

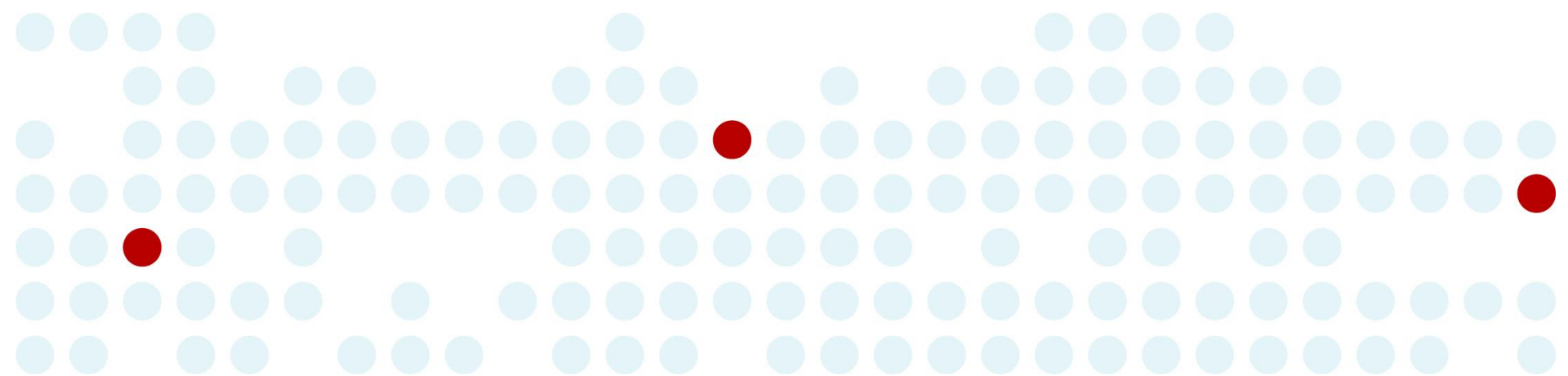


# EXIQON

Seek Find Verify

Science Session, Handelsbanken  
October 22, 2008

Lars Kongsbak, President & CEO



## Agenda

- Exiqon at a glance
- A paradigm shift
- How Exiqon addresses this paradigm shift
- Market description and current & future products for personalized medicine in oncology
- Consolidation and Exiqon as potential acquisition target

## Exiqon at a glance

### **EXIQON** Life Sciences

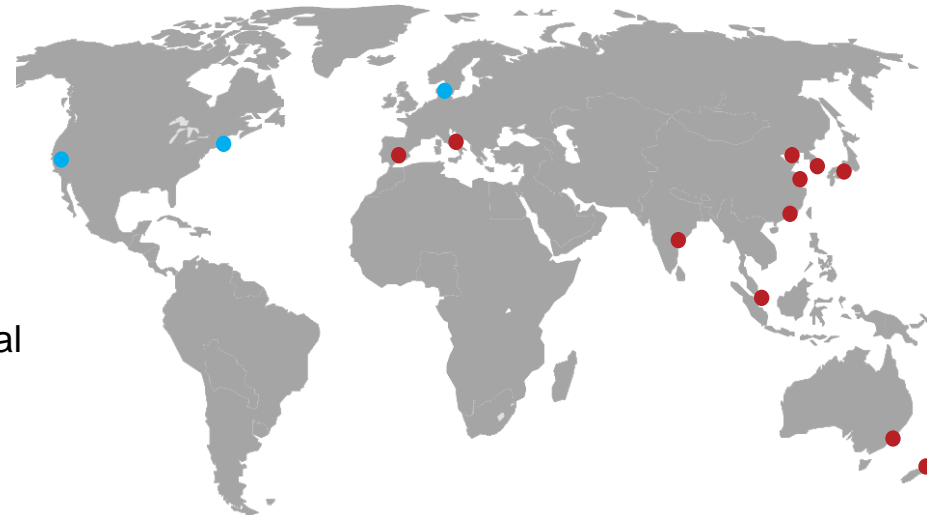
- Uses proprietary LNA™ technology to be the market leader in the Life Science field for gene expression analysis (miRNA and mRNA)

### **EXIQON** Pharma Services

- Uses the largest privately held human biobank (>150.000 tumor samples) for stratification of patients and product development (companion products) in partnership with the pharmaceutical industry

### **EXIQON** Diagnostics

- Uses proprietary miRNA biomarkers to develop, market and sell proprietary molecular diagnostic products for treatment selection (cancer), based on market leading position of Oncotech within cellular assays



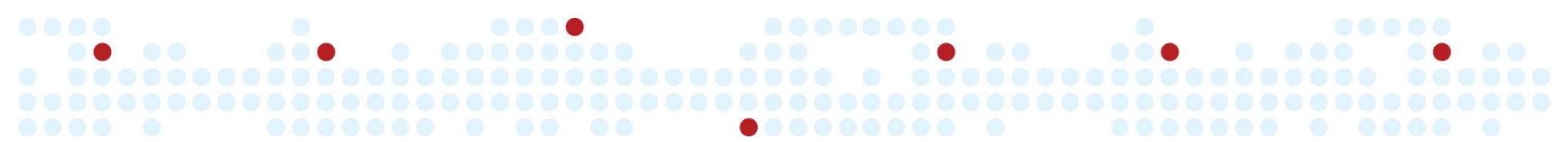
- Exiqon facilities
- Distributors

## Recent key events

- Recruitment of CSO, Cynthia French and CCO, Erik Holmlin
- Major agreement entered into with Roche. Potential: Up to \$200 mill

