Antibody Program Results & Orphan Drug Indication
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Roquefort Therapeutics PLC
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("Roquefort Therapeutics" or the "Company")

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Roquefort Therapeutics (LSE:ROQ), the Main Market listed biotech company focused on developing first-in-class medicines in the high value and high growth oncology market, is pleased to announce the progress of its anti-cancer antibody programs targeting Midkine expressing cancers.

Following the announcement in January 2023 where the Company announced the Midkine antibody programs, targeting metastatic breast cancer and metastatic lung cancer, successfully demonstrated *in vivo* safety, Roquefort Therapeutics has continued the development of its antibody portfolio with the Company's research partners at La Trobe University. The Company is now releasing the first *in vivo* efficacy results for its anti-Midkine antibodies CAB-101 (ROQA2) and CAB-102 (ROQA1), including a new program for an osteosarcoma orphan drug indication.

CAB-101 and CAB-102 are the lead programs in the Company's family of patented humanised antibodies, which were developed in-house, to target Midkine-expressing solid cancers. The *in vivo* efficacy study tested the anti-cancer killing ability of CAB-101 and CAB-102 in a validated experimental model of osteosarcoma. Treatment with CAB-101 was found to produce a statistically significant reduction in lung metastasis, and CAB-102 was found to reduce proliferation (growth rate) of the primary tumour. The more detailed experimental results remain under embargo pending publication at a leading cancer research conference.

CAB101 and CAB102 treat solid cancers, the market for which was estimated to be $32 billion in 2021 and growing with a 7.8% CAGR[1]. Osteosarcoma is the Company's first orphan drug indication and reflects the strategic decision to target cancer niches in which, there remains a high unmet clinical need (24% to 64% 5-year survival rate)[2], an accelerated development pathway[3] and the potential to offer a best-in-class treatment in a significant market niche.

Medicines to treat an orphan drug indication may be awarded market exclusivity for 7 years in the USA and 10 years in the EU and UK[4], tax credits for the clinical drug testing cost, fee reductions and, on average, will have a significantly higher success rate in clinical trials[5].

Osteosarcoma is the eighth-most common form of childhood cancer, comprising 2.4% of all malignancies in paediatric patients, and about 20% of all primary bone cancers[6], with a market estimated to reach $790 million by 2025 (6% CAGR)[7]. Midkine is over-expressed in osteosarcoma and the level of Midkine expression is correlated with poor prognosis[8] and therefore, represented a promising target for anti-Midkine medicines.

Commenting on the antibody program results, Ajan Reginald, Chief Executive Officer, said:

"*We decided to target metastatic osteosarcoma because of the high unmet medical need. There is a very poor 5-year survival rate of just 24%, and this poor survival is associated with over-expression of Midkine. Our rationale was that by targeting Midkine, we would be able to attack both primary tumours and metastasis. The in vivo results vindicate this approach and demonstrate the potential for a best-in-class medicine. We look forward to providing further information once the detailed results have been published.*

This strategy to focus on areas of high unmet medical need is underpinned by the orphan drug incentives (including market exclusivity, efficient clinical trials and tax incentives) and the higher market valuation and return on investment for the investors in companies with approved orphan medicines. Finally, this approach seems to reduce risk, with significantly higher clinical trial success rates particularly in phase 1 and 2 trials particularly with a biomarker, which in our case is Midkine.

Therefore, this is a particularly promising scientific and commercial strategy which was delivered on time and on budget. We expect to announce more updates on our clinical progress and business development activities over the next quarter."

-ENDS-

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About Roquefort Therapeutics

Roquefort Therapeutics (LSE:ROQ) is a Main Market listed biotech company developing first in class drugs in the high value and high growth oncology segment prior to partnering or selling to big pharma.

Since listing in March 2021, Roquefort Therapeutics has successfully acquired Lyramid Pty Limited, a leader in the development of medicines for a new therapeutic target, Midkine (a human growth factor associated with cancer progression), and most recently acquired Oncogeni Ltd, founded by Nobel Laureate Professor Sir Martin Evans, which has developed two families of innovative cell and RNA oncology medicines.

Roquefort Therapeutics' portfolio consists of five fully funded, novel patent-protected pre-clinical anti-cancer medicines. The highly complementary profile of five best-in-class medicines consists of:

- Midkine antibodies with significant *in vivo* efficacy and toxicology studies;
- Midkine RNA therapeutics with novel anti-cancer gene editing action;
- Midkine mRNA therapeutics with novel anti-cancer approach;
- STAT-6 siRNA therapeutics targeting solid tumours with significant *in vivo* efficacy; and
- MK cell therapy with direct and NK-mediated anti-cancer action

For further information on Roquefort Therapeutics,
please visit www.roquefortplc.com and @RoquefortTherap on Twitter.

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[1] https://finance.yahoo.com/news/solid-tumor-testing-market-worth-103300895.html

[2] https://www.cancer.net/cancer-types/osteosarcoma-childhood-and-adolescence/statistics#:~:text=If%20the%20cancer%20has%20

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[3] https://www.medpace.com/wp-content/uploads/2023/03/Article-Regulatory-Strategies-and-Considerations-for-Orphan-and-Pediatric-Drug-Designations.pdf

[4] https://www.propharmagroup.com/thought-leadership/orphan-drug-designations-in-the-u-s-and-eu

[5] https://www.sciencedirect.com/science/article/pii/S0040595720300093

[6] https://www.marketwatch.com/press-release/osteosarcoma-market-growth-2023-2030-2023-05-09

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[8] Biochem Biophys Res Commun 2007 Jul 6;358(3):757-62. doi: 10.1016/j.bbrc.2007.04.183. Epub 2007 May 7

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