

A photograph of an elderly man and woman in a grassy field flying a kite. The man, in the foreground, is wearing a light blue shirt and has a white beard. He is holding the string of a red kite high in the air. The woman, in the background, is wearing a striped shirt and is also smiling. The background shows rolling green hills under a clear blue sky.

ADVANCING
ORPHAN
ONCOLOGY

Ticker symbol: ACE
Nasdaq Stockholm
www.ascelia.com

Primary Endpoint Met with Strong Headline Results in Orviglance® Phase 3 Study

Quarterly Report Q1 2024

Conference call presentation on 16 May 2024, 10:00 CET

**ASCELIA
PHARMA**

FORWARD LOOKING STATEMENTS

This presentation, which includes all information and data on the following slides, any oral statements made when presenting these slides, and any other material distributed or statements made at, or in connection with, such presentation (the “Presentation”), relates to Ascelia Pharma AB (publ) (hereinafter, together with its subsidiaries, the “Company”) is furnished to you solely for your information and may not be reproduced or redistributed, in whole or in part, to any other person without the prior written consent of the Company. You should not rely upon it or use it to form the definitive basis for any decision, contract, commitment or action whatsoever, with respect to any transaction or otherwise.

The information included in this Presentation may contain certain forward-looking statements relating to the business, financial performance and results of the Company and/or the industry in which it operates. Forward-looking statements concern future circumstances and results and other statements that are not historical facts, sometimes identified by the words “believes”, “expects”, “predicts”, “intends”, “projects”, “plans”, “estimates”, “aims”, “foresees”, “anticipates”, “targets”, and similar expressions. The forward-looking statements contained in this Presentation, including assumptions, opinions and views of the Company or cited from third party sources are solely opinions and forecasts which are subject to risks, uncertainties and other factors that may cause actual events to differ materially from any anticipated development. Neither the Company nor any of its affiliates, directors, employees or advisors provides any assurance that the assumptions underlying such forward-looking statements are free from errors nor do any of them accept any responsibility for the future accuracy of the opinions expressed in this Presentation or the actual occurrence of the forecasted developments. This Presentation speaks as of the applicable reporting date, and there may have been changes in matters which affect the Company subsequent to the date of this Presentation. Neither the issue nor delivery of this Presentation shall under any circumstance create any implication that the information contained herein is correct as of any time subsequent to the date hereof or that the affairs of the Company have not since changed, and the Company does not intend, and does not assume any obligation, to update or correct any information included in this Presentation.

Each person should make their own independent assessment of the merits of the Company and should consult their own professional advisors. By receiving this Presentation, you acknowledge that you will be solely responsible for your own assessment of the market and the market position of the Company and that you will conduct your own analysis and be solely responsible for forming your own opinion of the potential future performance of the Company's business.



QUARTERLY REPORT Q1 2024 INVESTOR CONFERENCE CALL

Agenda

Ascelia Pharma highlights

Recent key events

Portfolio

Financials and priorities ahead

Presenters

CEO - Magnus Corfitzen

Deputy CEO - Julie Waras Brogren

CSO - Andreas Norlin



At Ascelia Pharma, we identify, develop and commercialize novel drugs that address unmet needs of people with rare cancer conditions

ASCELIA PHARMA - HIGHLIGHTS

Pipeline

ORVIGLANCE® – Registration phase

- 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 study successful and clinical development completed

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)

Q1 2024 PROGRESS

Key events in Q1 2024

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

Key events after the period

- ▶ SPARKLE image reading completed with expected headline results first half of May 2024
- ▶ Ascelia Pharma draws down SEK 15 million second tranche under existing loan
- ▶ **Primary endpoint successfully met with strong headline results in Orvigance phase 3 study**
- ▶ Bulleting from the Annual General Meeting in Ascelia Pharma AB on 6 May 2024
- ▶ Ascelia Pharma hosts Investor Update: Bringing Orvigance to Patients



ORVIGLANCE SUCCESSFULLY MEETS PRIMARY ENDPOINT

- Phase 3 study demonstrated strong superiority in visualization of focal liver lesions with Orviglance (CMRI) compared to unenhanced MRI
- All three readers meet the primary endpoint ($p < 0.001$) with statistical significance
- The reliability of the data is strong and conclusive for all readers – including variability

PRESS RELEASE

02 May 2024 11:12:00 CEST

**ASCELIA
PHARMA**

Ascelia Pharma Successfully Meets Primary Endpoint with Strong Headline Results in Orviglance Phase 3 Study

Ascelia Pharma AB (publ) (ticker: ACE), a biotech focused on improving the life of people living with rare cancer conditions, today announced that liver imaging drug candidate, Orviglance®, significantly improved visualization of focal liver lesions, successfully meeting the primary endpoint in the pivotal Phase 3 study SPARKLE. Investors and analysts are invited to the virtual Investor Update: “Bringing Orviglance to Patients”, on Tuesday, 7 May at 14:00 CEST

