



Basel, Switzerland  
August 22<sup>nd</sup>, 2022

## Vaderis Therapeutics AG Emerges from Stealth and Announces Initiation of Clinical Proof-of-Concept Trial in HHT

- **Focused on developing treatments for rare diseases associated with vascular malformations**
- **Recruited first patients in Proof-of-Concept study for lead drug candidate VAD044**
- **Financed by Medicxi in 2020**

**Basel, Switzerland. August 22, 2022** – Vaderis Therapeutics AG (Vaderis), a clinical stage biotechnology company focused on developing treatments for rare diseases associated with vascular malformations, today announced its emergence from stealth and initiation of its INSIGHT proof-of-concept clinical trial in patients suffering from Hereditary Haemorrhagic Telangiectasia (HHT), also known as Osler-Weber-Rendu Syndrome. Vaderis was established in 2019 and in 2020 raised over CHF18 million from Medicxi, enabling progression of its lead asset into the clinic and successful completion of Phase 1a.

### **Emergence from Stealth**

Vaderis was founded with the unique aim to develop therapeutics for rare and orphan diseases associated with vascular malformations. In April 2020 Vaderis acquired a portfolio of allosteric AKT inhibitors from Almac Discovery Ltd of Belfast, UK. AKT is a serine kinase which plays a crucial role in vascular growth and in vascular overgrowth. There is a significant number of rare diseases such as HHT in which upstream genetic mutations trigger over-activation of the serine kinase AKT, resulting in vascular overgrowth. Such diseases frequently manifest in ways that are debilitating, disfiguring, painful and sometimes life-threatening. The vast majority of these diseases are currently left untreated except for symptomatic interventions which tend to offer patients limited temporary relief of individual symptoms. VAD044 is a once daily, orally administered, allosteric AKT inhibitor which has the potential to treat the underlying cause of these diseases. If successful, Vaderis would be the first company in the world to develop a medicine for the treatment of HHT and other diseases associated with vascular malformations.



### **Initiation of Clinical PoC in HHT**

In pursuit of the company's core objective, Vaderis announces the initiation of the first study of VAD044 in HHT patients. Known as the INSIGHT proof-of-concept trial (INternational Study InvestiGating HHT), the study is unique in its robust design aimed at achieving a thorough understanding of the safety of VAD044 in HHT patients. A total of 80 HHT patients across the USA, Canada and Europe will be randomised in a double-blind, controlled trial comparing two doses of VAD044 to placebo. Initiation of the INSIGHT trial follows Health Authority approvals including FDA, Health Canada and key European agencies.

J.J. Mager, MD, PhD, pulmonologist at St. Antonius Hospital Utrecht, Chairman of the Global Research and Medical Advisory Board of Cure HHT and lead investigator of the INSIGHT trial commented, "The first patients recruited into the INSIGHT trial mark an exciting milestone in HHT clinical research. If successful, this trial may demonstrate the potential of this new drug to be effective beyond the symptomatic treatments we currently offer HHT patients, by addressing the course of this rare, progressive, and debilitating disease."

Nicholas Benedict, CEO of Vaderis added, "Working together with the HHT community, both caregivers and patient associations, Vaderis hopes to be the catalyst which transforms patient care in HHT. Success in the INSIGHT trial would be a major step towards developing the first ever registered treatment for HHT anywhere in the world."

Giovanni Mariggi, Partner at Medicxi and Chairman of Vaderis said, "Our vision is to create a unique company dedicated to delivering breakthrough therapies for rare diseases caused by vascular malformations. Initiation of the INSIGHT trial is an important milestone on the way to realising this vision. It is testament to the significant progress made by the company over the last two years".

- Ends -

### **About Vaderis**

Vaderis is a clinical stage biotech company with the aim to develop medicinal treatments for rare and orphan diseases associated with vascular malformations. Since its founding in 2019, Vaderis raised a Series A financing from Medicxi and acquired a portfolio of allosteric AKT inhibitors from Almac Discovery Ltd of Belfast, UK. There is a significant number of debilitating and largely untreated rare diseases, such as HHT (Hereditary Haemorrhagic Telangiectasia), in which patients suffer from over-activation of AKT triggered by upstream genetic mutations resulting in vascular overgrowth. There are no drugs approved anywhere in the world which specifically



treat HHT. Vaderis aims to be the first company to develop a medicine for the treatment of HHT and aims to treat other diseases associated with vascular malformations.

**About Medicxi**

Medicxi is a healthcare-focused investment firm with the mission to create and invest in companies across the full drug development continuum. Leveraging deep expertise in drug development and company creation spanning over two decades, Medicxi invests in early and late-stage therapeutics with a product vision that can fulfill a clear unmet medical need. For more information, please visit: <https://www.medicxi.com>

**For further information please contact**

Vaderis Therapeutics AG

Nicholas Benedict

[info@vaderis.com](mailto:info@vaderis.com)

[www.vaderis.com](http://www.vaderis.com)