

VALIRX PRESENTATION

Proactive Investors Jan 2016



- Founded: 2006
- Listed on LSE: 2006
- Stock Symbol: VAL (AIM)

www.valirx.com



**WE ARE A CLINICAL STAGE DRUG DISCOVERY LIFE SCIENCE
COMPANY FOCUSED ON DELIVERING BREAKTHROUGH
CANCER THERAPEUTICS, DIAGNOSTICS, AND GENOMICS FOR
PERSONALISED MEDICINE**

VALIRX IS A GROWING COMPANY WITH A CLEAR BUSINESS MODEL



- **Oncology portfolio of innovative technologies with worldwide rights from leading institutions**
- **Therapeutics, biomarkers and companion diagnostics in development**
- **A growing company with a clear business model to engineer scientific breakthroughs in human health and wellbeing.**
- ***Global market for cancer therapeutics is expected to cross \$100 billion this year with CAGR of 12.3% (Fiercebiotech 2015)***

OVERVIEW



VALIRX ADOPTS OUTSOURCING BUSINESS MODEL WITH LEAD COMPOUND IN CLINICAL DEVELOPMENT & A GROWING PIPELINE TO ADDRESS AN EXPANDING MARKET WITH HIGH UNMET MEDICAL NEEDS

- Growing pipeline of development & clinical stage products with potential to address large and growing market with UNMET needs - Technologies originate from world class universities and institutes
- The focus is to partner or out-licence within the Phase II development pathway, **before** the initiation of Phase III.

- *The early out-licensing of therapeutic candidates to crystallise value, lower development risk and maximise the likelihood of value creation*

- **VAL201 :**

- Significant Tumour static and Anti-Metastatic effect in preclinical models
- Phase I/II *dose escalation* study ongoing
- Initial outcomes anticipated Q2

- **VAL401**

- Preclinical phase completed
- Scientific meeting held with MHRA to determine point of entry to clinical trials
- Poised to jump to late stage trials

- **GENEICE:** Epigenetic gene silencing platform advancing through the preclinical phase with **VAL101** as initial product candidate targeting the BCL2 gene.

KEY ACHIEVEMENTS 2015



- **VAL201 Cancer trial** established and underway at the Clinical Trials Unit at **UCLH**
- First **patient cohort dosing** with VAL201 completed with no serious adverse events reported
 - Phase Ib completed / phase IIa ready
- **VAL401*** for lung cancer and other oncology indications; preclinical proof of concept completed - other indications are breast, pancreatic and prostate cancer
- Acquisition of TRAC of gene expression analysis platform
 - Initial revenues delivered

* being developed through ValiSeek Limited, a joint venture company in which ValiRx is the majority owner

THE VALIRX SOLUTION

LEAD PORTFOLIO PROGRAMMES TARGETING HIGH UNMET ONCOLOGY NEEDS



*Global market for cancer therapeutics is expected to cross \$80 billion this year with CAGR of 12.3%**

SOLID TUMOURS

VAL201 – Phase I/II clinical trial ongoing.

- **NOVEL MOA**
- Initial Target is Prostate Cancer
- Targeted
- Tumour-static
- Anti-metastatic action
- Follow-on indications

SOLID TUMOURS

VAL401 – Reformulated CNS drug for Lung Cancer

- **NOVEL MOA**
- Established safety profile
- Poised to jump from preclinical to later stage clinical trials
- Tumour growth suppression seen after 7 days
- Follow-on indications

DIAGNOSTICS

- **TRAC:** A high-content, rapid and cost effective gene expression analysis platform - integration of this platform with diagnostic and service products is in development
- **NAV3:** Developing biomarkers with an emphasis on the early detection of tumour cell formation