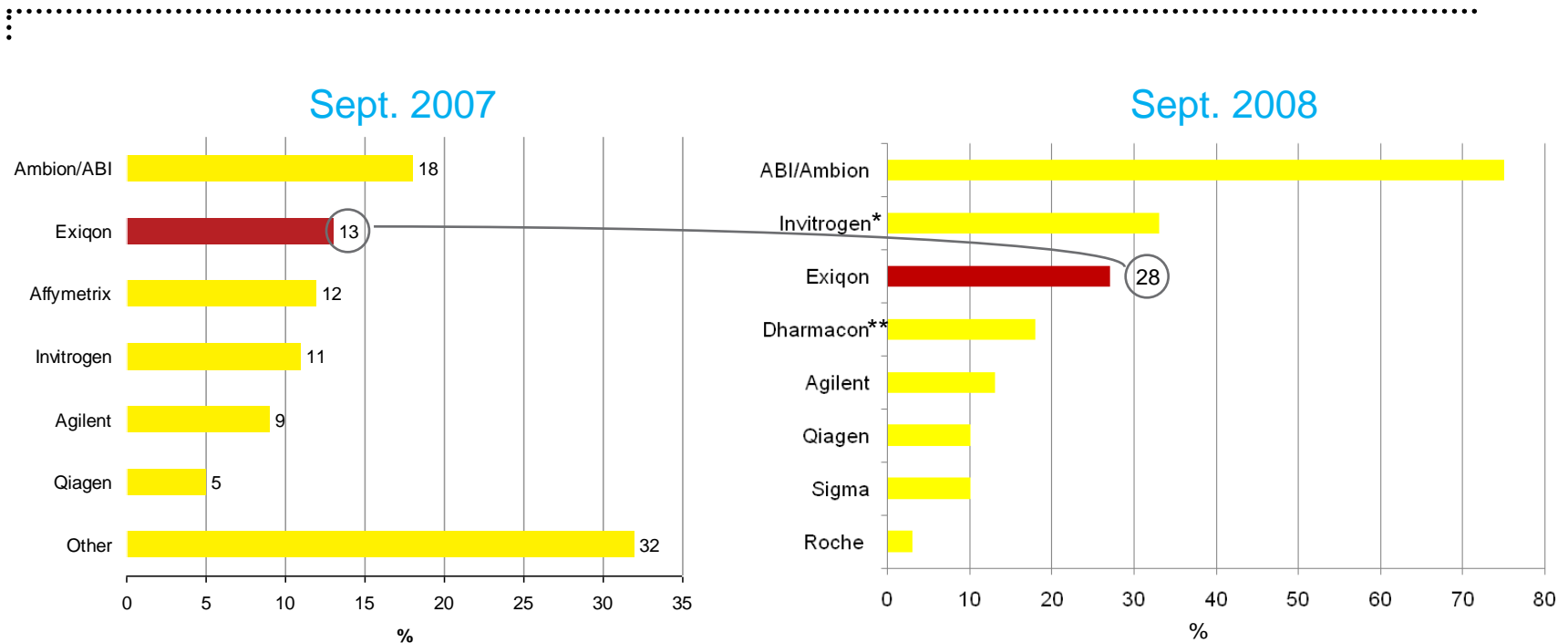
....and previous statements reiterated

- Fully financed to profitability in 2011
- Life Science business (tools) to be cash flow positive from end 2009
- First proprietary miRNA and LNA based diagnostic product to be launched end 2008



## Established as a leader in the market place for miRNA products

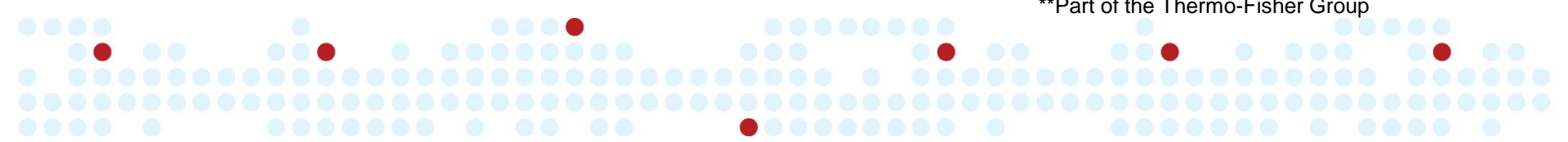
When considering a research product for your miRNA research, which manufacturers come to mind? (please list up to three)

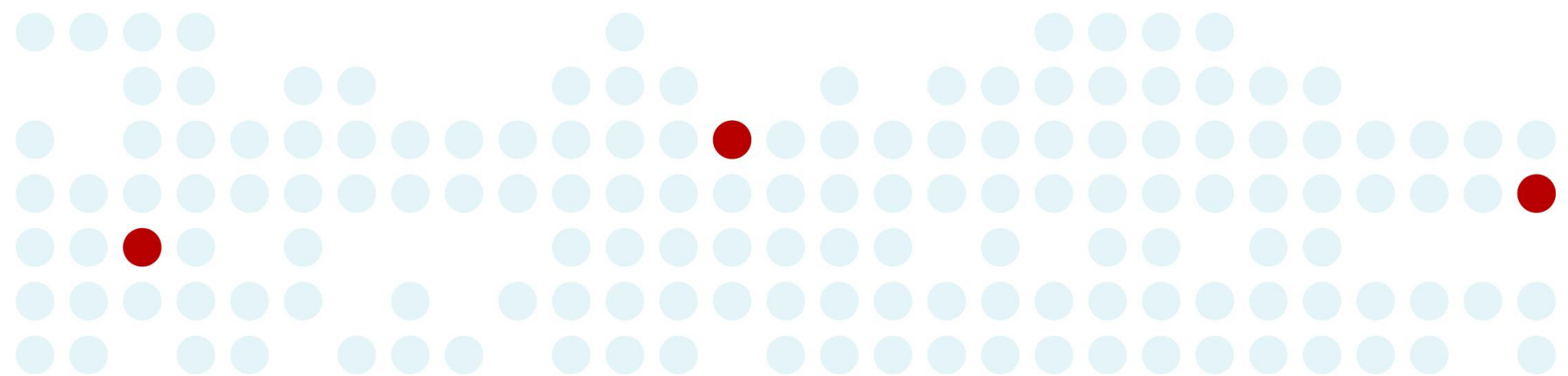


Source: Online survey (registered as visitors to the *Science* website): life scientists working in molecular biology were asked to participate; response based on 239 and 147 completed surveys, respectively.

March 30, 2009

\*Invitrogen has in the meantime acquired ABI 5  
\*\*Part of the Thermo-Fisher Group