ATTRACTIVE ORVIGLANCE OPPORTUNITY

- A well-defined unmet need for liver imaging in cancer patients with impaired kidney function
- A global addressable market opportunity of USD 800 million
- Clinical development completed with 9 studies and strong phase 3 results
- Commercial scale manufacturing
- Orviglance advances to regulatory filing and approval phase



SUBSTANTIAL VALUE CREATION OPPORTUNITIES

	 Advance Orviglance to approval	 Progress Orviglance commercialization readiness	 Develop pipeline potential
Objectives	Timely submission and approval by the US FDA as an orphan drug with an optimal label for the use in the target population	Focused launch for well-defined patient population Global commercialization through partners	Demonstrate Oncoral efficacy and safety in Phase 2 Expand Orviglance franchise with 2 nd generation
Milestones	<ul style="list-style-type: none">▪ Full Clinical Study Report early Q4 2024▪ Conclusions from FDA pre-submission meeting by Q1 2025▪ NDA submission mid-2025	<ul style="list-style-type: none">▪ Advance launch readiness▪ Commercialization partnership	<ul style="list-style-type: none">▪ Initiate Oncoral Phase 2 clinical study when financing allows

ORVIGLANCE®

Liver diagnostic imaging drug

ONCORAL

Daily, oral chemotherapy

PORTFOLIO



ORVIGLANCE – FILLING AN UNMET NEED IN LIVER MRI

Patient Landscape

Liver metastases are critical in cancer care



Liver metastases are common in many cancer types and often the cause of mortality¹⁻³

- Colorectal cancer, metastatic breast cancer, gastric cancer

Treatments

Contrast enhanced MRI is the gold standard

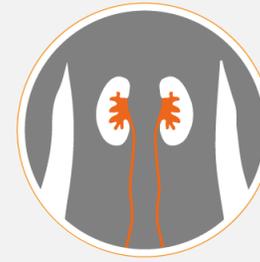


Contrast enhanced MRI

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

Unmet Need

A role for ORVIGLANCE in patients with severe 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 (NSF)

ORVIGLANCE

Aims to be the imaging option without gadolinium-related safety risks in patients with severe kidney impairment

- 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

STRONG SUPERIORITY OF ORVIGLANCE IN PHASE 3

Primary endpoint met successfully

- Three out of three readers scored primary visualization variables significantly higher with Orviglance than without



- Common adverse events were consistent with previous studies, such as mild to moderate nausea. No serious adverse drug reactions were observed

Orviglance clinical development completed

- Primary efficacy and safety of Orviglance confirmed in pivotal Phase 3
- Consistent positive efficacy and safety results across all nine studies
- Phase 3 full study report in progress for early Q4 2024

CLINICAL DATA PACKAGE FOR REGULATORY SUBMISSION



Nine studies with consistent positive efficacy and safety results¹⁻⁷

286 patients and healthy volunteers

Phase 1 studies investigating safety, absorption and signal intensity

Total 4 studies with 126 healthy volunteers

Phase 2 studies investigating efficacy and safety in patients with known metastases

Total 4 studies with 75 patients

Re-Evaluation Orviglance vs. Gadolinium & Unenhanced

Re-read with same endpoint as in phase 3 of phase 2 study (20 patients) with liver metastases

Phase 3 study to confirm efficacy and safety in the target population

Pivotal study on visualization of focal liver lesions and safety in patients with severe kidney impairment (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

ADVANCING ORVIGLANCE TOWARDS APPROVAL

Clinical



Nonclinical



CMC

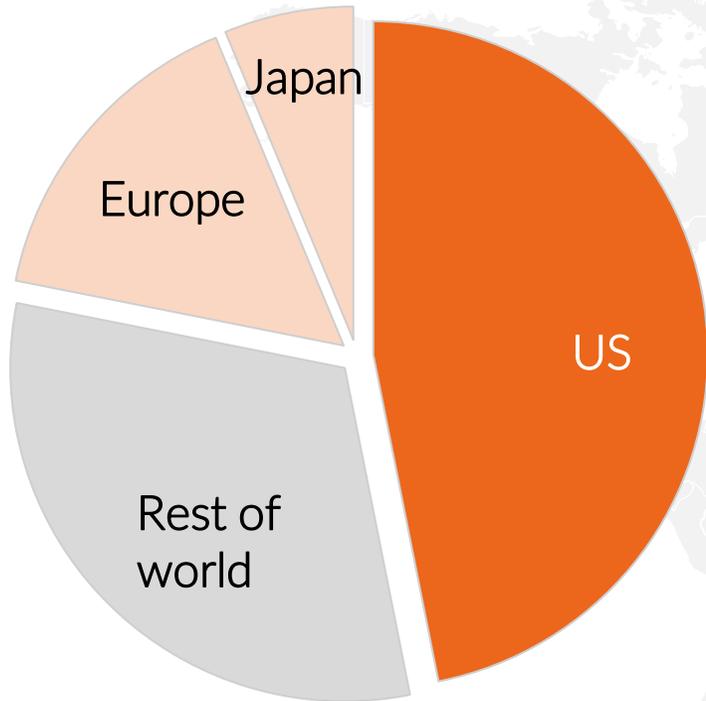


US FDA

Timely submission and approval by the US FDA as an orphan drug with an optimal label for use in the target population

