ROQUEFORT
THERAPEUTICS PLC

# MATERIAL BIOTECH COMPANY FOCUSED ON NEXT GENERATION CANCER MEDICINES

July 2023

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# Snapshot of Roquefort Therapeutics

## Company History

- Established in 2020 and listed on the London Stock Exchange in March 2021
- In December 2021, acquired Lyramid Pty Ltd, a leader in the development of medicines for a new therapeutic target, Midkine
- In September 2022, acquired Oncogeni Ltd which has developed two families of innovative cell and RNA oncology medicines
- In February 2023, signed diagnostic licencing deal with Randox

## Innovative Pre-Clinical Portfolio

Five fully funded, novel patent-protected anti-cancer medicines:

1. Orphan drug MDK antibodies, significant *in vivo* efficacy and toxicology studies;
2. MDK RNA therapeutics, novel anti-cancer gene editing action;
3. MDK mRNA therapeutics with novel anti-cancer approach;
4. Mesodermal Killer (MK) cells, new class of cellular medicine engineered to kill cancer both directly and by enhancing the activity of Natural Killer cells; and
5. Novel siRNAs (small interfering RNA) inhibit STAT-6, to kill solid tumours.

## Leadership Team & Facilities

- Leadership team with track record of taking medicines through pre-clinical development, winning regulatory approvals and commercialisation
- Prof. Sir Martin Evans, Nobel Laureate and Ajan Reginald >12yrs Biotech CEO with big pharma experience
- State-of-the-art laboratory and bio-pharmaceutical GMP manufacturing facilities

## Significant Valuation Potential

- Potential to meet significant value inflection with average valuation of biotech companies with a single lead asset completing pre-clinical development was circa US$71 million (£55 million, 2005-2020)* - we have five!
- Oncology market forecast to surpass $353 billion by 2023 with a CAGR of 8.4% in 2022-2023**
- Out licencing strategy to achieve early realised value
- Focused pathway to create significant value



*Therapeutic Innovation & Regulatory Science (2022) 56:313–322 https://doi.org/10.1007/s43441-021-00364-y

** https://www.globenewswire.com/en/news-release/2023/01/24/2594388/0/en/Global-Cancer-Therapy-Market-to-Surpass-US-353-5-Billion-by-2030-Says-Coherent-Market-Insights-CMI.html

# Board and leadership team

## Executives

**Stephen West:** Executive Chairman & Founder

- Fellow Chartered Accountant with over 26 years' international financial, corporate and public company experience
- Proven track record in working with growth companies with extensive experience in IPOs, secondary listings, corporate finance & fundraising

**Ajan Reginald:** Chief Executive Officer

- 20 years in BioPharma as a Biotech CEO and senior executive in public companies Roche (Global Head) and at Novacyt (COO & CTO) during the COVID-19 pandemic
- Track record of discovering and developing new medicines and diagnostics & value creation
- Experimental Medicine MSc, University of Oxford; AMP, Harvard Business school; Kellogg MBA (Fulbright scholar) and Boston Consulting Group

**Prof. Sir Martin Evans:** Group Chief Scientific Officer

- First scientist to identify embryonic stem cells
- Nobel Laureate
- Copley Medal, Royal Society & Gold Medal, Royal Society of Medicine
- FRS, FMedSci

Independent Non-Board

**Prof. Armand Keating:** Chief Medical Advisor

- Distinguished physician with over 40 years experience in cancer medicine
- Past President of the American Society of Hematology
- Professor of Medicine, University of Toronto
- MD, PhD and leading expert in the development of novel cancer drugs
- Track record of developing novel medicines through pre-clinical phases

## NEDs

**Dr Darrin M Disley OBE:** Non-Executive Director

- Renowned scientist entrepreneur & former CEO of Horizon Discovery Group plc for 11 years, where he led the Company from start-up through a US$113M IPO
- PhD (University of Cambridge) DSc (Salford), QAEP, OBE

