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Roquefort Therapeutics PLC  
07 June 2022

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**Roquefort Therapeutics plc**  
("Roquefort Therapeutics" or the "Company")

**Pre-clinical Update**

Roquefort Therapeutics (LSE:ROQ), the Main Market listed biotech company focused on early-stage opportunities in the biotechnology sector, is pleased to provide an update on progress on its a pre-clinical Midkine targeting drug development programs.

During the first half of 2022, the Company has been focused on:

- Progressing the collaborative work with Murdoch University, Western Australia to design and test a novel series of gene silencing reagents, antisense oligonucleotides, targeting Midkine; and
- Portfolio review including the Midkine antibody programs acquired from Lyramid Ltd ("Lyramid") in December 2021 to assess the commercial potential of these programmes; and
- Strengthening the Board by appointing industry experienced professionals.

**Antisense Oligonucleotide Program**

During 2022 the Company has continued its collaboration with Professor Steve Wilton's group at Murdoch University, Perth, Western Australia to design and test a novel series of gene silencing reagents, antisense oligonucleotides, targeting Midkine. These oligonucleotide drugs interfere with processing of the Midkine mRNA ultimately leading to reduced active Midkine protein produced in diseased tissues and tumours.

As announced on 17 January 2022, after design and selection of lead oligonucleotide drug candidates, the Company commenced *in vitro* screening in cancer cells. These *in vitro* experiments generated very positive results demonstrating that the Company's lead oligonucleotide drug candidates significantly reduce Midkine mRNA levels seen in human cancer cells, which is in line with initial pre-test expectations.

As announced on 21 March 2022, in order to protect the IP and the significant potential value of this new class of RNA therapeutic drugs, the Company filed for its first composition of matter provisional patent application covering antisense oligonucleotide drugs to block the action of Midkine, a very important milestone for the Company.

During Q2 2022 the collaborative pre-clinical development program work at Murdoch University has focused on the generation of modified Midkine mRNA. The Company has developed a patented family of proprietary oligonucleotides which achieve 90% splice switching and most recently demonstrated the ability to modify Midkine mRNA in *in vitro* cancer cell culture models.

The Company is now planning to commence collaborative work with leading cancer researchers to assess the ability of the novel Midkine inhibitors to modify *in vitro* tumour cell behaviour as a prelude to testing the efficacy of the lead oligonucleotide drug candidates using validated *in vivo* models of solid tumours.

The Company will update the market on the progress of these key programs in due course.

#### **Antibody Therapeutic Program**

During 2022 the Company carried out a strategic and scientific review of the antibody therapeutic programs undertaken by Lynamid prior to the acquisition in December 2021. As a result of this portfolio review, the Company has concluded that there are two potentially high commercial value antibody programs (ROQA1 and ROQA2) in the advanced stages of pre-clinical development.

Both ROQA1 and ROQA2 antibody programs demonstrated significant anti-cancer activity in validated *in vivo* models of metastatic tumours. The results showed a significant impact in metastatic breast cancer (P

Humanised antibodies have been manufactured for both programs, with ROQA2 being successfully tested in both rodent and a non-human primate GLP toxicology/pharmacokinetic studies. On the basis of these positive results and the early commercial potential to be first to market with an anti-Midkine oncology antibody, the Company is accelerating development of the ROQA1 and ROQA2 antibody programs which remain on track for CTA/IND filing in late 2023.

The Company will update the market on the progress of these two advanced antibody pre-clinical development programs in due course.

#### **Stephen West, Executive Chairman, said:**

*"It has been a very active start to the year during which we have made significant progress. The collaboration with Murdoch University is yielding encouraging results*

*with the Company's lead oligonucleotide drug candidates. In addition, we are delighted that the review of the wider portfolio acquired with Lyramid has resulted in the conclusion that two of the antibody programs have high commercial potential and we will therefore be accelerating development of those as we aim to be the first to market with an anti-Midkine oncology antibody. These are exciting developments that broaden the commercial potential within the Company and enhance the upside value that we believe can be delivered as we progress the programs."*

**-Ends-**

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For further information on Roquefort Therapeutics, please visit [www.roquefortplc.com](http://www.roquefortplc.com) and @RoquefortTherap on Twitter.

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**About Roquefort Therapeutics**

Roquefort Therapeutics (LSE:ROQ) is a Main Market listed biotech company developing products through the pre-clinical phase prior to partnering or selling to big pharma. The Company is a pre-clinical biotech company focused on developing first in class Midkine targeting drugs for the treatment of cancer.

Through extensive research resulting in validation through publication in over 1,000 scientific journals, Roquefort Therapeutics has identified the potential to exploit the broad therapeutic potential of Midkine for a number of clinical indications of unmet needs. Roquefort Therapeutics holds an exclusive licence to the largest global IP portfolio on Midkine. The Midkine blocking drug development markets have significant global market potential (in the multi-billion dollars). Roquefort Therapeutic's pre-clinical program is currently underway with an initial focus on cancer.

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Company Announcement - General

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