- Full Clinical Study Report early Q4 2024
- Conclusions from FDA pre-submission meeting by Q1 2025
- NDA submission mid-2025

ATTRACTIVE ADDRESSABLE MARKET



Global addressable market of USD 800 million, half of this in the US

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

Global commercialization through partners

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 based on epidemiology and real-world data¹

Around 400 healthcare provider accounts serve 75% of kidney impaired patients⁴

Pricing range benchmarks based on innovative diagnostics, payer and expert input and price testing^{2, 3}

~100,000
procedures annually

~400 accounts

\$3,000-4,500

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
- 4) Ascelia Pharma analysis based on market research with Decision Resources Group, 2020

UNMET NEED RECOGNIZED IN CLINICAL PRACTICE

NSF* risk
with warnings for target population

“Those of us who have seen NSF are frightened by it... you’ll get **buy-in** from a lot of nephrologists...”
- Head of Renal section at US university hospital
(from Ascelia Pharma Advisory Board meeting)

+90%



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

+16%



of providers have experienced GBCA-induced NSF

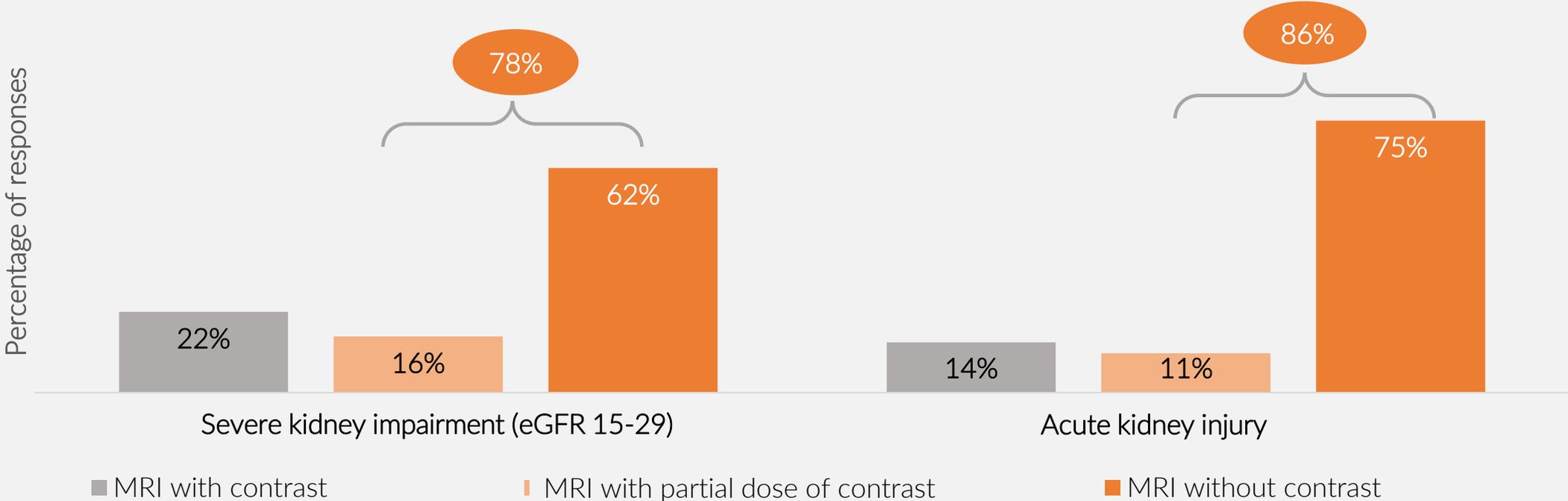
*nephrogenic systemic fibrosis

““The college [American Colleague of Radiology]...have a **growing sense of responsibility and accountability** about using these agents in high-risk patients.... our perception of which agents are “safe” has changed... this is another place where practice needed to evolve”
- SPARKLE Investigator and Head of Radiology at US university hospital

UNENHANCED MRI PREFERRED FOR TARGET PATIENTS TODAY

78% of physicians prefer MRI without or with partial dose contrast for patients with chronic kidney disease (CKD)