**Dr Simon Sinclair:** Non-Executive Director

- Over 15 years' pharma and medtech industry experience in translational medicine, clinical development, medical affairs and safety, vigilance and real-world evidence
- Chief Safety Officer, Reckitt Benckiser Group PLC & Executive Director, Reckitt Global Hygiene Institute (RGHI)
- Senior positions at DePuy Synthes, Johnson and Johnson, and Merck Inc.,
- MB BChir PhD (University of Cambridge)

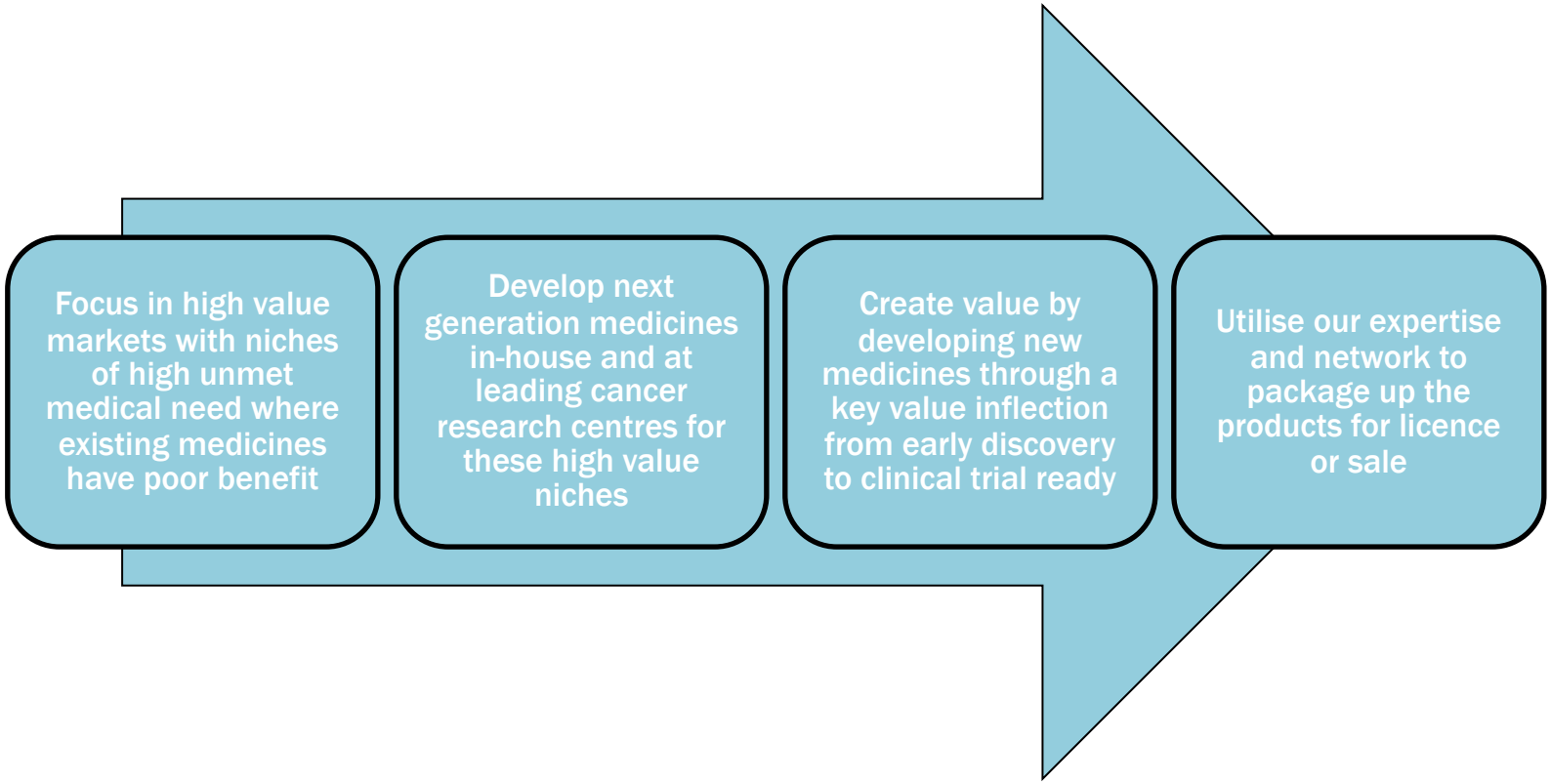
**Dr Michael Stein:** Non-Executive Director

