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Roquefort Therapeutics PLC  
21 March 2022

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

**Patent Filed - New Drug Class Targeting Midkine**

*Very positive pre-clinical trials lead to filing for Midkine patent*

Roquefort Therapeutics (LSE:ROQ), the Main Market listed biotech company focused on early-stage opportunities in the biotechnology sector, is pleased to announce that the Company has filed its first composition of matter provisional patent application for a new class of RNA therapeutic drugs targeting Midkine.

In 2021 the Company entered into a 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. Prof. Wilton is a pioneer in this gene silencing technology, having gained FDA approval for one of the first RNA therapeutic drugs of this style for the treatment of Duchenne's Muscular Dystrophy.

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 have 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.

In order to protect the IP and the significant potential value of this new class of RNA therapeutic drugs, Roquefort Therapeutics has 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.

The broad coverage of this composition of matter provisional patent application will ultimately afford extensive IP protection for the development of Midkine-based drugs using this novel technology, paving the way for subsequent methods patents covering the use of the Midkine antisense oligonucleotides in

different clinical indications.

The use of novel Midkine antisense oligonucleotides will ultimately block the action of Midkine in driving various disease processes involved in cancer, chronic inflammation and autoimmune disorders.

The Company is now firming up collaborations with leading medical research institutes in order to assess the lead oligonucleotide drug candidates efficacy in altering tumour cell behaviour and in vivo growth of tumours in mouse models of cancer, an essential next step in the drug discovery process.

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

*"We are extremely delighted with the positive results of the pre-clinical trials, which has been a collaboration between the Company and internationally recognised leaders in this specific style of RNA therapeutic drugs. The findings have given us encouraging signs for the future of Midkine-based drugs in treating a variety of disease processes, associated with cancer, chronic inflammation and autoimmune disorders - each of which are multi-billion dollar drug markets. The filing of this provisional patent application is an exciting step and a notable milestone for the Company as we progress with the next stage of efficacy testing. Roquefort Therapeutics looks forward to updating shareholders as we make further progress."*

**-Ends-**

**Enquiries:**

**Roquefort Therapeutics plc**

Stephen West (Chairman) +44 (0)20 3290  
9339

**Buchanan (Public Relations)**

Ben Romney / Jamie Hooper / George Beale +44 (0)20 7466  
5000

**Optiva Securities Limited (Broker)**

Christian Dennis +44 (0)20 3411  
1881

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 focused on developing first in class Midkine inhibiting RNA therapeutic drugs for the treatment of cancer, chronic inflammatory, autoimmune disorder and COVID-19. Recent progress within mRNA therapeutics has led to a reduction in drug development timelines and costs, increasing the chance of early value creation.

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

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Company Announcement - General

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