... 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

MOMENTUM FOR AN ALTERNATIVE TO GADOLINIUM

Deposition in brain & organs

concerns around safety for all patients

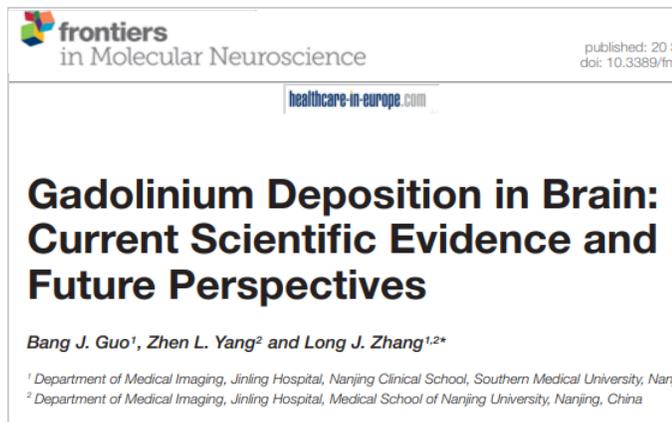
New safety category recommended for Symptoms Associated with Gadolinium exposure (SAGE), by Am. College of Rad. (2022)

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

Water contamination

scrutiny of environmental impact

Gadolinium excreted in urine is discharged into our environment and drinking water



Future with less/no gadolinium

focus of leading gadolinium manufacturers

Low dose full-body gadolinium contrast agents pursued by GBCA players with one approved by the FDA in priority review

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.

ON TRACK FOR OPTIMAL COMMERCIALIZATION

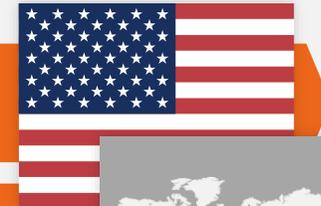
Strategic objectives for commercialization

- Optimal balance between investment required and future revenues
- Leverage established commercialization capabilities
- Maximize value with global launch strategy

Global commercialization through partners

Secure launch readiness

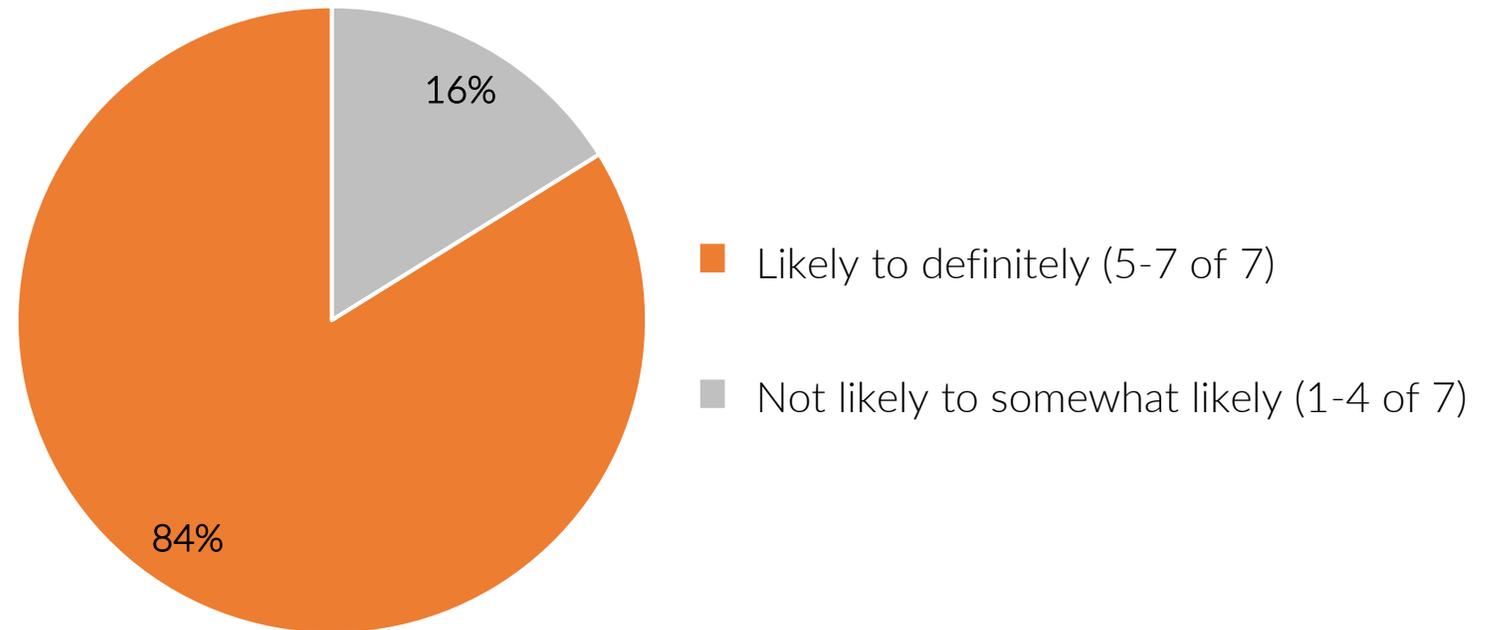
Establish commercial partnerships



Progress launch readiness securing VALUE CREATION

84% US HEALTHCARE PROFESSIONALS WILL USE ORVIGLANCE

LIKELIHOOD OF USING ORVIGLANCE FOR TARGET PATIENTS
(market research)



Reimagine imaging for people with poor kidney function.