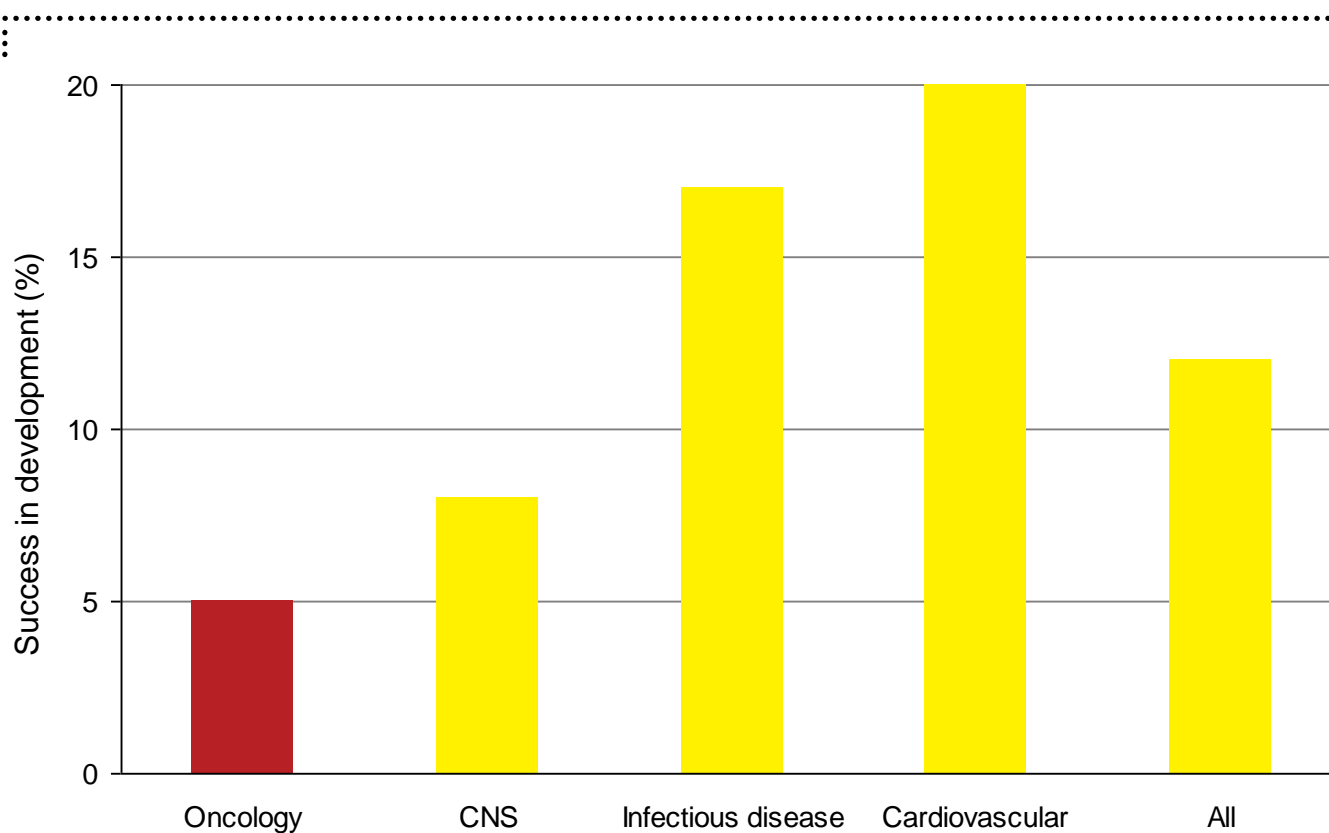




A paradigm shift

## Why a paradigm shift is needed within cancer

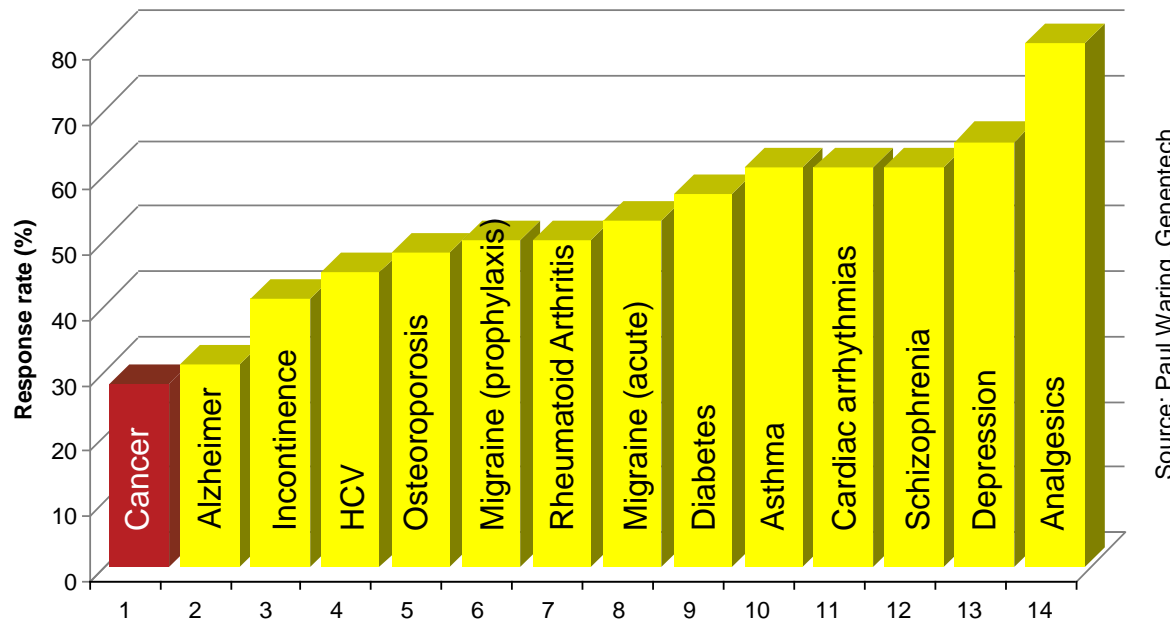
Oncology compounds have had significant lower success rate in drug development



March 30, 2009

Source: Kola & Landis J. Nature Rev. Drug Discov. 2004, 3:711-715

## Why a paradigm shift is needed within cancer



- 70% of cancer patients do not respond to chemotherapy
- \$8.4bn in annual drug costs associated with non-responding chemotherapy\*

## A paradigm shift is needed in oncology

Today:

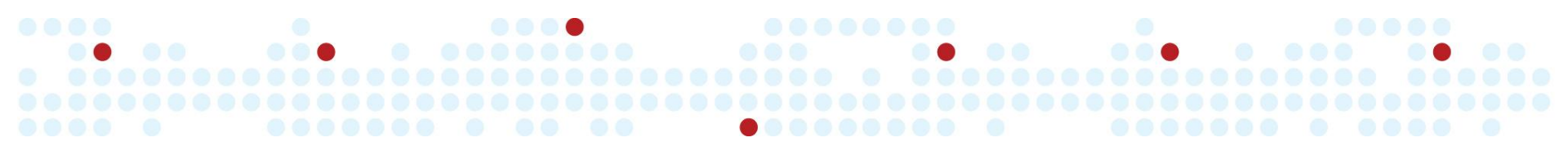
- Cancer is classified according to organ
- Drug development is driven by tumor location e.g. lung, colon etc.
- This fails, as cancer is a very diverse disease

Tomorrow:

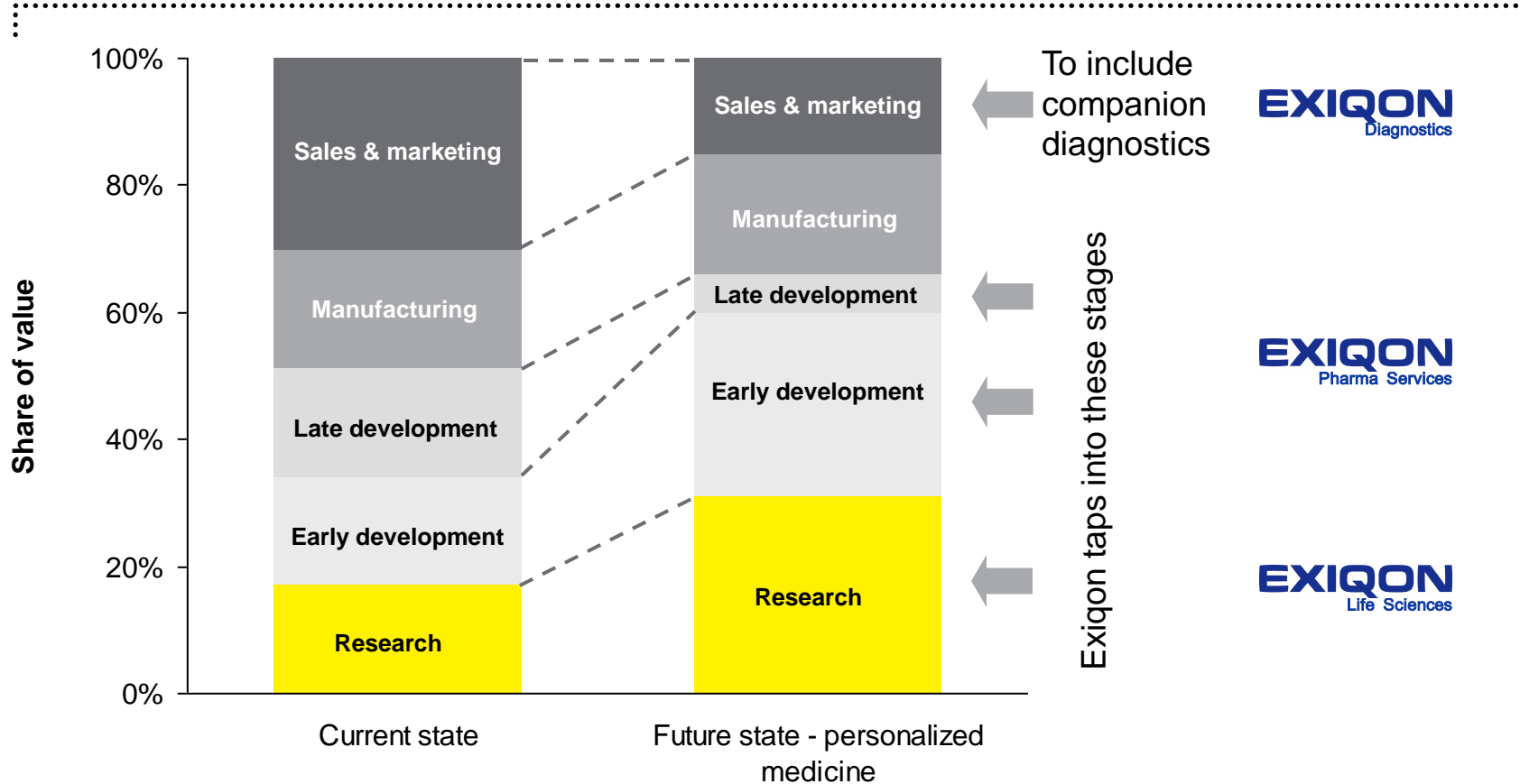
- Cancers will be classified according to mode of action (genomics)
- Drug development will be driven by mode of action e.g. cellular/functional status determines drug response
- That means: Biomarkers are used to drive product development & companion Dx