GenelCE

Epigenetic gene silencing platform

THERAPEUTIC ASSETS



VAL 201



PEPTIDE
VAL201 - PROSTATE CANCER & OTHER LOCALLY ADVANCED OR METASTATIC CANCERS
VAL201 – ENDOMETRIOSIS



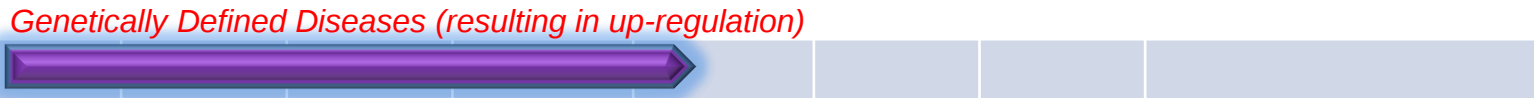
VAL 401

SMALL MOLECULE
VAL401 - LUNG CANCER



GeneICE

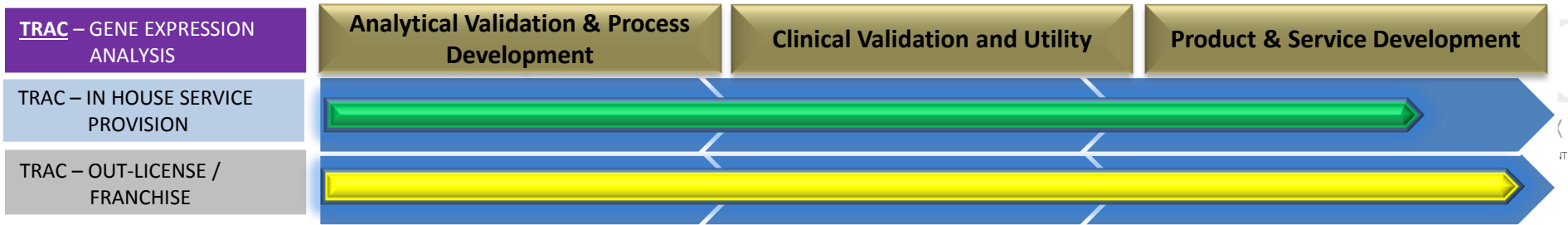
PLATFORM
GENEICE - BCL2 RELATED CANCERS – VAL101



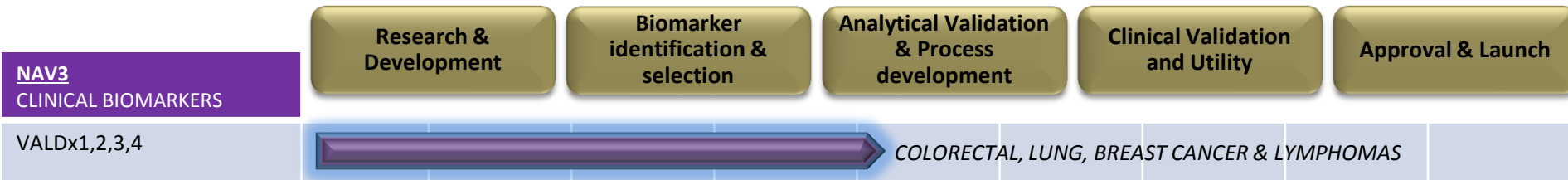
DIAGNOSTIC ASSETS



Gene Expression analysis for high content screening, assay development & high throughput screening)



CLINICAL BIOMARKERS

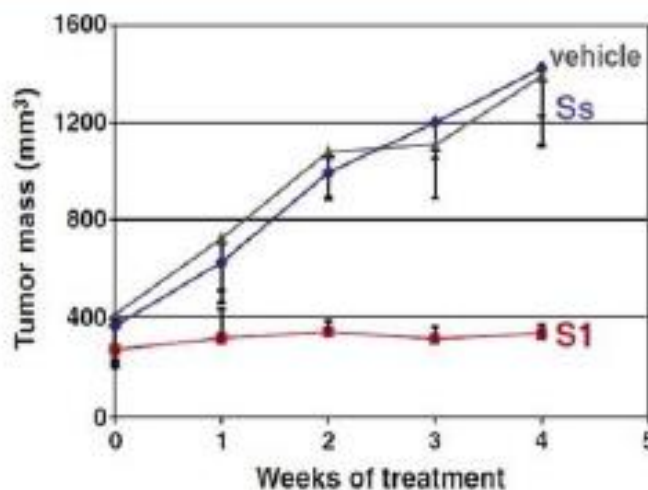


VAL201 : ANTI-TUMOUR ACTIVITY – FOR PROSTATE CANCER*



NOVEL “MECHANISM OF ACTION”

SUPPRESSION OF TUMOUR GROWTH IN XENOGRAFT MOUSE MODEL



KEY
NEGATIVE CONTROL
Ss-NEGATIVE CONTROL
S1 TREATMENT VAL201

- A novel “mechanism of action”
- Does not affect the androgen receptor

- Targeted
- Clinical Phase Ib/IIa in prostate cancer patients ongoing

*Prostate cancer market set to expand to \$7-9 billion by 2020, due to growing prostate cancer population (25% from 2010 to 2020). (GlobalData 2012)