Therapeutic Focus On

A New Approach to Imaging Focal Liver Lesions in Patients With Reduced Kidney Function

Current magnetic resonance imaging (MRI) methods used to identify liver cancer are inadequate in identifying a potentially fatal side effect in patients with poor kidney function – nephrogenic systemic fibrosis (NSF). Alternative imaging techniques are being developed to address this clinical need

Carl Björntor at Asceia Pharma

The early detection and localisation of focal liver lesions is critical for optimal management of patients with liver cancer or a range of cancers that metastasise to the liver, including colorectal, breast, and gastric cancer. The gold-standard method for detecting focal liver lesions is contrast-enhanced MRI. However, in patients with poor kidney function, all gadolinium-based contrast agents (GBCAs) have regulatory black box warnings, as they put those patients at risk of the severe- and sometimes fatal – side effect, NSF.

As patients with poor kidney function may not be able to tolerate these contrast agents, the imaging methods currently used – unenhanced MRI or non-liver specific lower-risk GBCAs – significantly reduce the ability of clinicians to find and treat focal liver lesions, ultimately impacting the patient's chance of survival. This patient population, which is estimated to account for around 4% of all patients requiring a liver MRI, is in dire need

of an alternative solution that provides similar imaging insights to those who undergo contrast drug-enhanced MRI.

The Risk of NSF

Although a rare condition, NSF is serious and potentially life-threatening. It causes sclerotic transformation and hardening of the skin, and can lead to joint contractures, and muscle and fascial fibrosis, which may lead to severe immobility. It can also affect the inner organs. NSF worsens over time and can cause death, which typically results from multi system failure. The FDA database has registered 3000+ cases of NSF since 2006 (of which 24% were fatal) and the severity of illness, time to disease manifestation, and GBCA dosing exposure vary individually (1, 2). It should be noted that not all global cases of NSF are reported to the FDA, however.

Group	Classification
I	Gadodiamide, gadopentetate dimeglumine
II	Gadobenate dimeglumine, gadobutrol, gadoterate acid, gadoteridol
III	Gadoversate

Table 1: American College of Radiology 2018 classification of gadolinium-based contrast agents into groups I, II, and III

Regulatory agencies, including the FDA and EMA, have issued warnings about the use of GBCAs, and clinical guidelines restrict use in patients with severe kidney impairment. The American College of Radiology guidelines for GBCA administration advise against administration of group I and group III agents (see Table 1) in those on dialysis or with chronic kidney disease stage four or five (3).



Downloaded from https://www.itn.org.uk

COVID-19 IMAGING INFORMATION TECHNOLOGY WOMEN'S HEALTH RADIATION ONCOLOGY

REVIEW ARTICLE

OPEN

Oral Manganese Chloride Tetrahydrate: A Novel Magnetic Resonance Liver Imaging Agent for Patients With Renal Impairment Efficacy, Safety, and Clinical Implication

Torkel B. Brismar, MD, PhD, Dominik Geisel, MD, Nikolaos Kartalis, MD, PhD, Beatrice L. Madrazo, MD, Hanna Persson Hedman, PhD, and Andreas Norlin, PhD

NEWS | RSNA | DECEMBER 03, 2022

Study compares effect of food intake on manganese-based MRI contrast agent absorption

A study presented at RSNA 2022 evaluated the effect of food intake on the absorption and signal intensity of Orviglance, a manganese-based MRI contrast agent, and successfully concluded that image enhancement is not impacted by a light meal.