- Founder of Doctor Care Anywhere, acquired by Synergix and Map of Medicine Ltd (the Map) licensed by NHS and acquired by Hearst; founding CEO of Valo Therapeutics and OxStem Ltd
- Medical doctor (Honours) and biochemist (First Class Honours) from the University of Cape Town (1988) and from the University of Oxford (Rhodes Scholar) with a doctorate in Physiological Sciences (Immunology)

**Jean Marie Duvall:** Non-Executive Director

- CEO & Director at Repronovo SA; Director, Executive VP & Group General Counsel at Ferring International Center
- Former Co-Chair of FerGene, Inc., Director & Chair-Cell & Gene Therapy at Trizell Holding SA, Director & Executive VP at Ferring Pharmaceuticals, Inc., General Counsel for Elan Corp. Plc and Director at Amzell
- Graduate degree from The Ohio State University and an undergraduate degree from Case Western Reserve University.

# Value creation strategy




- Paradigm in biotech/pharma is that Biotech companies discover and develop new medicines which are acquired by Big Pharma. Big Pharma is cash rich and acquires new products by licensing from or acquiring Biotech companies.
- Our model is to create value by finding very early innovation in high value niches and developing it, to create the package required by Big Pharma for a licence / acquisition.
- This is a sustainable business model which creates significant value

