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# Strengthened Financial Position Ahead of SPARKLE Phase 3 Study Headline Results in May 2024

## Q4 & FULL YEAR REPORT 2023

Conference call presentation on 9 February, 10:00 CET

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# Q4 & FULL YEAR REPORT 2023 INVESTOR CONFERENCE CALL

## Agenda

Ascelia Pharma highlights
Recent key events
Portfolio
Financials and priorities

## Presenters

CEO - Magnus Corfitzen
Deputy CEO - Julie Waras Brogren
CSO - Andreas Norlin


# ASCELIA PHARMA - HIGHLIGHTS

## Two drugs in advanced clinical development

### ORVIGLANCE® – Nearing completion of Phase 3

- First-in-class contrast agent for use in liver MRI in patients with severely impaired kidney function
- FDA Orphan Drug Designation
- Global addressable market of USD 800 million
- Phase 3 patient recruitment completed; readout by May 2024

### ONCORAL – Phase 2-ready

- Daily, oral irinotecan chemotherapy
- Clinical collaboration with Taiho Oncology
- Opportunity in gastric cancer and other solid tumors

## Global outlook and Nordic roots

Based in Malmö (Sweden), US affiliate in New Jersey (US)
Listed on NASDAQ Stockholm (Ticker: ACE)



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# Strengthened Financial Position Ahead of SPARKLE Phase 3 Study Headline Results in May 2024

## Key events in Q4 2023

- Ascelia Pharma gets acceptance for publication of Orviglance ® review article in Investigative Radiology
- Conversion of series C shares into ordinary shares for delivery to participants in incentive program and subsequent change in number of shares and votes
- Extraordinary General Meeting held on November 13, 2023 resolved on proposal to introduce an employee stock option program
- Ascelia Pharma starts image reading phase and re-confirms Phase 3 SPARKLE results by May 2024

## Key events after the period

- Nomination Committee appointed for the Annual General Meeting 2024
- Orviglance ® review article is published in Investigative Radiology
- Ascelia Pharma secures financing of up to SEK 35 million

## ORVIGLANCE®

**Liver diagnostic imaging drug**

## ONCORAL

Daily, oral chemotherapy

PORTFOLIO



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# CLEAR UNMET NEED AND CONSISTENT POSITIVE DATA

- A well-defined unmet need for liver imaging in cancer patients with poor kidney function
- A global addressable market opportunity of USD 800 million
- Consistent positive efficacy and safety in eight completed Phase 1 and 2 studies
- Commercial scale manufacturing in place
- Patient recruitment and MR image collection for SPARKLE Phase 3 study completed
  - Common adverse events were in line with previous studies
  - Efficacy conclusions available by May 2024

# ORVIGLANCE – FILLING AN UNMET NEED IN LIVER MRI

## Patient Landscape

### Liver metastases critical in cancer care

Liver metastases are common in many cancer types and often the cause of mortality [1-3]

- Colorectal cancer, metastatic breast cancer, gastric cancer

## Treatments

### Contrast enhanced MRI is the gold standard

Contrast enhanced MRI

- Detection and visualization
- Surgery & drug treatment plan
- Post-treatment surveillance

## Unmet Need

### A role for ORVIGLANCE in patients with kidney impairment

Patients with healthy kidneys

- Receive MRI with gadolinium-based contrast agent (GBCA)

Patients with severe kidney impairment

- Black Box warning for gadolinium contrast agents
- Risk of severe side effects, incl. Nephrogenic Systemic Fibrosis

**ORVIGLANCE**

Aims to be the imaging option without gadolinium-related safety risks patients with poor kidney function

- Manganese based
- Liver specific


1) Riihimäki, M. *et al.* Patterns of metastasis in colon and rectal cancer. *Sci. Rep.* 6, 29765; doi: 10.1038/srep29765 (2016); Journal of Pathology, 2014, 232:23-31
2) Guy diSibio and Samuel W. French (*2008*) Metastatic Patterns of Cancers: Results From a Large Autopsy Study. Archives of Pathology & Laboratory Medicine: June 2008, Vol. 132, No. 6, pp. 931-939
3) Rahbari et al. Metastatic Spread Emerging From Liver Metastases of Colorectal Cancer: Does the Seed Leave the Soil Again? Annals of Surgery: February 2016 - Volume 263 - Issue 2 - p 345–352




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# CLINICAL DATA PACKAGE FOR REGULATORY SUBMISSION

## Consistent positive efficacy and safety in completed studies[1-7]

Completed program of 8 studies in 201 patients and healthy volunteers

## Pivotal results pending

85 patients recruited


### Eight Studies Completed [1-7]

**Evaluating safety and efficacy**

Totally 201 patients and healthy volunteers

### Evaluation Before Phase 3

**Re-read of efficacy across all studies**

140 patients (72 study subjects and 68 compassionate use program)

### Re-Evaluation Orviglance vs. Gadolinium & Unenhanced

**Re-read by 3 blinded readers** of 20 patients with liver metastases

### Food Effect Study

**Effect of food intake** on absorption and signal intensity (39 subjects)

### Hepatic Impairment Study

**Effect of liver impairment** on safety and pharmacokinetics (35 subjects)

### Phase 3
### Pivotal Study SPARKLE

**Safety and efficacy**
in target patient population
(85 patients)


1) Thomsen HS *et al*, Acad Radiol 2004: 11: 630-636
2) Thomsen HS et al. Eur Radiol 2007, 17: 273-278
3) Rief M *et al*. Invest Radiol. 2010; 45: 565-71
4) Brismar TB *et al*.. Eur Radiol 2012; 22:633-41
5) Albiin N *et al*. MAGMA. 2012; 25:361-368
6) Study CMC-P005, primary objective to study of Orviglance for imaging of bile ducts (not published)
7) Results from Phase 1 and 2 and Food Effect and Hepatic Impairment Studies presented at RSNA and ESGAR conferences between 2022 and 2023




