203 avoids unwanted side effects, combined with unprecedented clinical efficacy. The European regulator, EMA has granted the drug orphan drug designation for moderate to severe brain injury.

vasopharm was founded in July 1998 as a spin-off from the University of Würzburg Medical School by Harald Schmidt, MD, PhD, Professor of Pharmacology and Toxicology, and Ulrich Walter, MD, Professor of Clinical Biochemistry and Pathobiochemistry. To date, the company has raised about Euros 50 million to date through several financing rounds from various Family Investment Offices and Venture Investors.
See www.vasopharm.com for further details.

About Traumatic Brain Injury:
Traumatic brain injury (TBI) occurs when a sudden trauma causes damage to the brain. Every year, over 1,600,000 patients sustain a traumatic brain injury in the EU, and 70,000 of these die, with a further 100,000 being left disabled. Significantly, 75% of the victims are children and young adults, and TBI is the leading cause of disability in people under 40 years of age. Traumatic brain injury results in more lost working years than cancers, stroke and HIV/AIDS together. On a global scale, the number of life years lost due to traumatic brain injury is four times that of diabetes-related loss. Recent statistics show a steep increase in the incidence of TBIs, with an increase of 21% over the last five years – threefold greater than the rate of increase in population, at an annual cost of over Euros 100 billion. Despite this, TBI has been seriously under-represented in medical R&D efforts compared to many other, less significant health problems.[1]

About VAS203:
VAS203 is an analogue of the natural co-factor tetrahydrobiopterin, which is involved in the generation of nitric oxide by the Nitric Oxide Synthase (NOS) family of enzymes. The mechanism of action of VAS203 is believed to confer selective down regulation of inducible NOS (iNOS) without significantly inhibiting the function of other NOS enzymes. It is believed that iNOS has a significant involvement in the cascade of damaging sequelae following a traumatic brain injury. Technical: VAS203 is (4-amino-(6R,S)-5,6,7,8-tetrahydro-L-biopterin dihydrochloride dihydrate) a structural analogue of (6R)-5,6,7,8-tetrahydro-L-biopterin, the natural endogenous cofactor of NOS and phenylalanine hydroxylase.

About NOSTRA III
NOSTRA III is a randomised, double-blind, placebo-controlled trial. The study will enroll 220 patients who have suffered a moderate to severe TBI resulting in hospitalisation and who have received an intra-cranial pressure probe. Currently, 35 European neuro-trauma centres in Germany, Austria, France, UK and Spain are participating. The primary endpoint is the eGOS evaluated at six months after the injury. Secondary efficacy assessments include Quality of Life (QOLIBRI) as well as Therapy Intensity Level (TIL) over 14 days after brain injury. NOSTRA III is a registration trial which seeks to validate the data from the highly significant NOSTRA II trial.[2] The study aims to confirm the data from a Phase II trial in which VAS203 has shown a (2 point) improvement in the extended Glasgow Outcomes Score.

[1.] *www.tbicare.eu*
[2.] *Journal of Neurotrauma 31:1599-1606 (October1 2014).*