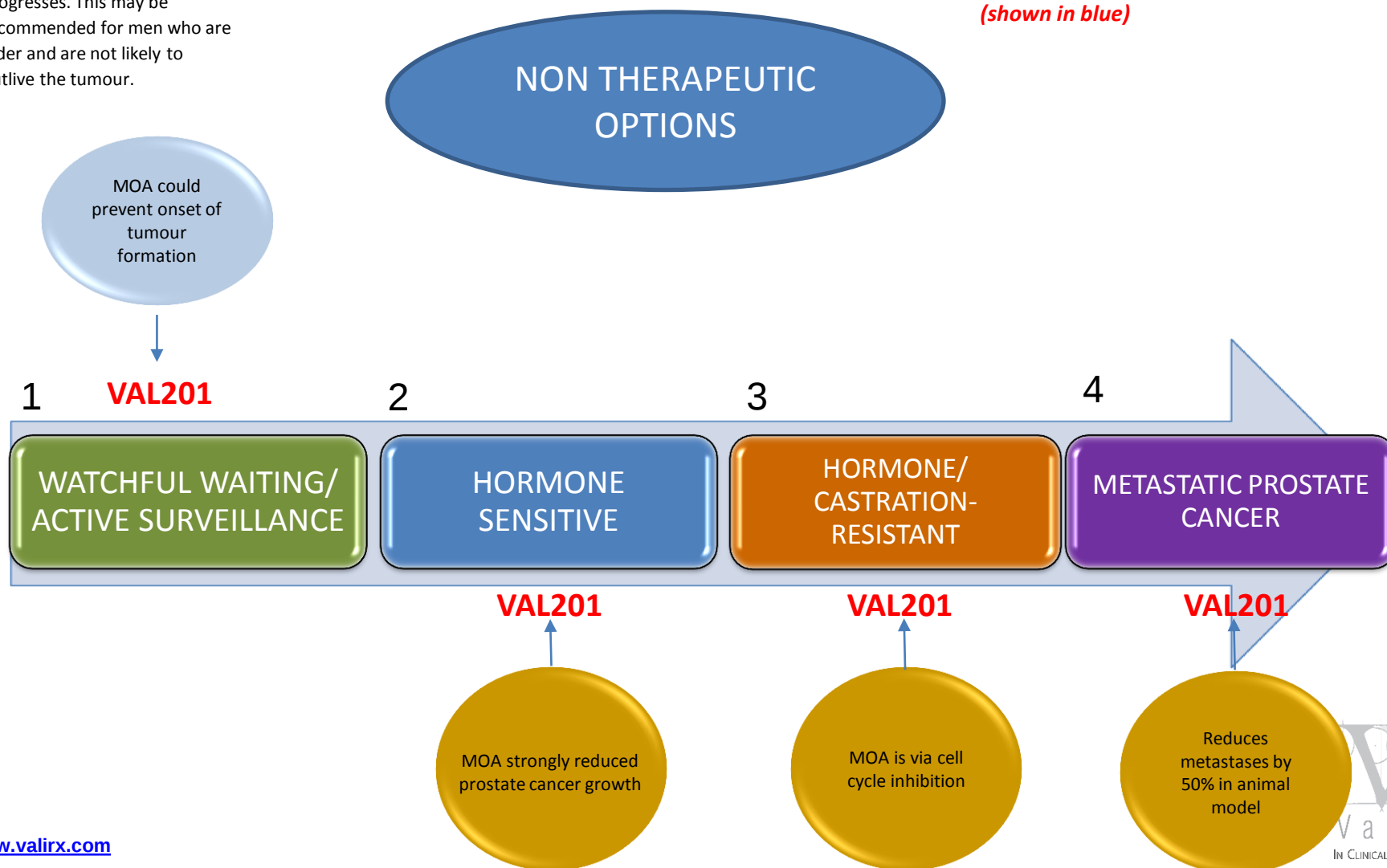
**A common health problem among women, Endometriosis market predicted to reach \$1.66 billion by 2017. (GlobalData 2012)