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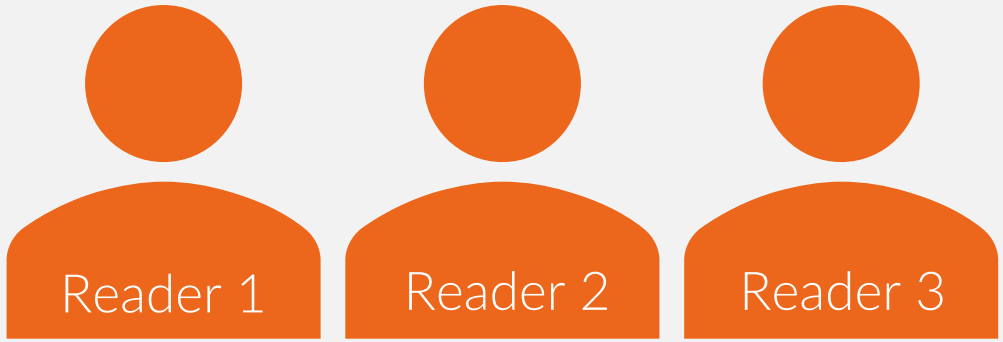
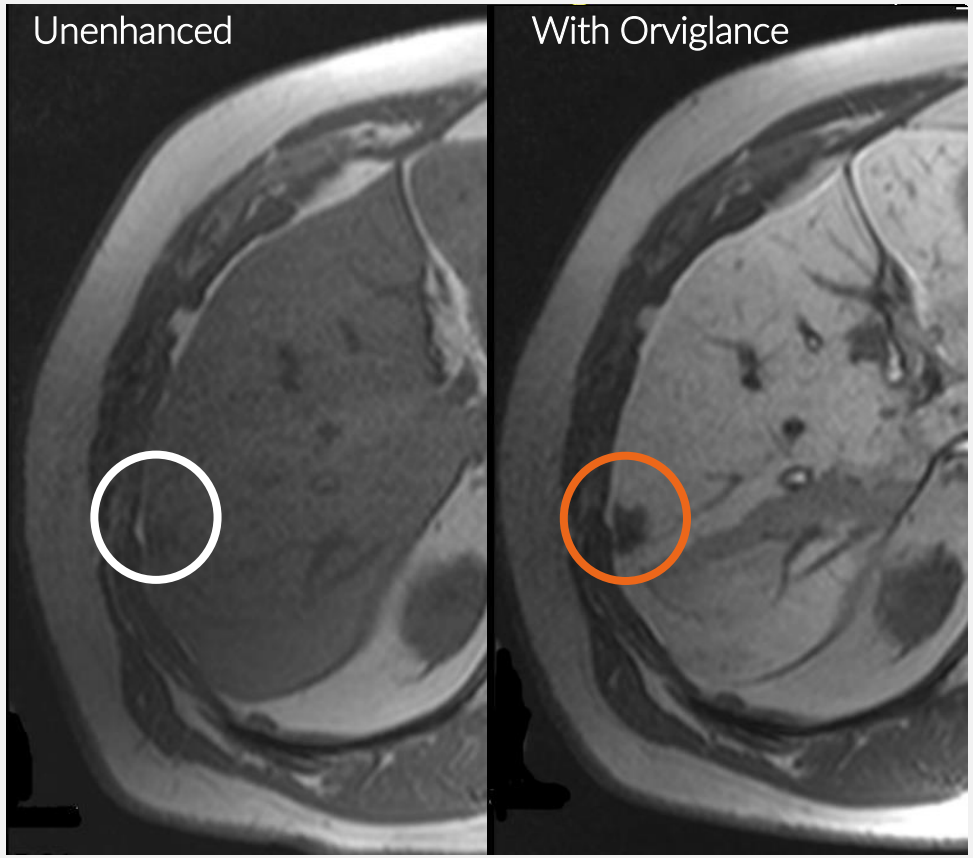
# SPARKLE SUCCESS DETERMINED BY IMPROVED VISUALIZATION

## Criteria for statistical test of primary endpoint




**Primary endpoint is met if 2 out of 3 independent radiologists** rate both lesion border delineation and lesion contrast for Orviglance MRI higher than unenhanced MRI with statistical significance




Images from Study CMC-P002, patient with colon cancer and liver metastasis.



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# ASCELIA EXPERIENCE WITH EVALUATION METHODOLOGY

| | Number of Patients | Liver Lesion Types* | Number of Radiologist Readers | Primary Endpoint | Orviglance Superior to Unenhanced | Statistical Significance |
|---|---|---|---|---|---|---|
| P004A Re-read | 20 | Metastases | 3 | Co-primary: Border delineation; Lesion contrast | Yes | P=0.009 |
| Phase 3 Pivotal Study SPARKLE | 85 | Known or suspected lesion (metastases, primary tumors, benign lesions) | 3 | Co-primary: Border delineation; Lesion contrast | ? | ? |

* Apart from the difference in population heterogeneity, other differences between P004A Re-read and SPARKLE includes dose and use of Diffusion Weighted Imaging in SPARKLE



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# HEADLINE RESULTS ON TRACK FOR READOUT BY MAY 2024

## Data collected with results of image re-evaluation pending

- **Clinical data collected** from 85 patients; no further patient enrollment required
- **Common adverse events** in line with previous studies
- **Re-evaluation of all images required** due to unreliable scoring by two readers with high intra-reader variability