# Significant R&D and Commercial Progress

## H1 2023 – positive strides across portfolio

## H2 2023 strategy

**Out-licensing to strategic partners:**
- Diagnostics to Randox
- Therapeutics to Big Pharma

**Developed R&D programs**

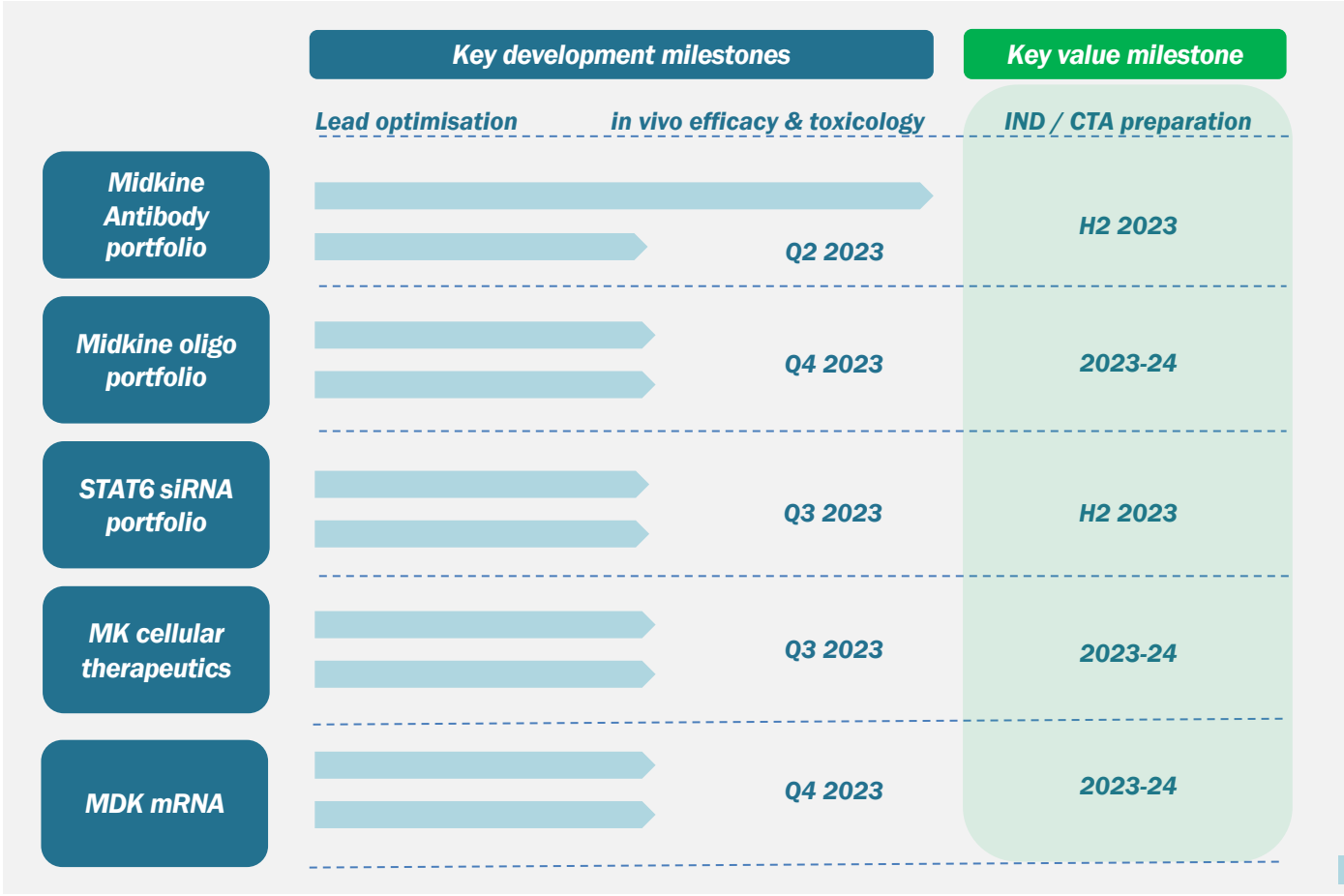
- Olivia Newton John Institute
- Lowry cancer institute
- Hawkins laboratory
- Sydney Uni Medical school
- Toronto Uni Keating lab

**Internal R&D and drug delivery development**
- siRNA & Midkine oligo delivery systems

- Antibody in vivo studies
- mRNA + LNP studies in liver / breast
- Oligonucleotide + LNP in liver / breast
- sIRNA + LNP in colon cancer
- MK cell + NK cell in lymphoma / leukemia

# R&D program progress reporting

## Anticipated development timeline (subject to change)




Represents drug candidates

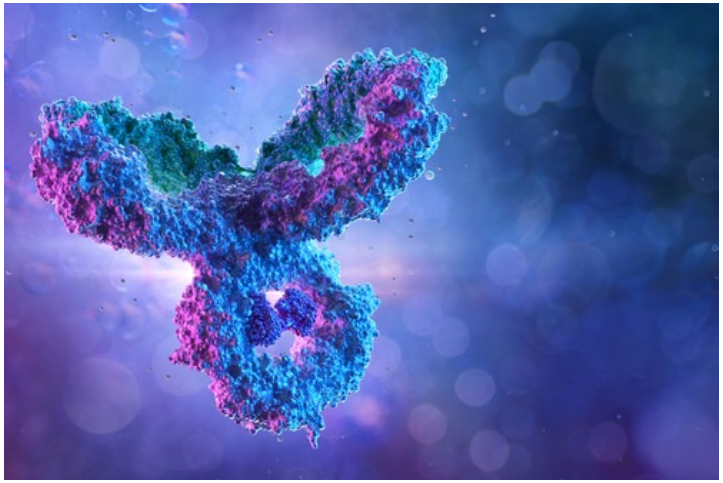
# 1 Midkine Antibodies

## Midkine antibody family shows *in vivo* efficacy in validated animal models

- Midkine family of antibodies includes four novel patent protected antibodies (IP9,10,13 & 14)
- In a validated animal model, CAB101 antibody reduces lung metastasis ($p<0.5$)
- CAB102 completed GLP toxicology studies in 2 species & recently showed efficacy in osteosarcoma