- This is personalized medicine -

- With that concept the market may not be more fragmented than today (from a pharmaceutical point of view)



## This paradigm shift affects the oncology segment



March 30, 2009

Source: Ernst & Young, Beyond Borders – Global Biotechnology Report, 2008 <sup>10</sup>

## Increased healthcare costs drive the need for a new paradigm

- US healthcare costs are expected to double by 2017 reaching \$44.3 trillion nearly consuming 1/5 of the economy\*
- CareFirst BlueCross BlueShield spent 36% of their care cost on chemotherapy amounting to \$936 mill in H1, 2007\*\*
- This is 25% up over 2006\*\*
- 81% of their oncology costs relate to breast, colon, lymphomas and lung cancers\*\*
- 150 cancer drugs have been approved. Why not start using these drugs in a more efficient way?

## This paradigm shift is also picked up by the US government



*“Markers can serve as the basis of new genomics-based diagnostic tests for*

- identifying and/or confirming disease*
- assessing an individual’s risk of disease*
- identifying patients who will benefit from particular interventions*
- tailoring dosing regimens to individual variations in metabolic response.”*

*“Current high level of interest in personalized medicine from a policy perspective is attributable not only to the promise of improved patient care and disease prevention, but also to the potential of personalized medicine to positively impact two other important trends – the increasing cost of health care and the decreasing rate of new medical product development.”*

A copy of the report is available online at <http://ostp.gov/cs/pcast>. 12

## This paradigm shift is picked up by big pharma



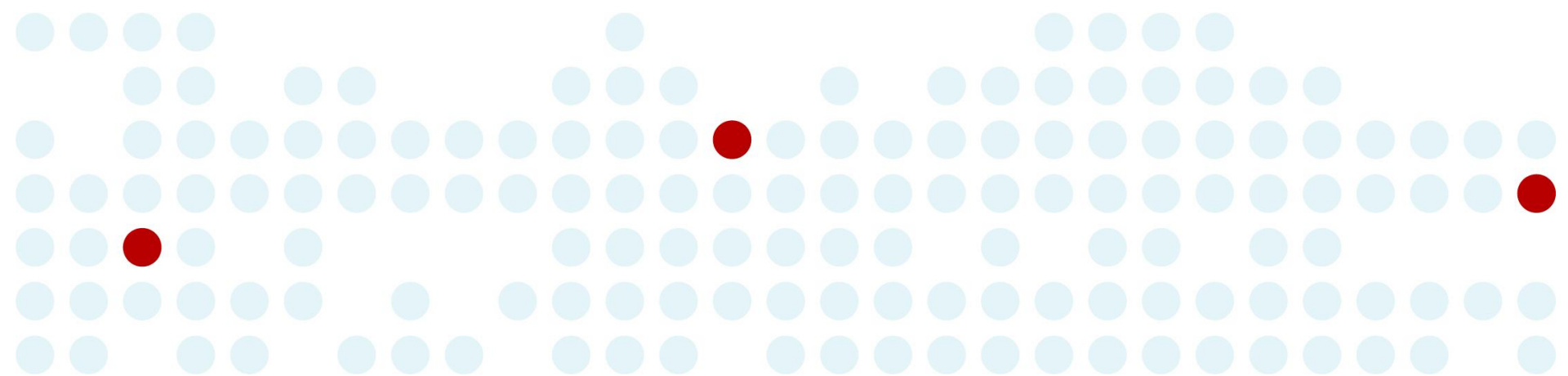
"One can say that, except for the very rare instance, Novartis' approach is that [unless] there is a drug with a really solid biomarker attached to it, we don't develop it," Robert Schmourder, executive director of translational medicine at Novartis, said at a Cambridge Healthtech Institute conference on translational medicine, held here last week.

Novartis peers Bristol-Myers Squibb, Wyeth, and Roche, among others, have similarly changed their R&D strategies and pharmacogenomic outlook.

Novartis is not alone in incorporating biomarkers into drug development and embracing the learn-and-confirm model. Encouraged by a willingness at the FDA to accept adaptive clinical trial designs, Novartis is following a larger shift within pharma toward more predictive drug-development strategies.

For instance, Bristol-Myers Squibb uses biomarkers and pharmacogenomics to expand the indications for existing oncologics [see [PGx Reporter 01-10-07](#)].

Also, Wyeth instated a learn-and-confirm model of its own last year, hoping that in two years the strategy will enable 75 percent of its drug program to have some kind of pharmacogenomic component.