*Intra-Reader Variability Assessment*

*1st evaluation* ≠ *2nd evaluation*

## Re-evaluation on track

- **Re-evaluation designed** to secure reader consistency
- **Readers selected** and trained
- **Reading and monitoring initiated** early December
- Headline results on track for read-out by May



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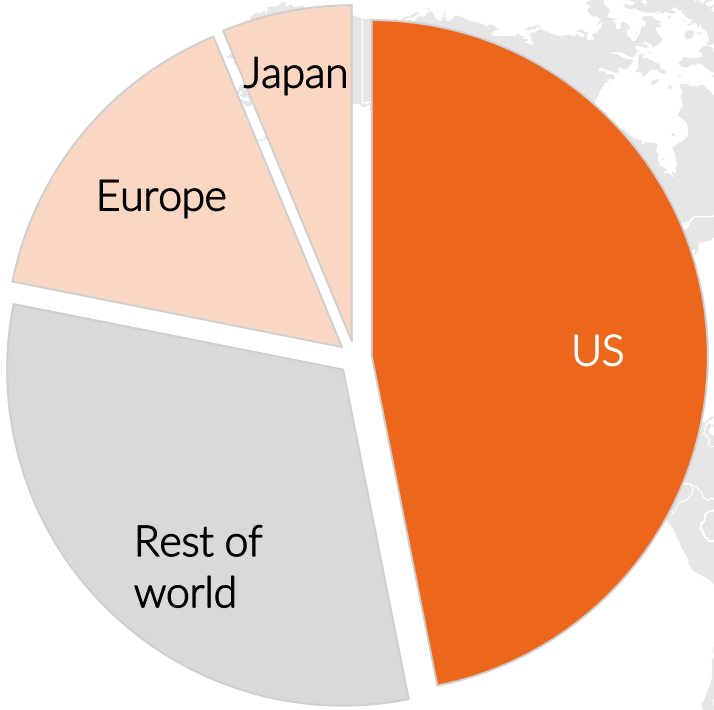
# ATTRACTIVE ADDRESSABLE MARKET




Global addressable market of USD 800 million

**Focused launch** for well-defined patient population in liver imaging in cancer patients with severe kidney impairment

**Global commercialization through partners** with potential for Ascelia led launch in the US

Sources:
Ascelia Pharma market research on real-world volumes with Decision Resources Group, 2020.. Ascelia Pharma market access research and analyses with Revenue Reimbursement Solutions and Charles River Associates (2020), Triangle (2022) and Trinity (2022), incl. 75 stakeholder and expoert interactions. 1) Final pricing strategy is subject to Phase 3 data, payer evidence, negotiations, discounts and access strategy

# ATTRACTIVE US OPPORTUNITY

**Abdominal imaging procedures in cancer patients with severe kidney impairment (CKD 4/5/AKI)** based on epidemiology and real-world data[1]

**Pricing range benchmarks** based on innovative diagnostics, payer and expert input and price testing[2, 3]

**Volume growth driven** by demographics as well as prevalence and long-term care in cancers and kidney disease

~100,000
procedures annually

$3,000-4,500

4-5%
vol. annually

Sources:
1) Ascelia Pharma market research with Decision Resources Group, 2020. Literature on prevalence and epidemiology of kidney disease, cancer and liver metastases.
2) Ascelia Pharma market access research and analyses with Revenue Reimbursement Solutions and Charles River Associates (2020), Triangle (2022) and Trinity (2022)
3) Final pricing strategy is subject to Phase 3 data, payer evidence, negotiations, discounts and access strategy

# UNMET NEED RECOGNIZED

## NSF* risk
with warnings for target population

+90%

of HCPs are concerned by issues relating to GBCAs (including NSF)

+16%

of providers have experienced GBCA-induced NSF

*nephrogenic systemic fibrosis

Source: Independent research by Two Labs Pharma Services for Ascelia Pharma in the USA conducted in Q4 2021/Q1 2022 included 16 in depth interviews and a survey of 254 healthcare professionals (HCPs), including 154 radiologists, 50 nephrologists and 50 oncologists. Participants in the study were asked about their choices of imaging and contrast agents in patients with cancer



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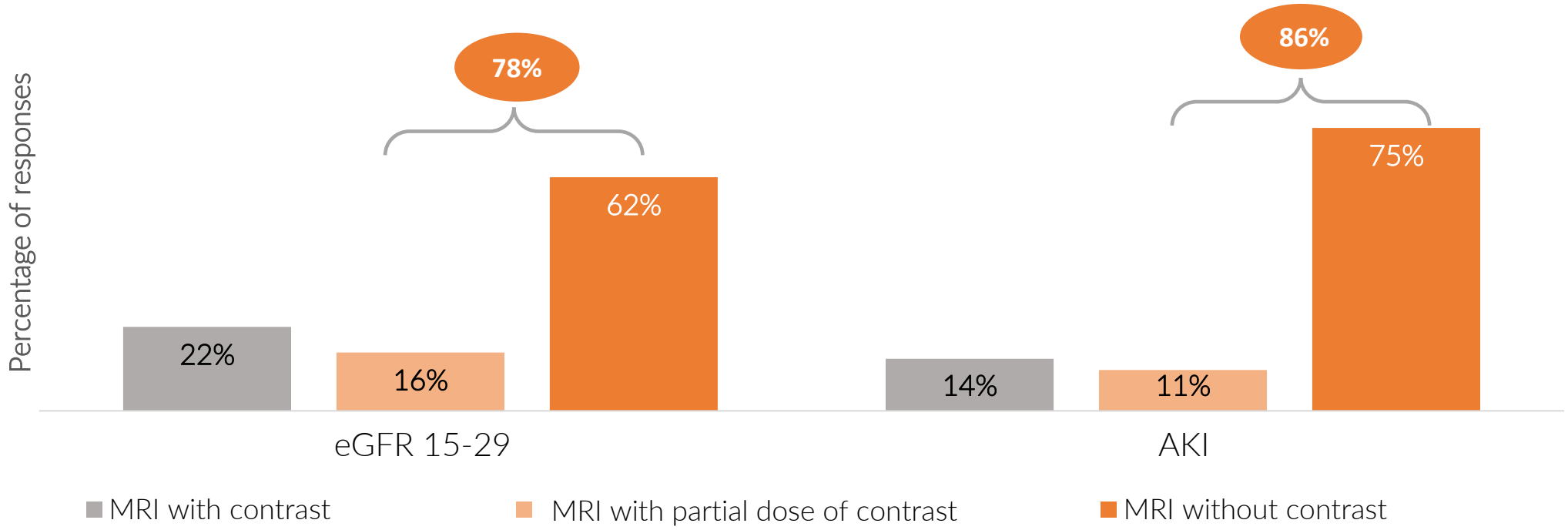
# UNENHANCED MRI IS PREFERRED FOR TARGET PATIENTS