## Osteosarcoma: rare bone cancer designated as an Orphan disease (<200k p.a patients in US)

- Orphan drug scheme: US, EU & UK regulated program to incentivise new medicines for rare diseases
- Commercial incentives:
  - Market exclusivity: 7 years USA; 10 years EU
  - Reduced costs: clinical trial tax credits & fee reductions
- Faster lower risk drug development:
  - Higher success rate in clinical trials
  - Smaller trial size
  - Faster: 5 years



# 2 Midkine Oligonucleotides

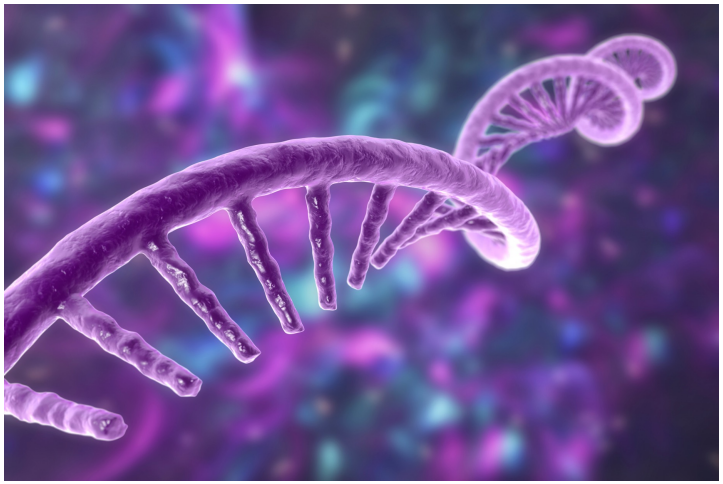
## *in vitro* study demonstrated ROQ's proprietary oligo reduces cancer Midkine

- Midkine (MDK) family of oligonucleotides includes 4 novel patent protected RNA sequences
- Lead oligonucleotide drug candidates significantly reduces MDK mRNA levels
- Truncated MDK shows efficacy in validated animal model
- Patent protected with composition of matter IP
- Highly complementary approach to antibodies to produce an anti-cancer MDK portfolio

# 3 Midkine mRNA

## *in vitro* study demonstrated ROQ's proprietary oligo reduces cancer Midkine

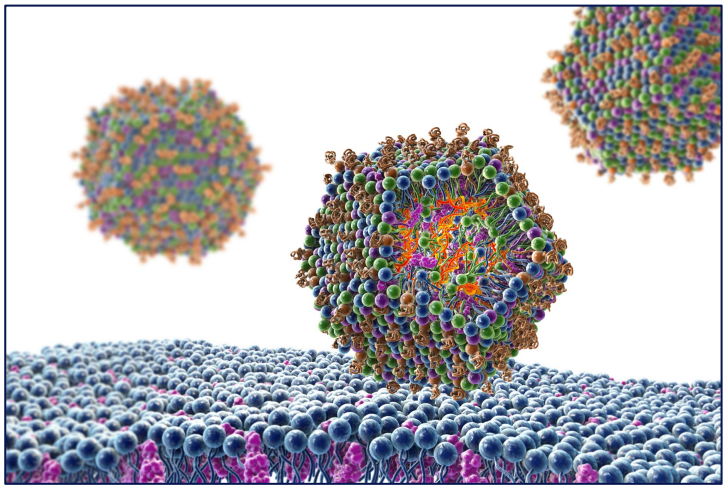
- mRNA family includes two novel patent protected mRNA sequences
- mRNA significantly reduces MDK mRNA levels in *in vitro* breast and liver cancer models
- Patent protected with composition of matter IP
- Highly complementary approach to antibodies to produce an anti-cancer MDK portfolio
- Anti-cancer mRNA is very attractive field ($31 billion, 7.8% CAGR) in Biotech with few competitors and high deal values