How Exiqon addresses this paradigm shift

## This paradigm shift is defining our product offering

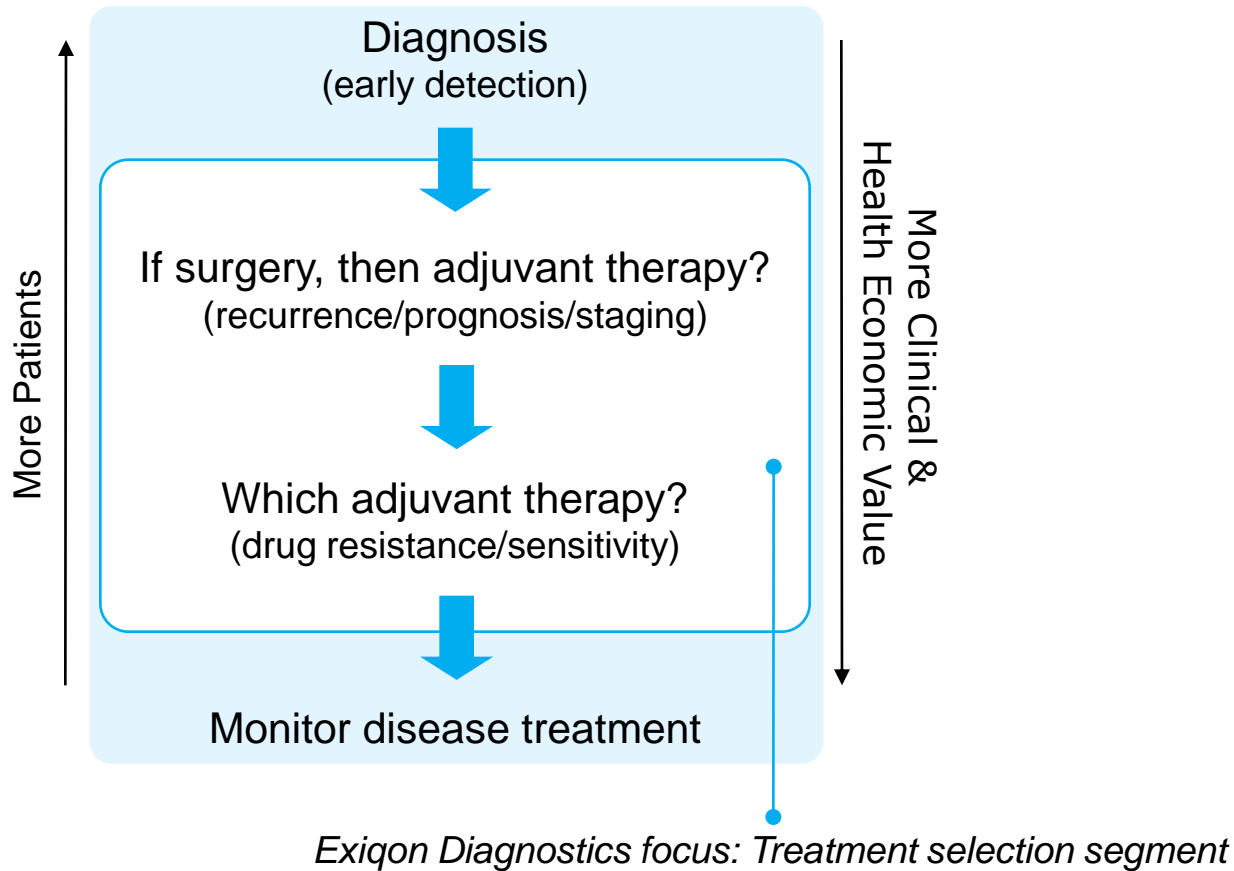
### **EXIQON** Pharma Services

- High throughput biomarker screening
- Proprietary biomarkers and assays
- 150.000 tumor bank
- 40.000 of the tumors still alive
- EDR tests (analysis of next generation drug)

### **EXIQON** Diagnostics

- CLIA services
- Proprietary diagnostic tests
- Non-proprietary tests
- One-stop supplier product offering
- Treatment selection only

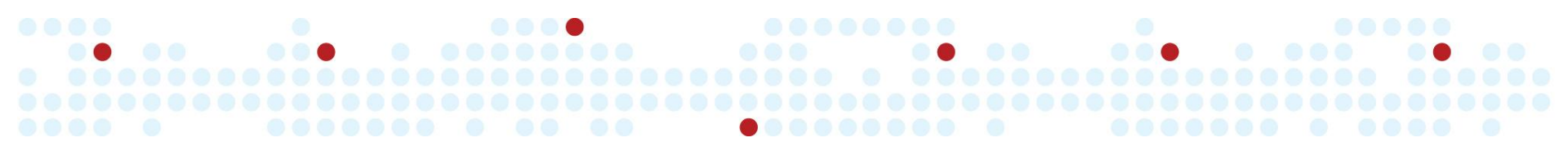
## Exiqon Diagnostics focuses on treatment selection



## Example of markets potentially addressed by Exiqon Diagnostics

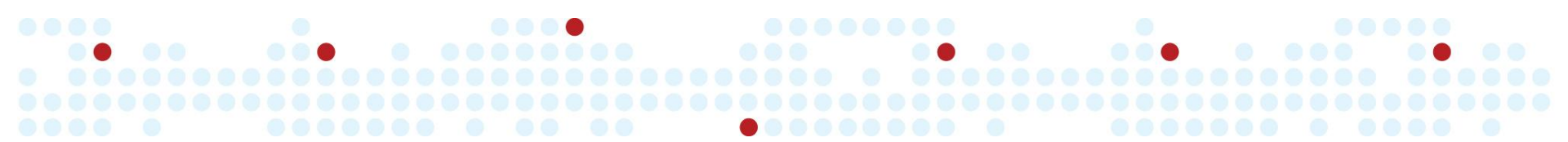
- Recurrence/prognostic tests may be applied in all stages
- The unknown category may need a staging test
- Drug resistance tests may be applied in some stage II and all stage III and IV patients

Cancer	Total Incidence	Stage Classification			
		I	II & III	IV	Unknown
Colon	148.810	59.524	53.572	28.274	7.440
Lung	170.704	27.312	42.676	87.059	13.656



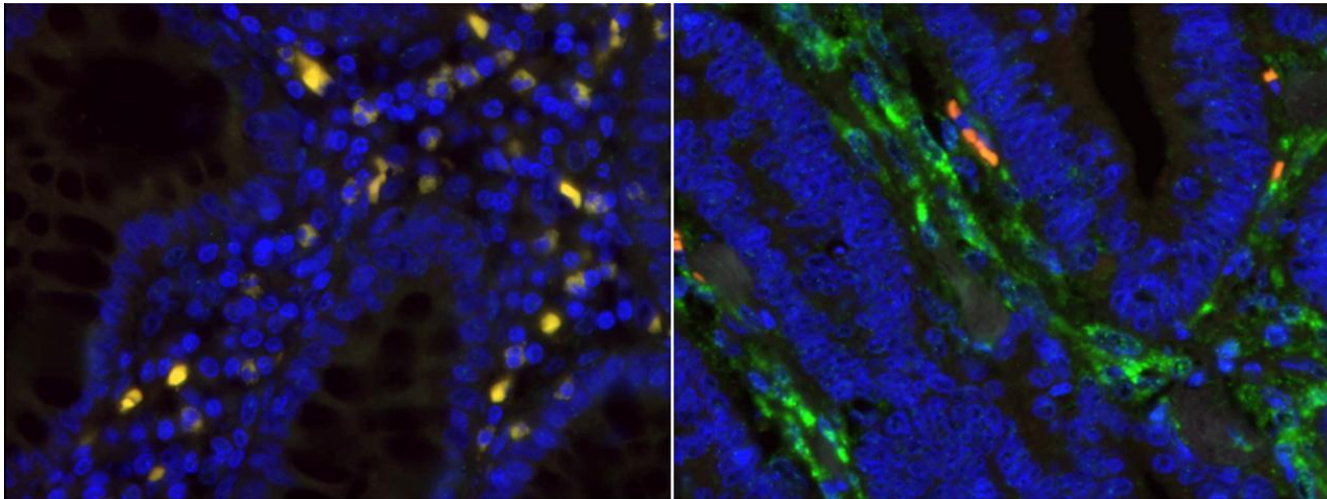
## How Exiqon might apply miRNA biomarkers and LNA in the new paradigm

- Standard of care for stage II colon patients recommends no chemotherapy
- However, 30% of colon cancer patients get recurrent cancer
- These patients may need chemotherapy upon surgery
- No recurrence test available