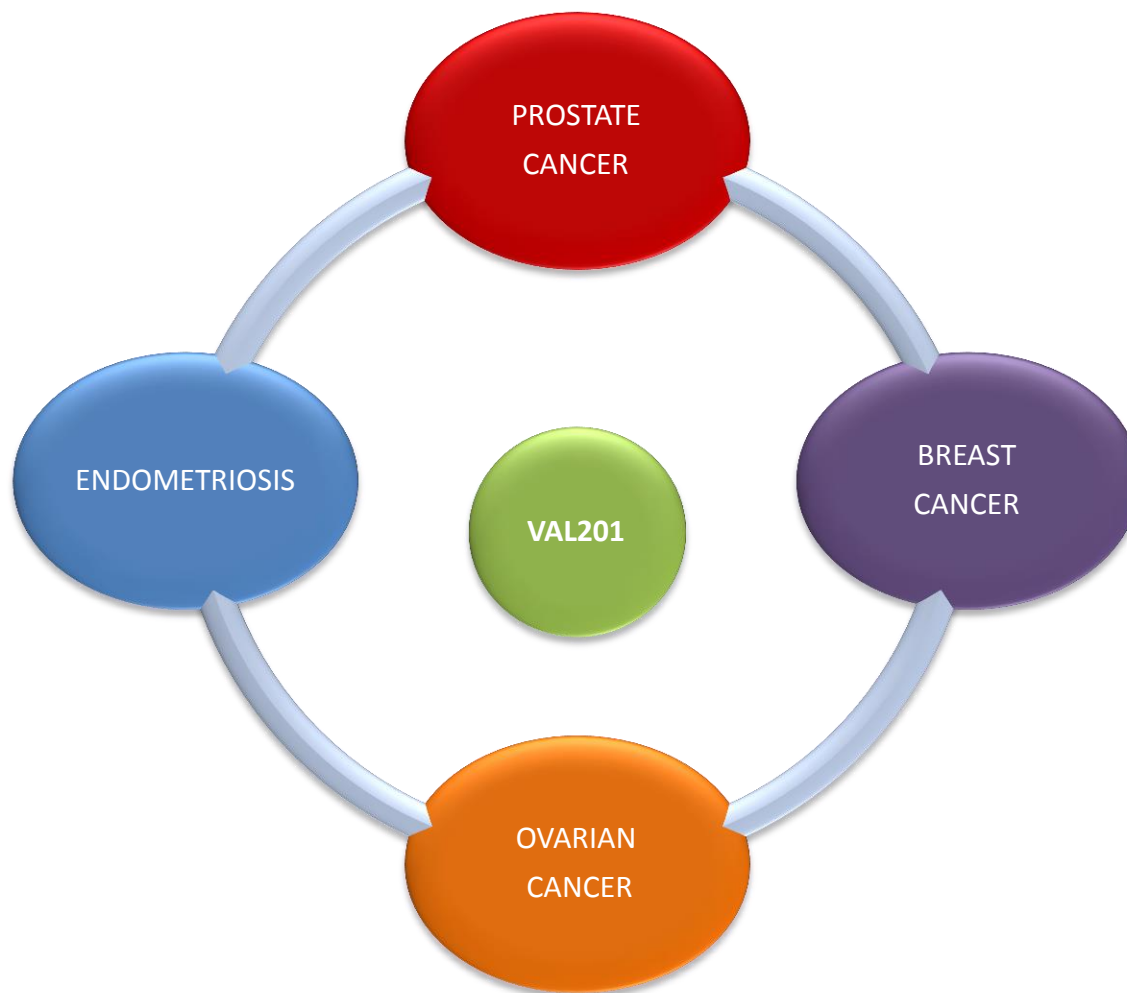
THE LANDSCAPE: PROSTATE CANCER STAGES, TREATMENTS & THERAPIES



Watchful waiting means monitoring prostate cancer in the early stages and only starting treatment if the cancer progresses. This may be recommended for men who are older and are not likely to outlive the tumour.

VAL201 could be used in all stages of prostate cancer either as a single treatment or in conjunction with the other therapeutics (shown in blue)



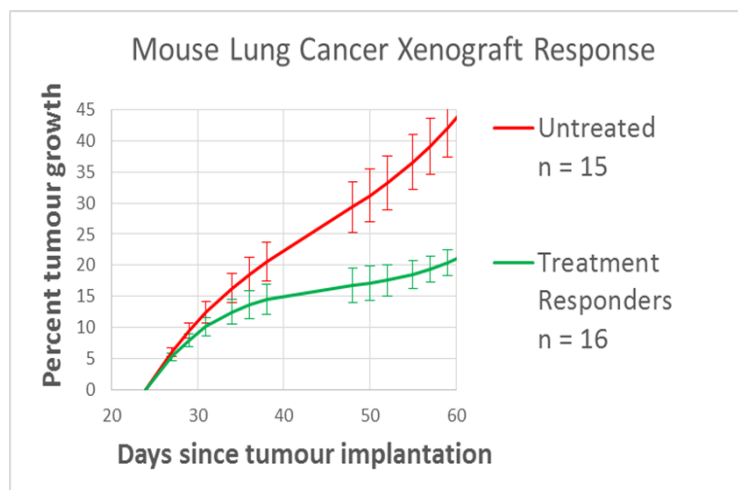


*VAL401: A NOVEL REFORMULATION FOR LUNG CANCER



REFORMULATED GENERIC DRUG PREVIOUSLY USED IN NON-ONCOLOGY INDICATION WITH 2 DECADES OF CLINICAL AND SAFETY DATA

SUPPRESSION OF TUMOUR GROWTH SEEN AFTER 7 DAYS OF DOSING



- **Secondary benefits include anti-cachexia**
- **Can be used for late stage patients as single or concomitant treatment**
- **Can be used at different stages of lung cancer progression**
- **Known & beneficial side effect profile**

- **Delivered as a reformulated oral capsule for NSCLC**
- **Vastly reduced side effect profile**
- **Novel mechanism of action**

LUNG CANCER STAGING



STAGE 1- A/B

- The tumour is small (up to 3cm)
- The cancer is between 3 to 5cm. It may have spread into nearby structures such as the main airway of the lung (bronchus) or the membrane covering the lung (pleura).
- The lung may have partly collapsed

STAGE 2- A/B

- The cancer is between 5 and 7cm but there are no cancer cells in any lymph nodes..
- The cancer is between 5 and 7cm and there are cancer cells in the lymph nodes close to the affected lung
- Larger than 7cm but there are no cancer cells in any lymph
- Part of the lung has collapsed

STAGE 3

- The whole of the affected lung may have collapsed or may be inflamed
- Has spread into one or more of the following areas – the chest wall, the muscle under the lung, or the layers that cover the heart
- Has spread into lymph nodes close to the main airway or in the centre of the chest on the same side as the affected lung
- In more than one lobe of the same lung
- Has spread into lymph nodes on the opposite side of the chest from the affected lung