# 4 STAT-6 siRNA

## siRNA targets STAT-6: novel cancer target prevalent in cancers with high mortality

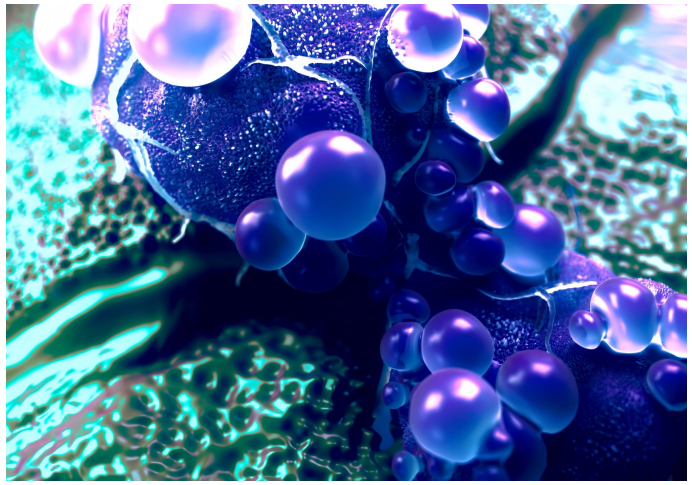
- STAT-6 is an intracellular target, that is not druggable with conventional medicines, that is implicated in cancer development, progression, metastasis and resistance to treatment
- siRNA (small interfering RNA) inhibits STAT6 driving cancer cell death and slowed growth
- siRNA showed significant anti-cancer activity (*p<0.05) *in vivo* in validated animal models
- Combination of siRNA with LNP being tested in colon cancer



# 5 MK cellular therapeutic

## MK are a novel, human cell engineered to kill cancer

- Novel engineered anti-cancer cell type invented by Nobel Laureate, Prof. Sir Martin Evans
- Engineered to kill cancer directly, attract NK cells and to activate (prime) NKs to kill cancer
- Designed to be well tolerated with a low risk of serious side effects associated with CAR-T
- *in vitro* results for MK cell type #2 and #4 showed priming of NK cells and direct cytotoxicity in a leukaemia and myeloma cancers
- Studies currently being complete to demonstrate efficacy with NK cells in high value cancer market



# Summary

1. **Material** biotech company focused on next generation cancer medicines
2. **Delivered** positive R&D results for all 3 Midkine programs and Randox commercial licence
3. **Partnered** with leading academic cancer research centres which complements our own world-class in-house expertise and laboratory infrastructure
4. **Established** the foundation and team to deliver the key R&D and commercial milestones that will drive value from five fully funded R&D programs
6. **Developed** a new anti-Midkine mRNA platform – a potential game changer for the Company
7. **Near-term value** inflection milestones of IND and licensing opportunities from advanced stage of development of Midkine, MK and siRNA products

# Appendix

# Licencing & Royalty Agreement

- Exclusive worldwide licence agreement (excluding Japan) for 10 years to utilise Midkine antibodies in medical diagnostics
- Collaborative research programs to identify new cancer diagnostics treatable with ROQ's Midkine therapeutics
- Company can remain focused on developing first in class oncology medicines

**Validates Midkine** as a target

Clinical trials with companion diagnostics have a much higher **success** rate – 15.9% vs 7.6%

Diagnosing patients early will **accelerate** our ability to diagnose patients for clinical trials, dramatically reducing time and cost for clinical trials

www.roquefortplc.com