78% physicians prefer MRI without or with partial dose contrast for patients with poor kidney function (low eGFR)

... even more for patients with acute kidney injury (AKI)




Source: Independent research by Two Labs Pharma Services for Ascelia Pharma in the USA conducted in Q4 2021/Q1 2022 included 16 in depth interviews and a survey of 254 healthcare professionals (HCPs), including 154 radiologists N =103 oncologist, nephrologist, and radiologist responses.
Q: Please assign priority to the imaging tests in the sequence or order in which you would recommend or perform them



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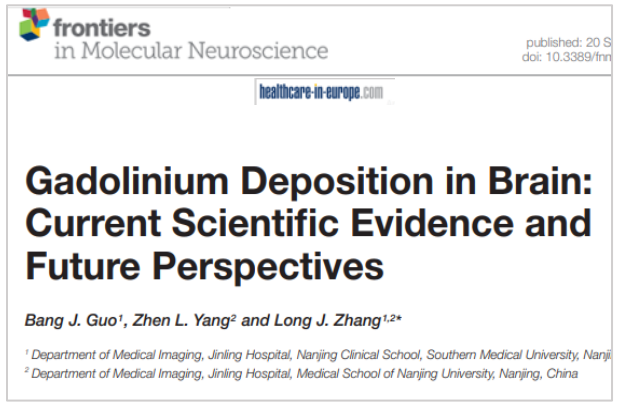
# MOMENTUM FOR AN ALTERNATIVE TO GADOLINIUM

## Deposition in brain & organs
concerns around safety for all patients

New safety category recommended for Symptoms Associated with Gadolinium exposure (SAGE), by Am. College of Rad. (2022)

Multiple-GBCA effect on body movement and mental skills study requested by the FDA (ODYSSEY, 2020)


frontiers in Molecular Neuroscience

published: 20 S
doi: 10.3389/fnn

healthcare-in-europe.com

**Gadolinium Deposition in Brain: Current Scientific Evidence and Future Perspectives**

*Bang J. Guo[1], Zhen L. Yang[2] and Long J. Zhang[1,2]*

[1] Department of Medical Imaging, Jinling Hospital, Nanjing Clinical School, Southern Medical University, Nanji
[2] Department of Medical Imaging, Jinling Hospital, Medical School of Nanjing University, Nanjing, China

## Water contamination
scrutiny of environmental impact

Gadolinium is excreted in urine. Hard to remove in our sewage systems, it is discharged into our environment and drinking water

"The increasing use of gadolinium-based contrast agents (GBCAs) for MRI is leading to widespread contamination of freshwater and drinking water systems"[1]


## Future with less/no gadolinium
focus of leading gadolinium manufactures

Low dose full-body gadolinium contrast agents

- FDA approved in priority review (2022) and EMA (2023) approved (gadopiclenol, Guerbet/Bracco)
- Initiation of Phase 3 (gadoquatrane, Bayer 2023)

Completion of Phase 1 patient enrollment in full-body IV manganese-based contrast agent (GE HealthCare 2023)


1) Brünjes R. et al. Anthropogenic gadolinium in freshwater and drinking water systems, Water Research, Volume 182, 2020..
Other sources include:
Macke et al. Fast and automated monitoring of gadolinium-based contrast agents in surface waters, Water Research, Volume 207, 2021.
Oluwasola et al, Gadolinium based contrast agents (GBCAs): Uniqueness, aquatic toxicity concerns, and prospective remediation. Journal of Contaminant Hydrology, Volume 250, 2022.
M. Nicholl. Seeking alternatives to gadolinium-based contrast agents. Healthcareineurope.com. July 22022
Bang G. Gadolinium Deposition in Brain: Current Scientific Evidence and Future Perspectives. Mol. Neurosci., 20 September 2018.

