



EXECUTIVE SUMMARY – QUERCIS PHARMA AG 18 DEC 2023

- **Lead indication:** Prevention of thrombosis in cancer patients
- Next to cancer itself, thrombosis is the *second most important* reason for mortality in these patients affecting *20-30% of all cancer patients with solid tumors*. The company's lead drug candidate is an anti-thrombotic vs an anticoagulant and has been shown to be extremely safe in Phase 2 studies in prevention of thrombosis in high-risk cancer patients as well as in steady state Sickle Cell Disease patients.
- **Unmet Need:** Thrombosis in cancer patients represents an area of *high unmet medical need* as current anticoagulants (e.g., *factor 10a* and *factor 11a's* have not yet surpassed the efficacy of *factor 10a* blockers) cause significant increased risk for bleeding and are not suitable for prevention and therefore prophylactic use. In addition, just recently Bayer's *factor 11* asset **failed** in phase 3 study. BMS is also currently running clinical trials with a separate *factor 11* asset.
- Thrombosis in general represents an area of high unmet medical need with high socio-economic burden and no adequate therapy available.
- **Technology:** *Kinisoquin™* (IP protected combination of Isoquercetin with vitamin B3 and C and optionally folate in a later version) is a novel anti-thrombotic with a very distinct MoA through *blocking of PDI* (protein disulfide isomerase) that has a *significantly lower risk of bleeding* addressing this significant unmet need.
- Efficacy has been *proven in a phase 2 clinical trial* in prevention of thrombosis in multiple solid tumors (Pancreatic, NSCL and Colorectal) as well as being confirmed in a further phase 2 study in SCD (sickle cell disease) conducted by NIH in collaboration with Quercis.
- **Next steps:** There is a *well-defined development* plan in place with an *FDA agreed SPA* – (so *Special Protocol Agreement* vs assessment) will lead to two small phase 3 trials in two different cancer types – leading to a broad *prevention of thrombosis in all solid tumor-based cancers* label.
- **Market Potential:** Asset in its *lead indication* prevention of thrombosis in cancer patients has *Blockbuster* potential with *US\$ multi bn* sales as well as a *significant upside potential* should already observed *anti-neoplastic effects* translate into better outcome for cancer patients as well as other large LCM opportunities (SCD (sickle cell disease); ALS; anti-viral properties)
- **IP:** Broad and *strong IP protection* up to *2042* (not including potential IP extensions). IP lawyers are DLA Piper (one of the best IP Lawyers worldwide)
- **Experience:** Small, *very experienced team* supported by a Scientific Advisory Board with all the *key thought leaders* in the field engaged
- **Next inflection Point:** Phase 3 interim analysis in the prevention of thrombosis in cancer patients – 12 to 18 months out, with a clearly defined exit strategy with a strategic partner. There are a number of potential strategic partners engaged. Phase 3 ready for SCD.
- **Additional possibilities:** the Company has patent protection for a repurposed drug, Zafirlukast, both alone and in combination with Kinisoquin™ and certain other cancer drugs. The Company also has a program in ALS where very promising pre-clinical data exists. The company's lead drug candidate has also been shown to reduce resistance to and possibly re-sensitize some off patent drugs for different cancers and as such could result in new patents in combination with some off-patent medications.
- **Summary:** The company has 3 Phase 3 ready assets with blockbuster potential, each of which are expected to generate Billions of Dollars per annum at peak sales. A unique situation in Pharma globally.

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