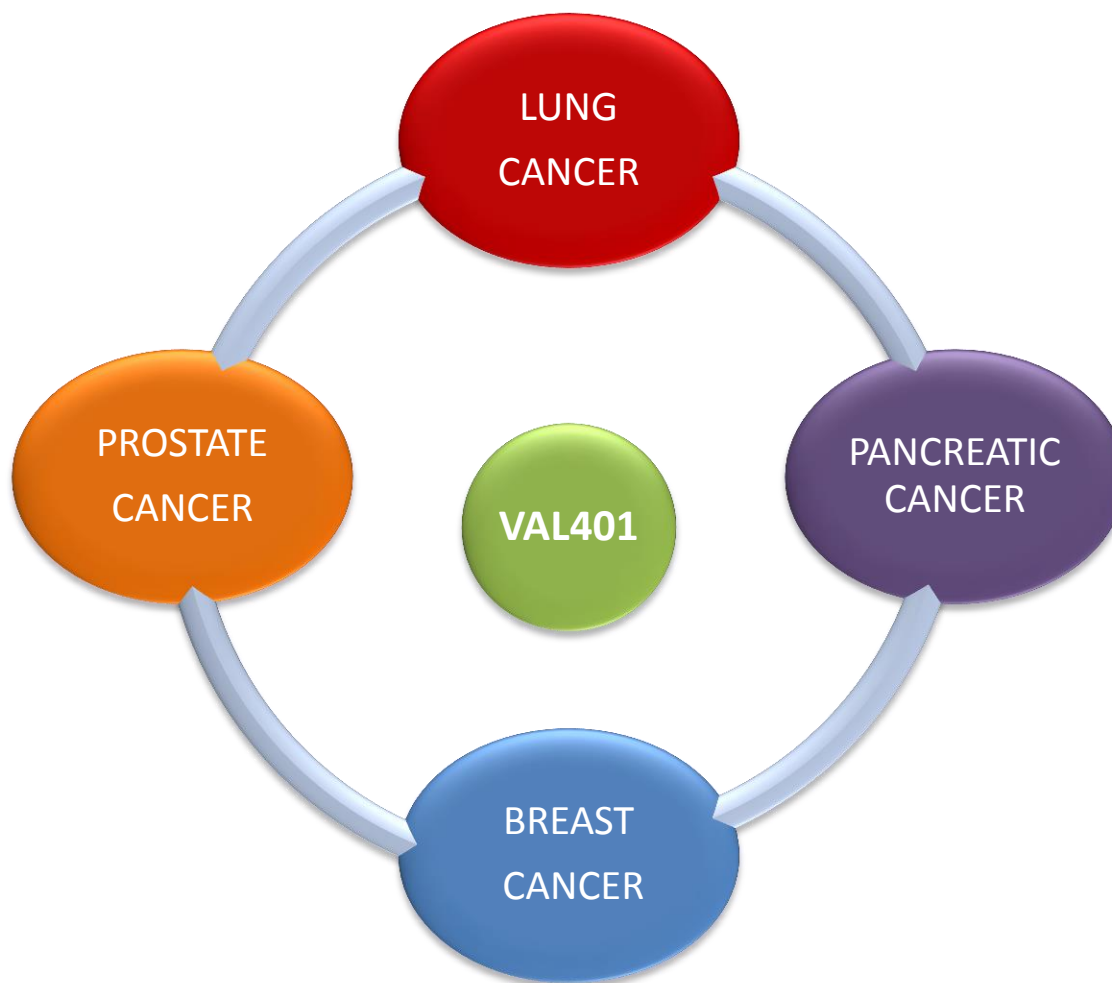
STAGE 4

- Is in both lungs **OR**
- Has spread to another part of the body, for example, the liver or bones **OR**
- Has caused a fluid collection around the lungs or heart that contains cancer cells (a malignant pleural effusion) or pericardial effusion
- Palliative care

VAL401: Potential for use at or just after diagnosis since it may prevent the spread of the cancer

VAL401: Potential for use at Mid-stage to prevent further spreading of the cancer, and possibly regression