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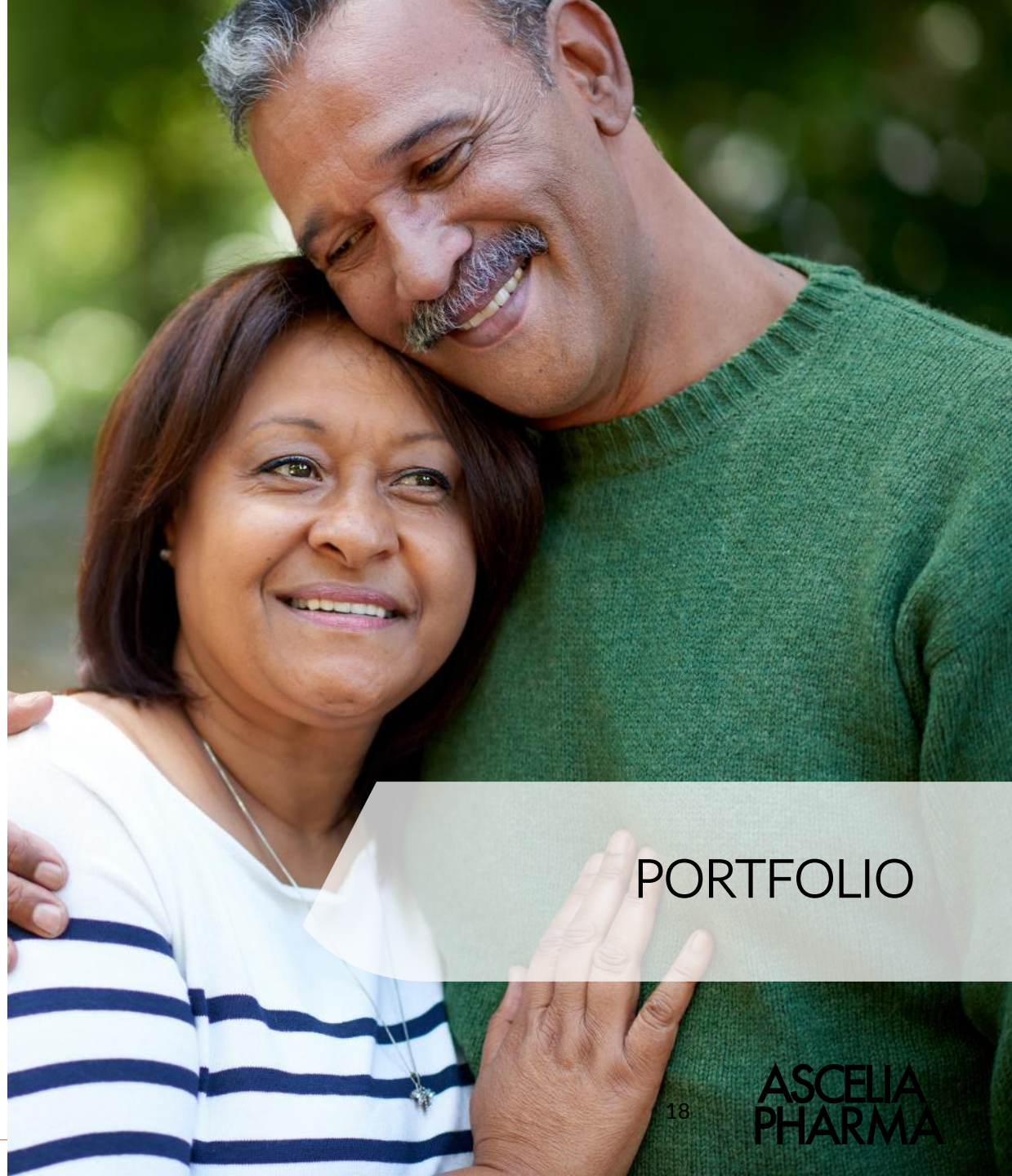
## ORVIGLANCE®

Liver diagnostic imaging drug

## ONCORAL

**Daily, oral chemotherapy**



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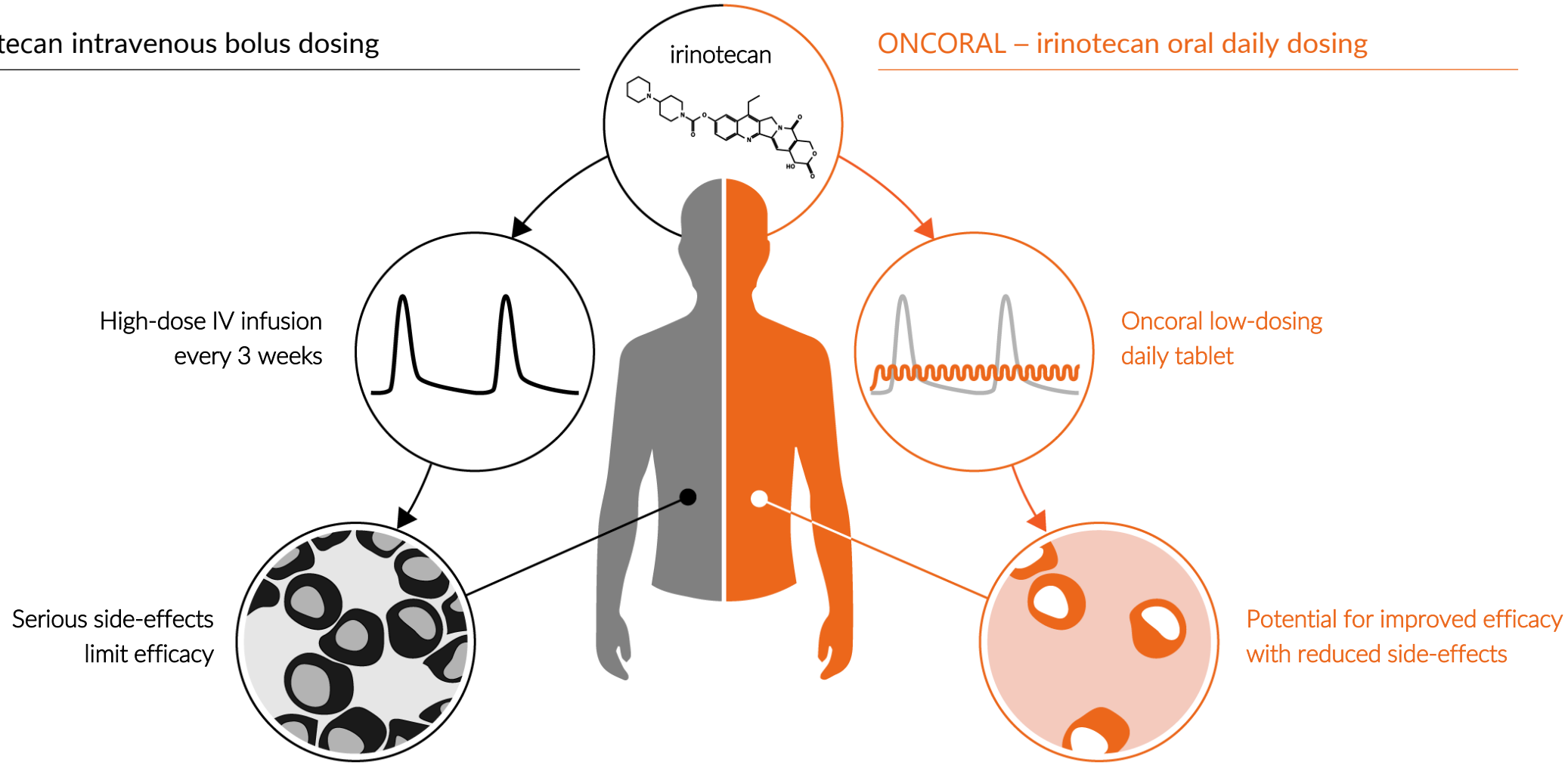
# IMPROVING IRINOTECAN **EFFICACY** and **TOLERABILITY**