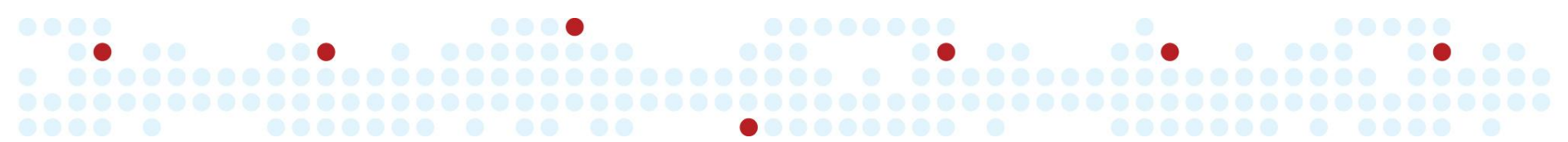


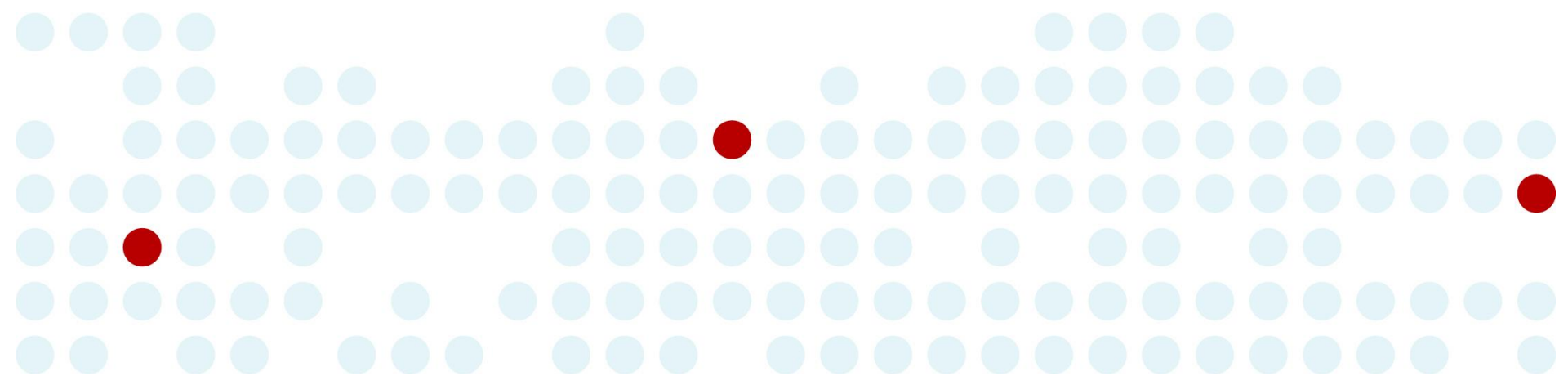
## How Exiqon might apply miRNA biomarkers and LNA in the new paradigm

- High miRNA-xxx\* activity indicates high risk of recurrence
- Detected by *in situ* hybridization and truly enabled by LNA



Colon cancer stage II: Green color indicates high risk of recurrence





## Market description and current & future products for personalized medicine in oncology

## Market outlook for molecular diagnostics

Kalorama Information "Molecular Diagnostics: Major World Markets" (2007) concludes:

- Molecular diagnostics is predicted to grow to over \$92bn by 2016, up from \$17bn in 2006 (41.5% annual growth)
- Growth fuelled by the push\* for personalized medicine
- Molecular diagnostics in oncology is expected to grow by 68% per year reaching \$9.8bn in 2016
- Kalorama Information emphasizes that this is a young industry with only a few significant players

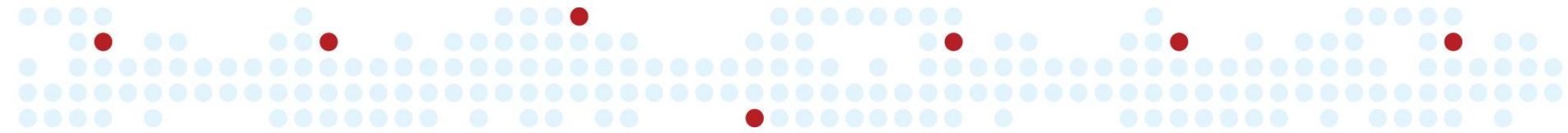
## Huge potential in the market segments addressed by Exiqon

- Below is listed addressable market for colon and lung cancer treatment selection
- World wide figures are 2.5x US figures
- Addressable US market for colon and lung amounts to \$538 mill at USD 2000\* per test

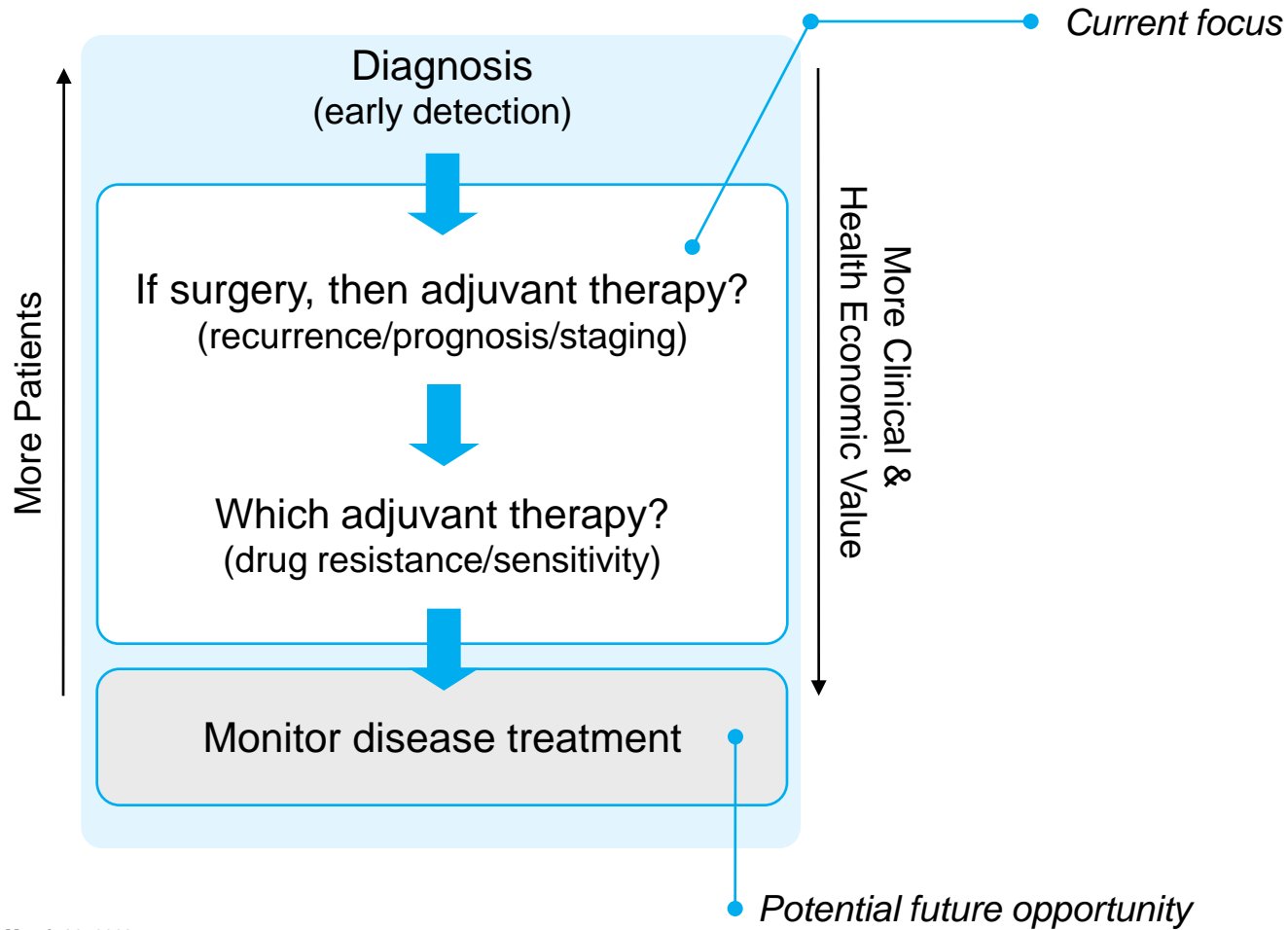
Indication	Recurrence test	Chemotherapy test
Colon	Stage II & III	Stage III & IV (some stage II)
Patients in the US**	54.000	40.000
Addressable market	\$108.000.000	\$80.000.000
Lung	Stage I, II & III	Stage II, III & IV (some stage I)
Patients in the US**	70.000	152.000
Addressable market	\$140.000.000	\$304.000.000