VAL401: initial trials focus on end stage patients



GENETICALLY DEFINED DISEASES

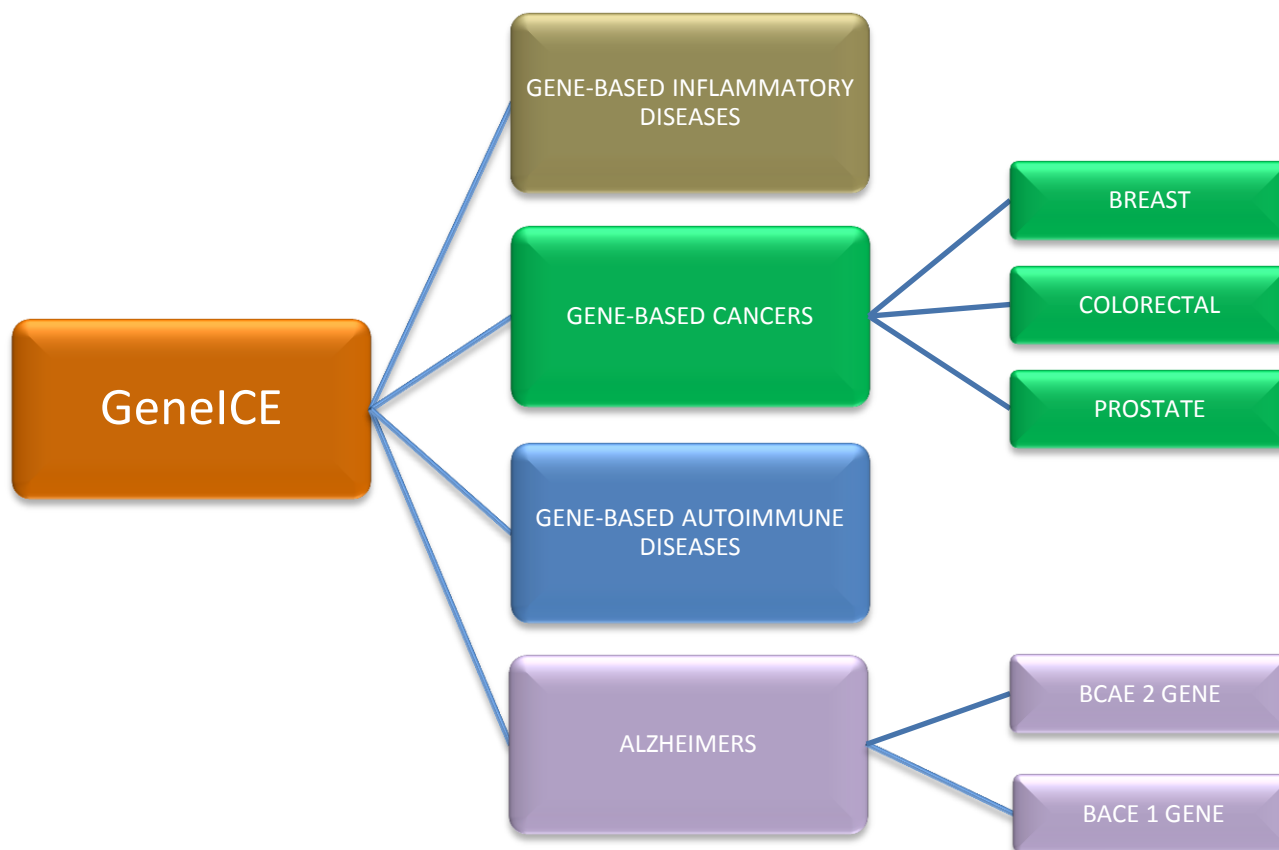
GeneICE PLATFORM

RESOLVING GENETICALLY DEFINED DISEASES USING GENE SILENCING



VAL101

VAL101 is the first molecule developed from the GeneICE Platform that targets the BCL2 gene which is upregulated in certain cancers



A CLINICAL STAGE COMPANY WITH ONCOLOGY THERAPEUTICS AND DIAGNOSTICS

LEAD PORTFOLIO PROGRAMMES TARGETING HIGH UNMET ONCOLOGY NEEDS

*Global market for cancer therapeutics is expected to cross \$80 billion this year with CAGR of 12.3%**



TRAC

A gene expression and biomarker technology
'**TR**anscript Analysis with the Aid of **A**ffinity **C**apture'-

A HIGH-CONTENT GENE EXPRESSION ANALYSIS PLATFORM.

- Low sample volumes
- Rapid testing
- Large number of genes analysed at once
- Lower cost