orviglance®
800 mg powder for oral solution
manganese chloride tetrahydrate

ORVIGLANCE®

Liver diagnostic imaging drug

ONCORAL

Daily, oral chemotherapy



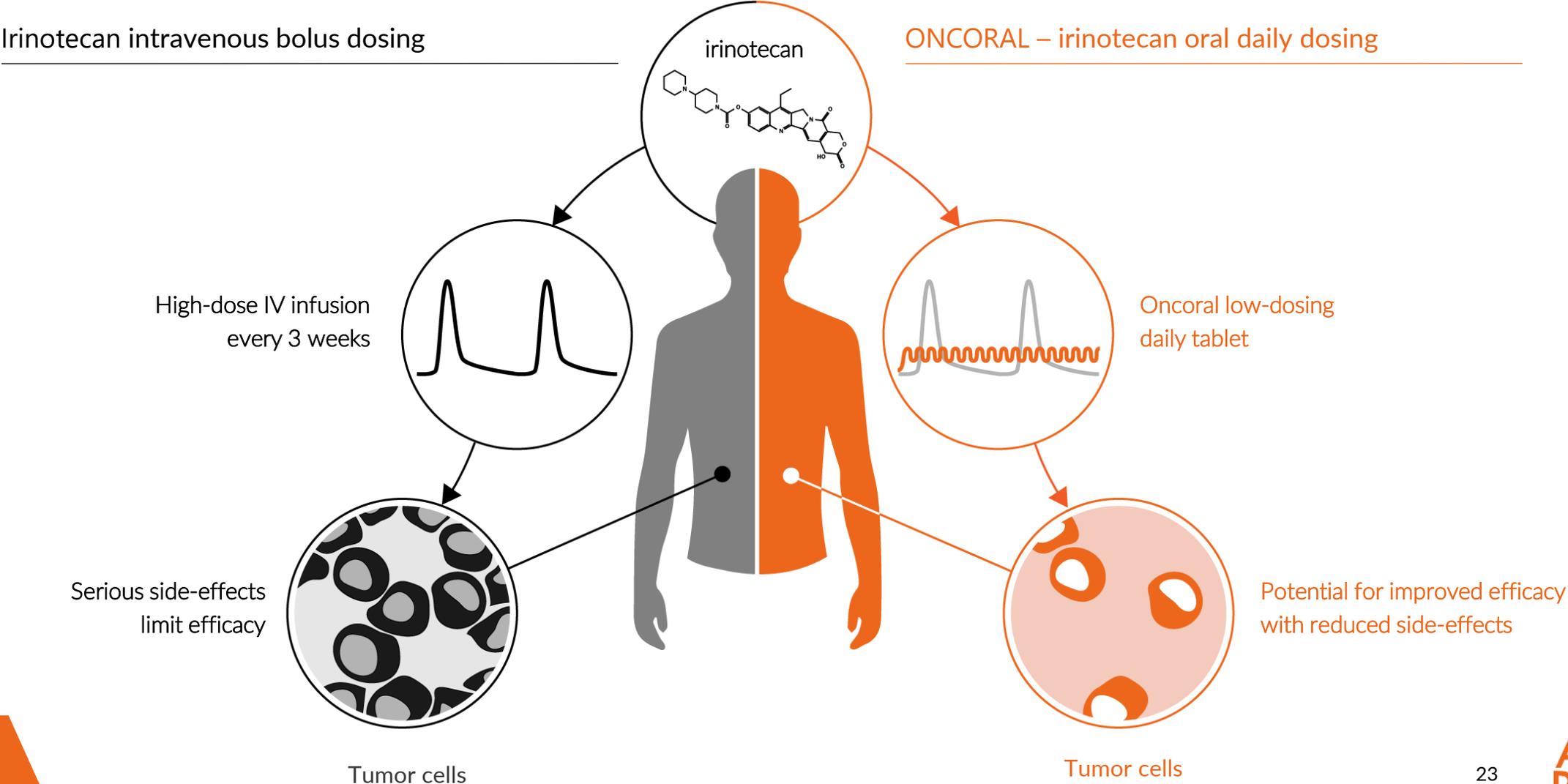
PORTFOLIO

ASCELIA
PHARMA

IMPROVING IRINOTECAN EFFICACY and TOLERABILITY

Irinotecan intravenous bolus dosing

ONCORAL – irinotecan oral daily dosing

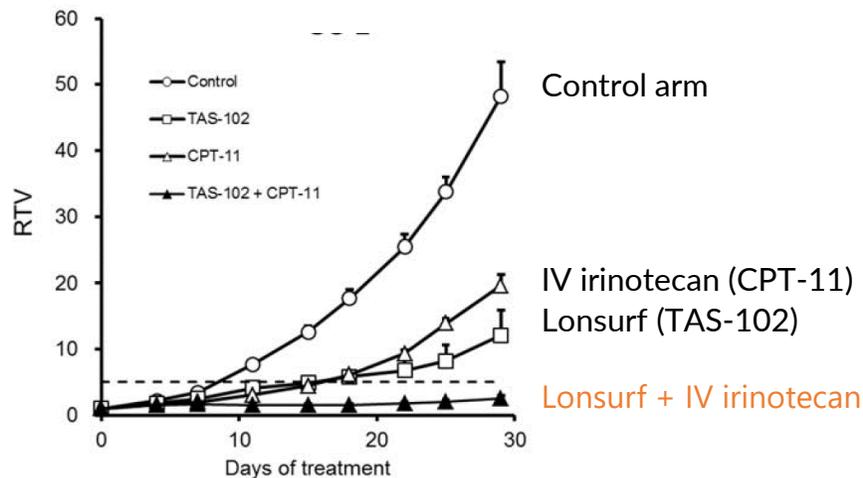


ONCORAL PHASE 2 IN GASTRIC CANCER

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 cancer¹
(Relative Tumor Volume, RTV)



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 Orvigance, patient enrollment is not initiated until it can be done effectively