## Examples of personalized medicine applied in oncology

Product	Company	Test type	Application
EDR test	Exiqon & PTI	Cellular assay	Drug resistance testing
HER2 test	Several incl Ventana	<i>In situ</i> test	Eligibility of breast cancer patients for treatment with Herceptin
KRAS	Several	PCR of SNPs	Drug response to EGFR inhibitors (Colon & lung)
TPMT	Several	Genetic (SNP) or enzymatic	Dose of thiopurine drugs
UGT1A1	Third Wave	PCR (SNP)	Dose of Irinotecan
OncotypeDx	Genomic Health	21 mRNA profile based on PCR	Risk of recurrence and assesses the value of chemotherapy
AmpliChip CYP450	Roche	PCR of SNPs	Dosing decisions
Warferin metabolism	Several	PCR of SNPs	Warferin dosing decisions



## Monitor disease treatment is an unexploited potential of miRNA

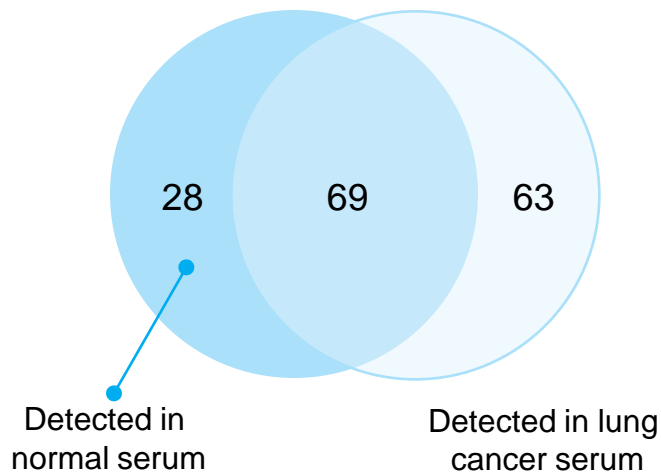


## miRNA in serum (blood)

### Background

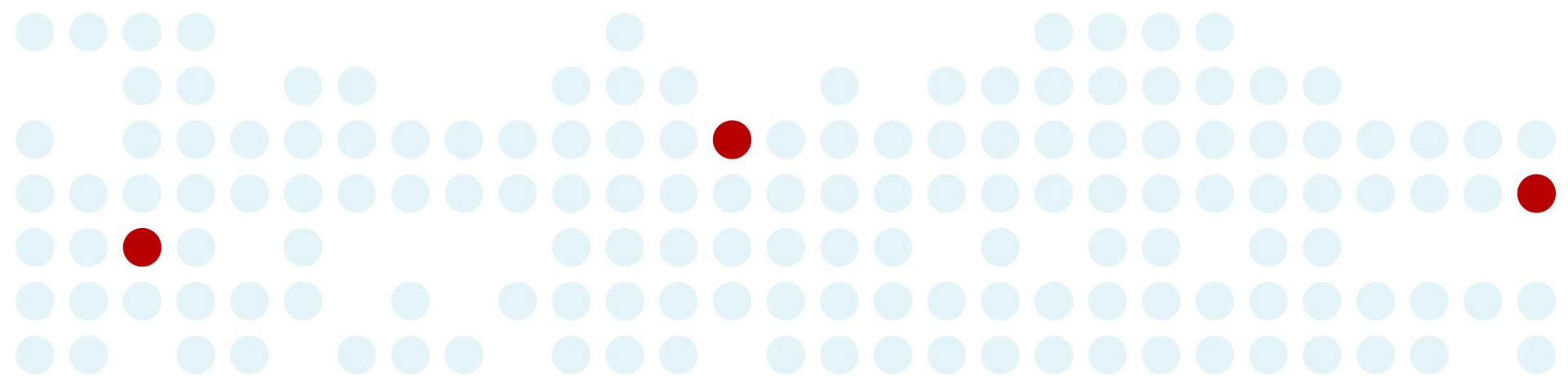
- miRNA can be detected in serum (extremely stable and shown to be a strong biological indicator)
- First publications appearing Q3 2008
- Exiqon has proof of concept for detection of miRNA in serum (array and qPCR)

miRNAs detected in serum



“We also identified specific expression patterns of serum miRNAs for lung cancer, colorectal cancer, and diabetes, providing evidence that serum miRNAs contain fingerprints for various diseases”

“This new approach has the potential to revolutionize present clinical management, including determining cancer classification, estimating prognosis, predicting therapeutic efficacy, maintaining surveillance following surgery, as well as forecasting disease recrudescence.”



Consolidation and Exiqon as potential acquisition target

## The “risk” of being “consolidated”: Potential acquirors of Exiqon

Significant consolidation occurs in the diagnostic industry: Over \$20bn in M&A occurred in 2007 for the diagnostics industry – the highest in 10 years\*

- **Roche:** Long standing relationship, wants to go into miRNA Life Science space and has interest in applying miRNA in personalized medicine (entrance into personalized medicine was the motivation for acquiring Ventana: *in situ* tests). Strong in acquiring companies. See next slide.
- **Qiagen:** Could apply both our Life Science and Diagnostics businesses in their business. Strong in acquiring companies (just acquired Digene and Corbett to strengthen their Dx business)
- **GE:** Is working on building a new molecular diagnostics business and is looking for emerging opportunities. Has a tradition of acquiring companies to gain access to new market opportunities.
- **Agilent:** Could readily apply the Life Science business (upon acquisition of Stratagene) and is moving into the diagnostics market segment
- **Illumina:** Has announced that they are moving into diagnostics. Illumina is dedicated to nucleic acid detection. Could apply our product offering in the Life Science and Diagnostics businesses
- **Siemens:** Has secured a leading position in diagnostics, through acquisitions.
- **Invitrogen:** Is moving into diagnostics. Has a strong tradition of acquiring companies and could apply both our Life Science and the Diagnostics businesses.

## Big diagnostics players are looking at miRNA



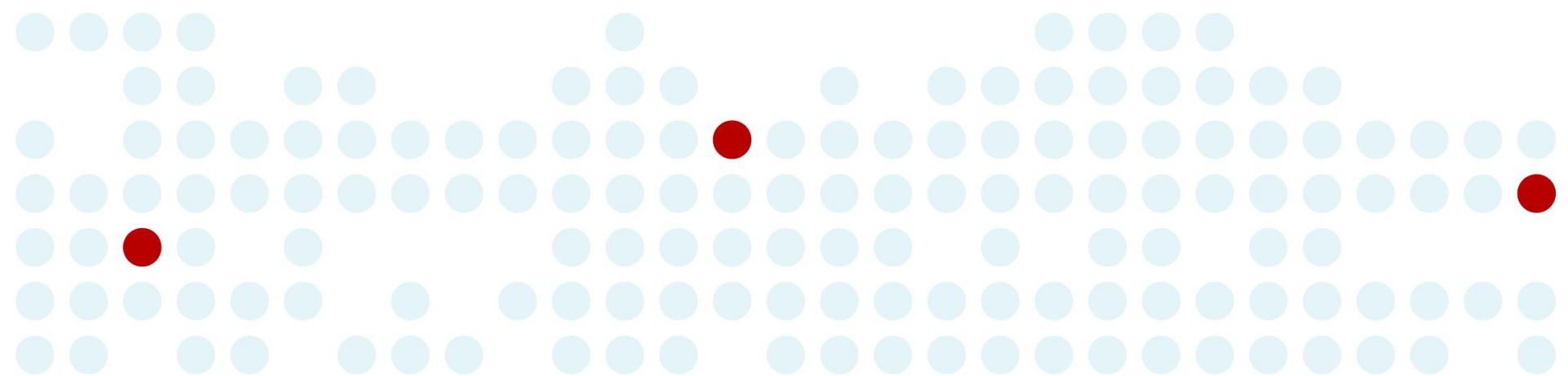
February 14, 2008

### Roche Aims to Move First RNAi Drug into Clinic in 2010; Keeps Eye on miRNA Space

At the same time, Roche has at least two research groups examining the diagnostic potential of the small, non-coding RNAs, he added. “As the largest diagnostics company in the world, we don’t have a deaf ear” to miRNA-based diagnostics, he said.