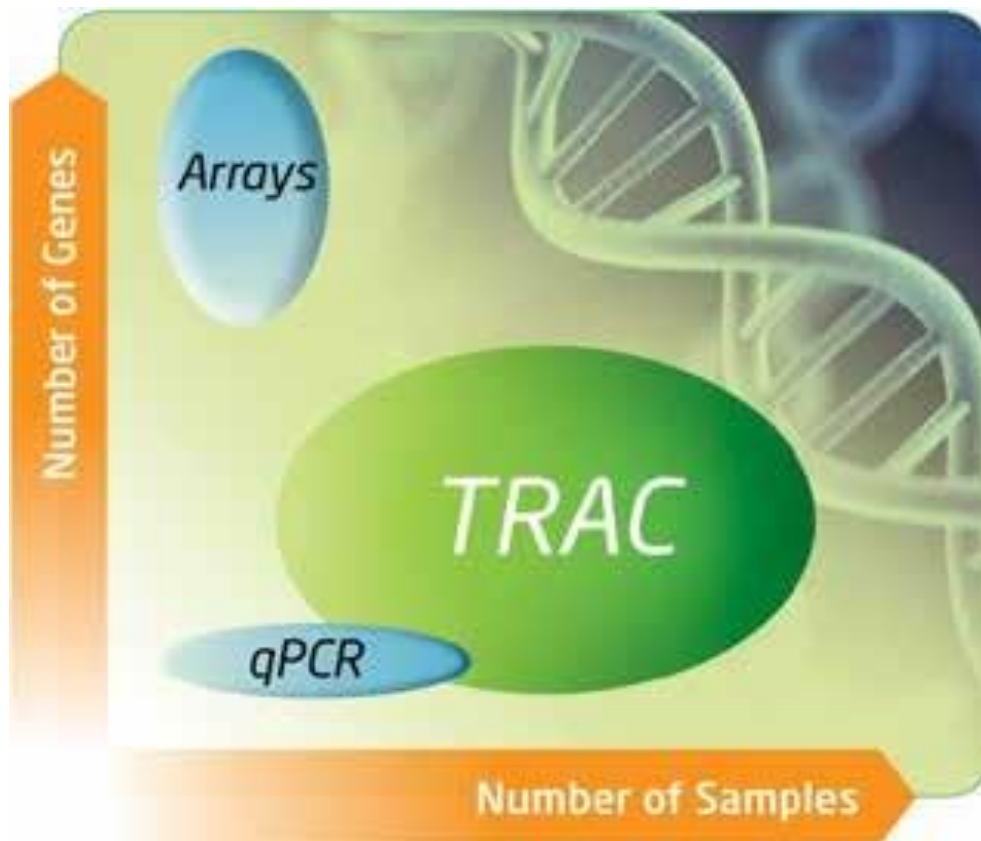
NAV3

A set of biomarkers for colon cancer, breast cancer, lung cancer as well as neurological cancers and lymphomas; these biomarkers will allow the **EARLY DETECTION OF PRE-MALIGNANT CELLS** at a stage where tumour development is only about to start.

Oncology Indications :

- Colorectal
- Lung
- Breast
- Lymphomas
- Neurological

TRAC IN CONTEXT



A high-content gene expression analysis platform to support the next wave of gene function analysis.

TRAC (Transcript Analysis with the Aid of Affinity Capture) is a gene expression method that enables the screening of hundreds to thousands of samples for dozens of marker genes.

PROVIDING CLARITY ON INITIAL TRIALS & ENHANCING THE POWER OF FUTURE CLINICAL TRIALS

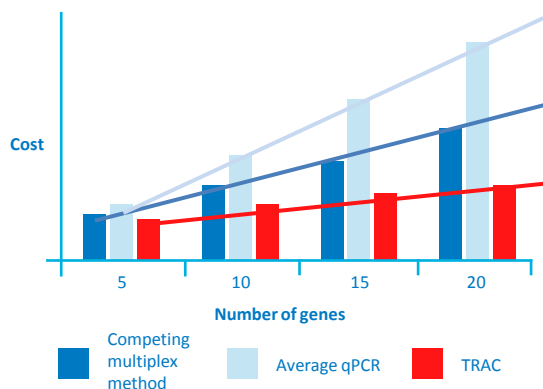
GENOMICS - DIAGNOSTICS SERVICE – TRAC IN CONTEXT



THE GENE EXPRESSION CHALLENGE DIRECTLY ADDRESSED

- Effect expressed by genes
- Effect of drugs on genes or gene products
- Rapid testing – turnover time orders of magnitude faster
- Large number of genes analysed at once
- Low cost
- Gene response easy to analyse
- Minimal hands on time

TRAC is cost-effective



IN SUMMARY :



- **A clinical stage development company** with an experienced and focused executive team.
- Our **technologies originate from world-class universities** and institutes.
- Our lead compound **VAL201 is progressing well with its Phase Ib/IIa clinical trial** addressing prostate cancer. Our drug trials are progressing and continuing to **show favourable safety profiles with early indications of efficacy even at low dosage.**
- ValiSeek has **completed its pre-clinical work on VAL401 for lung cancer and is phase IIb ready.**
- **The acquisition of TRAC**, the Finnish gene expression and biomarker technology, **has delivered immediate benefit and a growing revenue stream.**
- We have **opened a new US office** in Boston, Massachusetts.
- And we look forward to an exciting and busy year as we seek to broaden our clinical trial programmes and **look to commence work with a first clinical study of VAL401 in patients with lung cancer.**

SENIOR MANAGEMENT & BOARD OF DIRECTORS



Dr Satu Vainikka **CHIEF EXECUTIVE OFFICER** - Satu has many years' experience of the biotechnology industry, including extensive first-hand experience of equity financing, business management and developing life science technology into commercial enterprises. Prior to her current role as CEO of Valirx, she was a founder, director and CEO of Cronos Therapeutics Limited.