Irinotecan intravenous bolus dosing

ONCORAL – irinotecan oral daily dosing

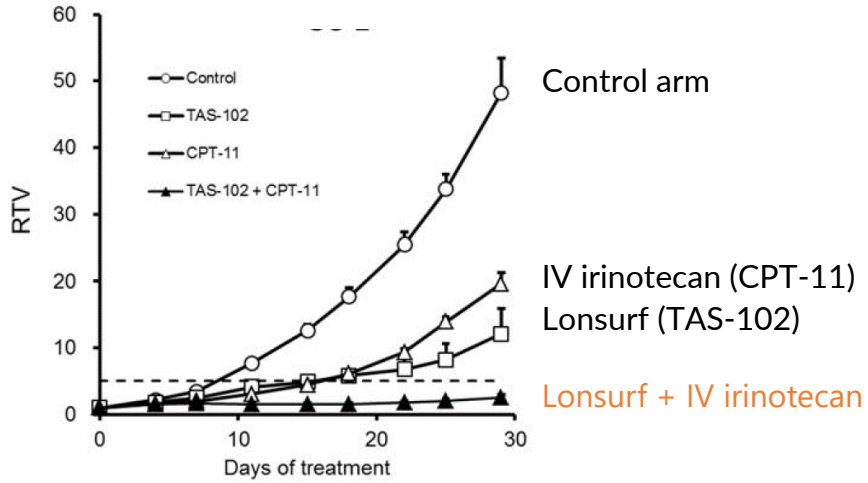


# ONCORAL PHASE 2 IN GASTRIC CANCER

## STRONG RATIONALE FOR GASTRIC CANCER

- High unmet need and clinically demonstrated
- Potential for synergistic effect between Lonsurf and irinotecan

Efficacy study in an animal model of gastric cancer[1]
(Relative Tumor Volume, RTV)




Control arm

IV irinotecan (CPT-11)
Lonsurf (TAS-102)

Lonsurf + IV irinotecan

## LONSURF AND IRINOTECAN COMBINATION

RANDOMIZED CONTROLLED PHASE 2 STUDY

- ~100 patients with metastatic gastric cancer
- Study arms: Oncoral + Lonsurf vs. Lonsurf
- Endpoints: Progression Free Survival (Primary), Response Rate, PK, Safety (Secondary) and Overall Survival (follow-up)
- IND approved in the US
- To focus all resources on Orviglance, patient enrollment is not initiated until it can be done effectively

Clinical collaboration with

TAIHO ONCOLOGY

LONSURF is approved for treatment of metastatic gastric cancer and metastatic colorectal cancer

1) Nukatsuka et al: Combination Chemotherapy Using TAS-102 and Irinotecan Hydrochloride, ANTICANCER RESEARCH 35: 1437-1446 (2015)