As such, Roche has not ruled out the possibility of using acquisitions to give a boost to its miRNA diagnostics research, Babiss said.

“There are different ways of doing [this]: one is to jumpstart it internally and the other way is to go out there and buy what you might need,” he added. Roche’s diagnostics group “knows how to do that work very well. They’re on top of it.”



## Appendices

## Competitive Landscape – Treatment Selection, CLIA Based

Company	Comments
Clariant	Publicly traded. Menu of non-proprietary products. Q2, 2008; \$17 mill. Struggling financially. Also treatment selection incl KRAS
Combimatrix	Publicly traded. Using arrays. Q2, 2008; \$0.4 mill in Dx revenue.
Genomic Health	Publicly traded. OncoPrint. Has shown >100% growth for the past 4 quarters. \$28 mill in Q2, 2008.
AviaraDX	Publicly traded. Just acquired by BioMerieux.
Genoptix	Publicly traded. Profitable and extreme growth. Non-proprietary tests. \$28 in Q2 2008 – strong growth
Genzyme Genetics	About \$100 mill in revenue from oncology testing.
Monogram Bioscience	Publicly traded. Many products in oncology (treatment selection). Approx \$15 mill in revenue in Q2, 2008.
Pathwork DX	Private. Offer CUP test (FDA approved). Just raised another \$20 mill.
Precision Therapeutics	Private. Biggest competitor in assays for drug resistance. Has successfully applied a very aggressive marketing strategy.
Caris Diagnostics	Private. Offers many SNP and expression analyses incl. KRAS mutational analysis

## Large unmet need to improve the cost of drug development

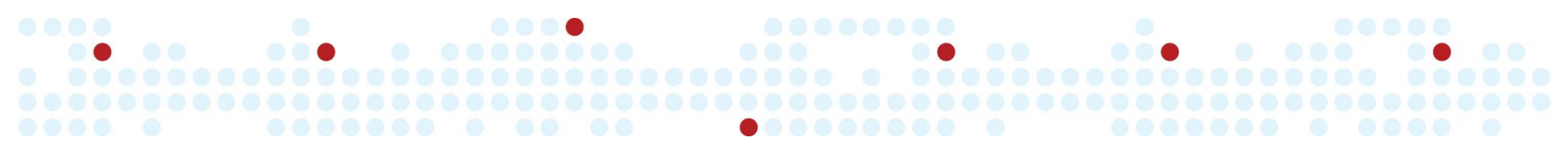
Savings in clinical trial costs ~ \$35 million

Income from 8 year acceleration of products ~ \$2.5 bn

Access to drug from acceleration ~ 120.000 patients

Experimental design	With HER2 test	Without test
Number of patients	470	2200
Response rate	50%	10%
Years follow-up	1.6	10

Source: Press and Seeling, Targeted Medicine 2004.

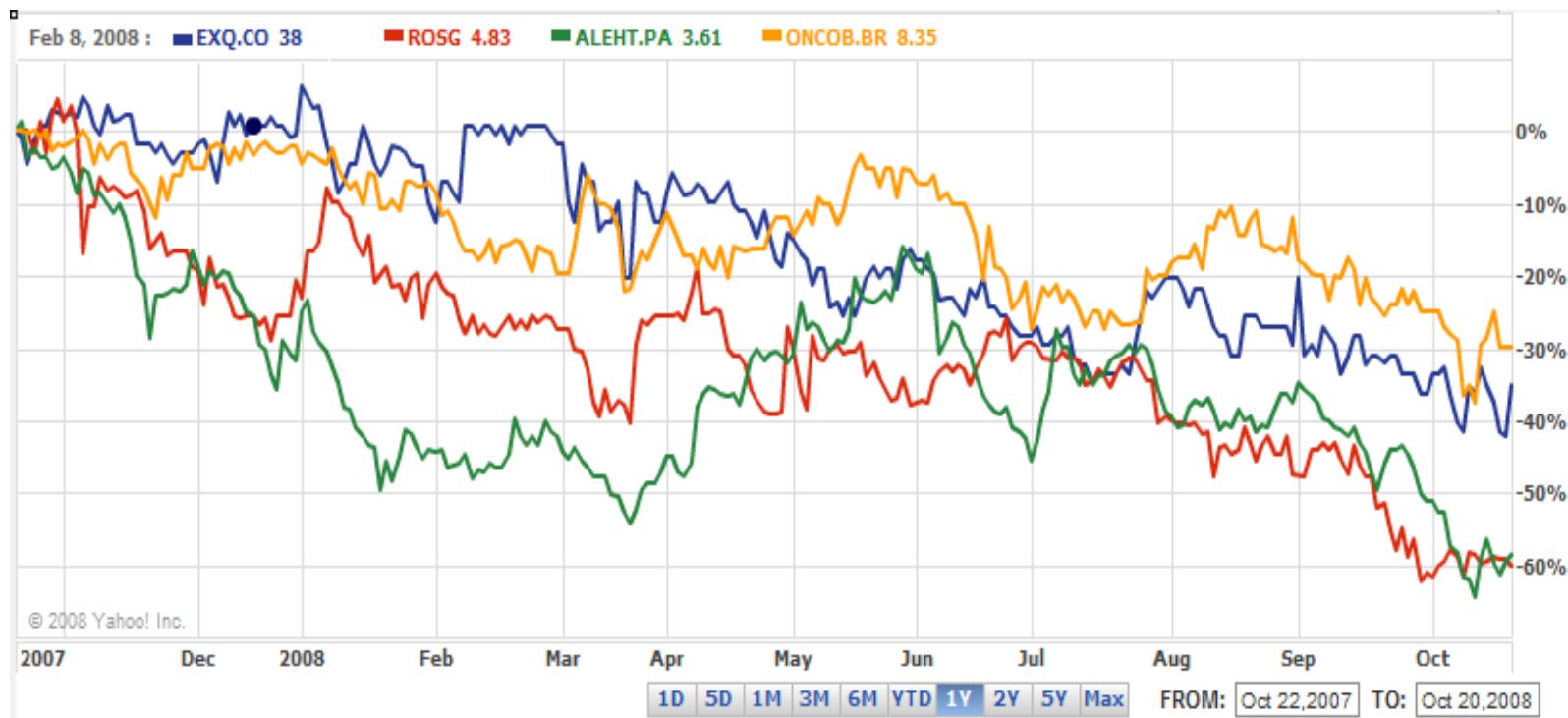


## Peers in Danish biotech (past year)



## International SmallCap biotech peers (past year)

Exiqon      Rosetta Genomics      Exonhit      Oncomethylome





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