Dr George Morris **CHIEF OPERATIONS OFFICER** - George has over 25 years' experience in biological and medical research, and financial services. In the past he has worked for Guy's hospital medical school department of medicine, King's college and University College London. As a Research Scientist, He is an author of numerous books and articles on refereed papers, approximately 70 abstracts, short reports and posters, and an inventor of multiple patents.



Mr Gerry Desler **CHIEF FINANCIAL OFFICER** - Gerry is a chartered accountant, who qualified in 1968 with a City firm, before becoming a partner in 1970. Between 1985 and 1990 he was the Senior Partner. During his time in the City, he has specialised in consultancy work, much of it involving funding and venture capital.



Mr Oliver de Giorgio-Miller **CHAIRMAN** - Oliver has a wealth of experience in the management and commercial advancement of life science companies. He has worked for over 30 years with several global pharmaceutical and medical device companies including Schering AG, Hoffman la Roche, Intavent-Orthofix and Photo Therapeutics, a Cancer Research UK company and he has extensive experience advising a number of other early stage biopharmaceutical and medical device companies.



Mr Kevin Alexander **NON-EXECUTIVE DIRECTOR** - Kevin is a qualified solicitor in England and an attorney in New York and he was a partner at major law firms in both London and the United States for over 25 years. Since leaving the law he has been involved in forming and managing various businesses, both private and public. Kevin is a director of Valirx Plc, and joined the board in September 2006. He has an MA in law from Cambridge University.



Mr Seppo Mäkinen **NON-EXECUTIVE DIRECTOR** - Seppo Mäkinen has more than 25 years of senior advisory and executive experience in board level strategic leadership and venture capital management on life science, his special expertise is on Medtech / Diagnostics. His career includes ten years as Director in Life Sciences at Sitra (Finnish Government Fund), followed by thirteen years as co-founder and Managing Partner in Bio Fund Management Oy from this time also five years as President of BioFund A/S, Copenhagen. With EUR 200M under management, BioFund was one of the biggest European VC funds investing into international life sciences. In 2008,

SCIENTIFIC ADVISORY BOARD



Professor Calvert BA, MB BChir, MD, MSc, PhD., who is Director of Cancer Drug Discovery and Development at the UCL Cancer Institute, is internationally known among medical oncologists for his seminal work on chemotherapy and the development of the Calvert formula, which has benefited countless patients around the world. - He has a longstanding interest in developing new drugs for cancer treatment and has been associated with several successful drugs currently used worldwide, which include carboplatin (for ovarian cancer), pemetrexed (for mesothelioma and lung cancer) and inhibitors of poly (ADP- ribose)polymerase (PARP) for familial breast and ovarian cancers. He has previously worked in the Royal Marsden Hospital and the University of Newcastle (as professor of Medical Oncology). – Prof Calvert is also Chair of the New Agents Committee at Cancer Research UK. He is a member of the Department of Health's Gene Therapy Advisory Committee and has recently served on the Health Services and Public Health Research Board at the Medical Research Council. Internationally, he is a member of the EORTC Pharmacokinetics and Molecular Mechanisms Group. Professor Calverts awards include, British Oncological Association Lifetime Achievement Award, European Society for Medical Oncology, Lifetime Achievement Award, and their Pfizer Award for Innovative Science.



Professor Alan Boyd BSc, MB, ChB, FFPM, began his 30 years' pharmaceutical career with Glaxo Group Research Ltd. From 1988 he led ICI's cardiovascular medical research team, later assuming the role of Director of Clinical and Medical Affairs at ICI Pharma, Canada. In 1999, after four years as Head of Medical Research for Zeneca Pharmaceuticals, he became Director of Research and Development for Ark Therapeutics Ltd where he was responsible for delivering the majority of key development milestones that have shaped the company. In particular he led the development of their gene based medicines portfolio. A graduate in Biochemistry and Medicine from the University of Birmingham, UK, Professor Boyd is also a Fellow, and Chair of the Specialist Advisory Committee in Pharmaceutical Medicine at the Faculty of Pharmaceutical Medicine, Royal College of Physicians, United Kingdom. He was elected Vice-President of the Faculty in November 2012. In November 2009 he was appointed an Honorary Professor in the College of Medical and Dental Sciences at the University of Birmingham Medical School, in recognition of his expertise in medicines development.



Dr Richard Harbottle., PhD., is currently the Group Leader of the DNA Vectors Group at the DKFZ – (German Cancer Research Centre), his research group is focused on generating novel, next-generation DNA vectors for gene therapy, a vector system, which is uniquely suited for the genetic modification of cells. He was formerly at Imperial College London where he focused on the development of DNA technologies for gene therapy, with a particular focus on the application of scaffold/matrix attachment region vectors. Richard is a founding member of the British Society for Gene Therapy and served as an elected member of its executive board since the inception of the society in 2003 until 2010. He is an active member of the society and currently contributes as a co-opted member of the executive. He is also an active member and participant of the American and European gene therapy societies and the American Association of Cancer Research.



www.valirx.com

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