Clinical collaboration with

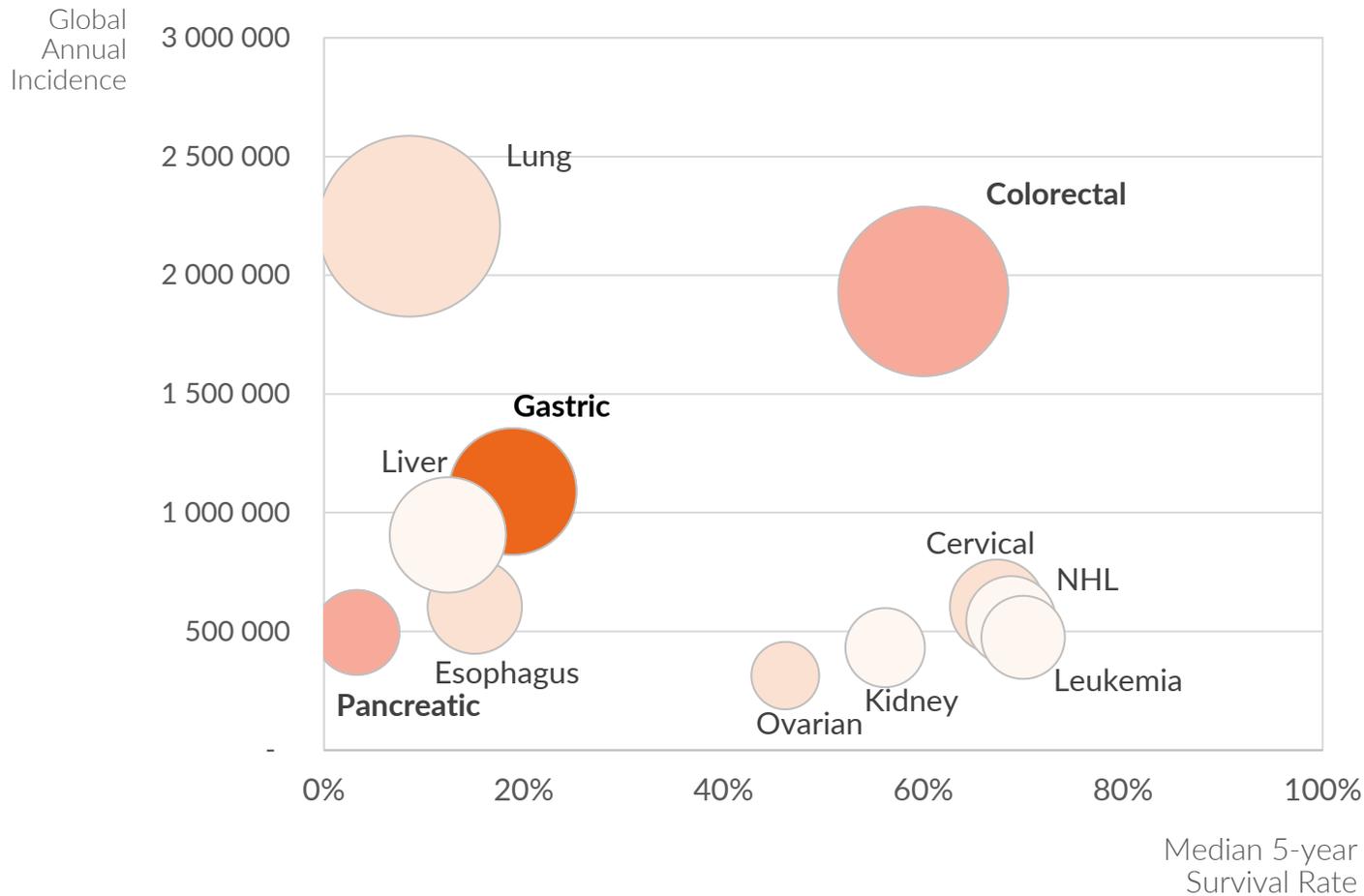


LONSURF is approved for treatment of metastatic gastric cancer and metastatic colorectal cancer

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

HIGH VALUE OPPORTUNITY IN GASTRIC CANCER AND EXPANSION

POTENTIAL FOR ORAL, DAILY DOSING OF IRINOTECAN³



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

- **Current focus: Gastric cancer**
 - Clinically demonstrated
 - Guidelines recognized
 - 3rd highest cancer deaths¹
 - Orphan disease (US and EU)
 - \$3-4bn market²
- **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