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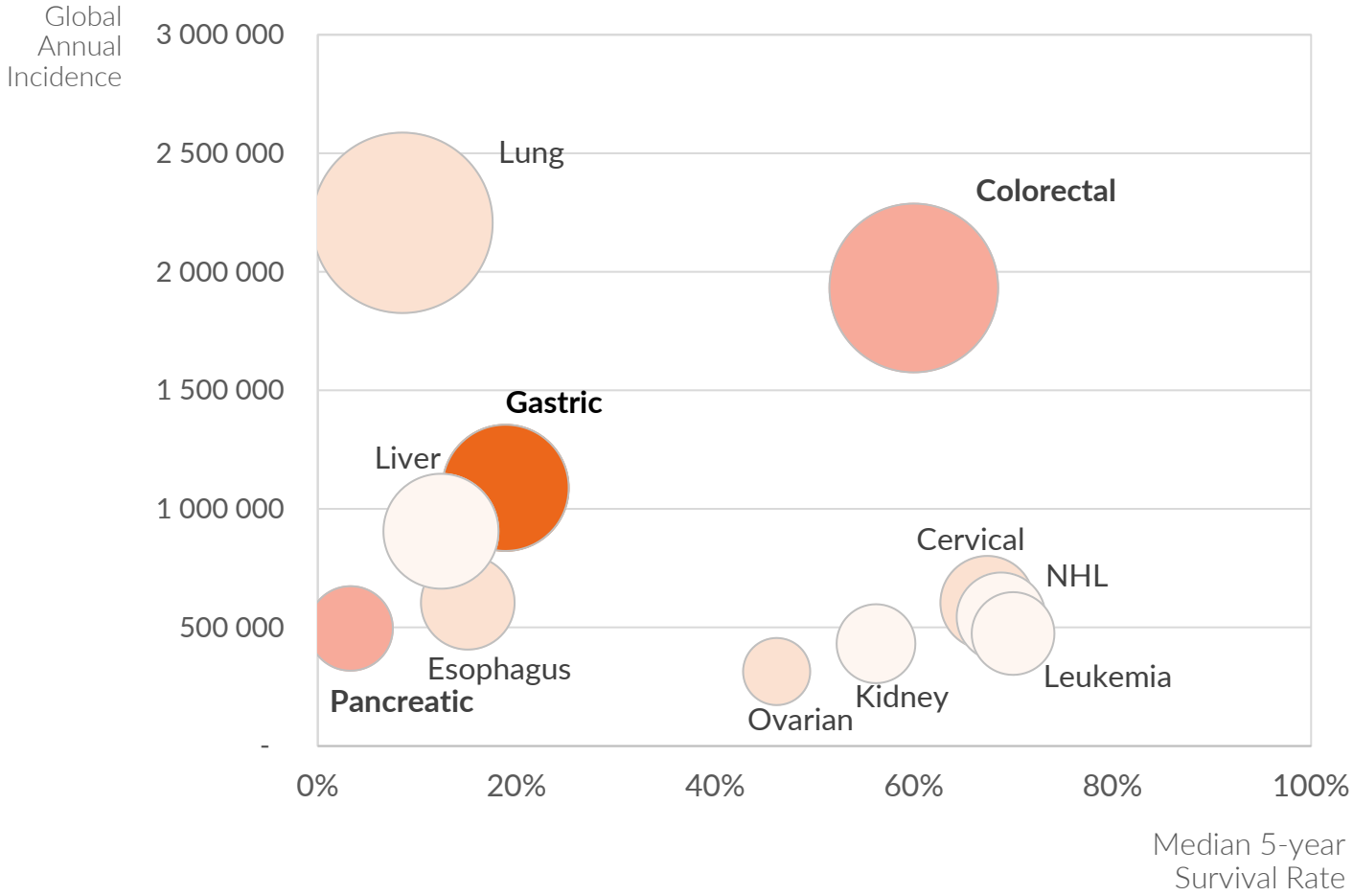
# HIGH VALUE OPPORTUNITY IN GASTRIC CANCER AND EXPANSION

## POTENTIAL FOR ORAL, DAILY DOSING OF IRINOTECAN[3]




### A WELL-ESTABLISHED CHEMOTHERAPY
with recognized anti-tumor effect in solid tumors

- Current focus: **Gastric cancer**
  - Clinically demonstrated
  - Guidelines recognized
  - 3rd highest cancer deaths[1]
  - Orphan disease (US and EU)
  - $3-4bn market[2]
- **Approved indications** for IV irinotecan
- Indications where IV irinotecan are clinically demonstrated & guidelines recognized
- Indications where IV irinotecan are clinically demonstrated

1) International Agency for Research on Cancer (IARC, 2021)
2) GlobalData - Gastric and Gastroesophageal Junction Adenocarcinoma – Global Drug Forecast and Market Analysis to 2024
3) Globocan 2020, WHO, Cancer Research UK



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# FINANCIALS & OUTLOOK

# ASCELIA PHARMA SECURES FINANCING OF UP TO SEK 35 MILLION

**PRESS RELEASE**

04 February 2024 20:54:00 CET

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## Ascelia Pharma Secures Financing of up to SEK 35 Million

**Ascelia Pharma AB (publ) (ticker:ACE) ("Ascelia Pharma" or the "Company"), a biotech focused on improving the life of people living with rare cancer conditions, today announced that the board of directors has resolved on a directed issue of convertibles to Formue Nord Fokus A/S ("Formue") raising gross proceeds of SEK 15 million (the "Convertibles"). Further, the Company has also entered into an agreement with Formue for a loan facility of up to SEK 20 million (the "Loan Facility" and together with the Convertibles, the "Financing"). The transaction ensures financial and strategic flexibility, with the full Financing extending the cash runway into the second quarter of 2025.**

## Strengthened financial position

- Ensures financial and strategic flexibility
- Extends cash runway into Q2 2025 with the full financing
- Limited dilution of current shareholders (around 4 percent)

## Attractive and competitive terms

- First tranche financing of SEK 20 million
  - SEK 15 million is convertibles (10.53 SEK per share)
  - SEK 5 million loan
- Second tranche loan of up to SEK 15 million

- Repayment by 20 May 2025, with option to repay at any time at no additional costs

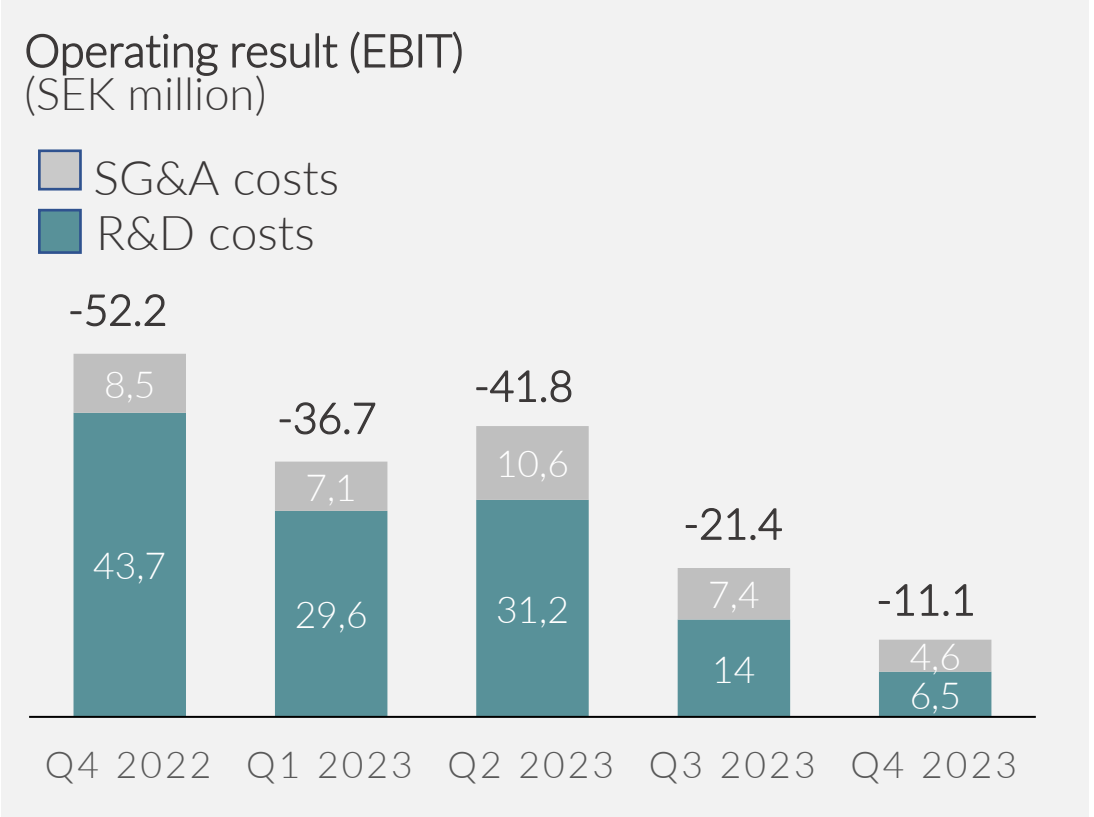
# OPERATING RESULT– LOWERED OPERATING EXPENSES

Operating loss of SEK 11 million in Q4 2023

**Significant decrease in loss (costs)** compared to Q3 2023 driven by:

- Completion of SPARKLE patient recruitment
- Effect of cost-cutting initiatives, including organizational reduction
- Focus on image re-evaluation with other activities on hold

Maintained lower cost level expected into 2024




Notes:
1) Other operating income and other operating costs added to SG&A

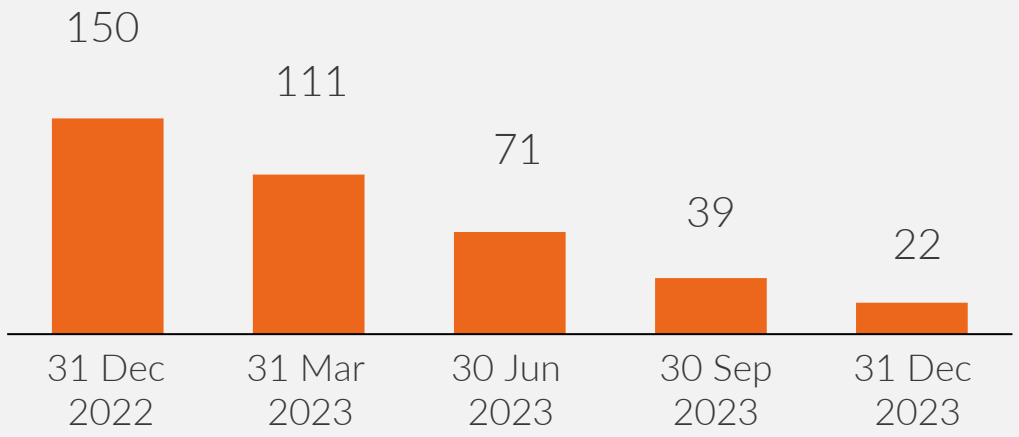
# LIQUIDITY - CASH RUNWAY INTO Q2 2025

**Liquid assets of 22 MSEK** (31 Dec 2023)

**Runway into Q2 2025 with full additional SEK 35 million financing** covering

- Headline results read-out from SPARKLE Phase 3
- Completion of time critical activities for the New Drug Application (NDA) for the US Food and Drug Administration (FDA)

**Liquid assets including marketable securities**
(SEK million)



# SUBSTANTIAL VALUE CREATION OPPORTUNITIES

## ORPHAN ONCOLOGY FOCUSED WITH TWO DRUGS IN CLINICAL DEVELOPMENT

### ORVIGLANCE

- ✓ First-in-class orphan diagnostic drug targeting $800m market
- ✓ Consistent positive efficacy and safety data; incl. significantly improved visualization (Phase 3 endpoint) in a 20-patient phase 2 study (p=0.009)
- ✓ Phase 3 patient recruitment completed

### 2024 FOCUS

- Phase 3 headline results on track for read-out by May 2024
- Progress US FDA NDA file
- Progress launch readiness including options for partnering

### ONCORAL

- ✓ Phase 2 ready oral daily irinotecan with potential in gastric cancer and other solid tumors

### 2024 FOCUS

- Prepare for initiating Phase 2 study when financing allows



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