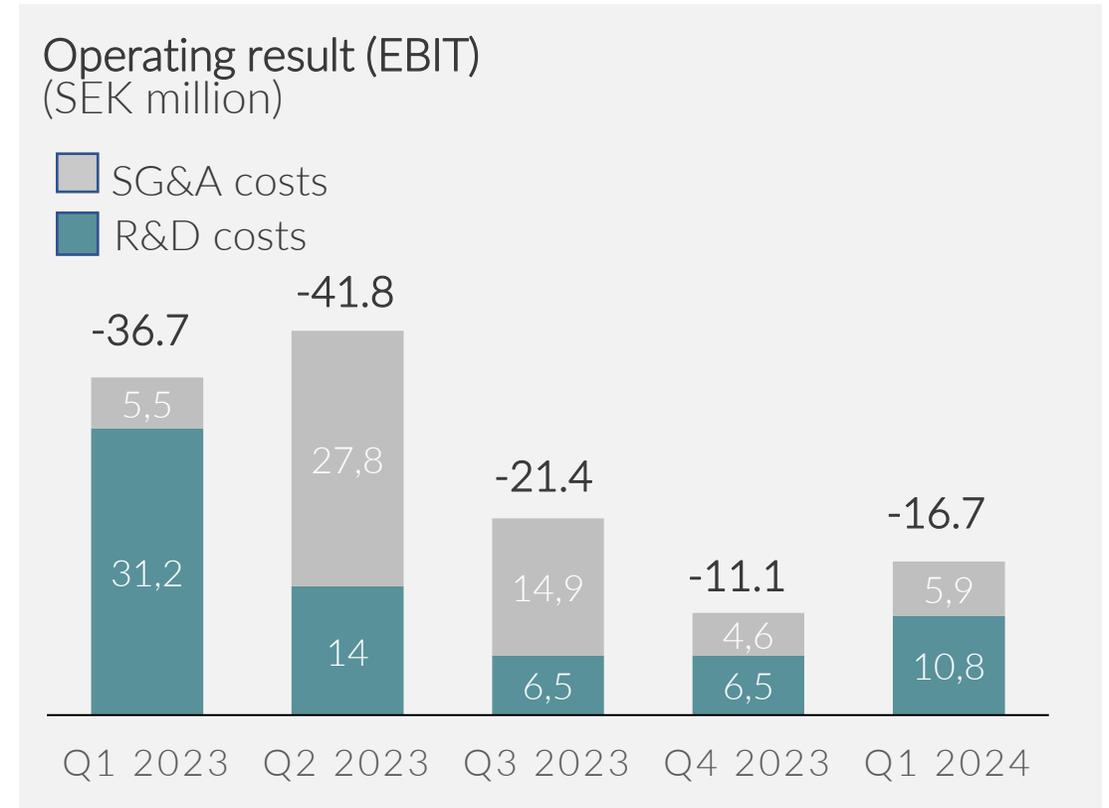
FINANCIALS & OUTLOOK

OPERATING RESULT- MAINTAINED LOW OPERATING EXPENSES

Operating loss of SEK 16.7 million in Q1 2024

Slightly increased loss (costs) compared to Q4 2023 driven by:

- Completion of SPARKLE re-evaluation
- NDA preparation activities



Notes:

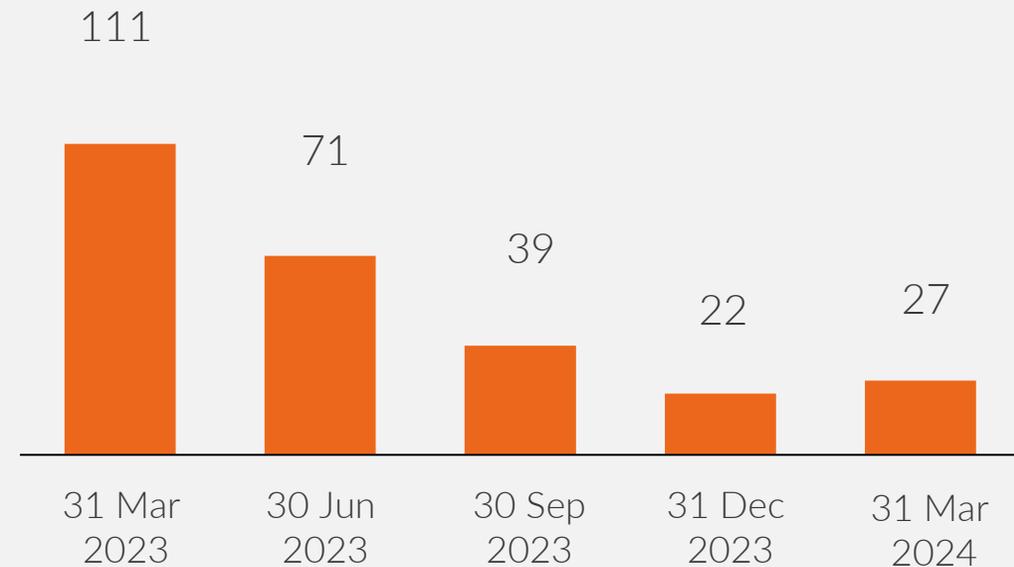
1) Other operating income and other operating costs added to SG&A

LIQUIDITY - CASH RUNWAY INTO Q2 2025

Liquid assets of 27 MSEK (31 Mar 2024)

- On 2 February, a financing of up to 35 MSEK (loan and convertibles) was secured with Formue Nord with 20 MSEK drawn in February
- On April 18, the remaining 15 MSEK was drawn under the loan agreement with Formue Nord
- **Runway into Q2 2025** covering completion of time critical activities for the NDA

Liquid assets including marketable securities
(SEK million)



SUBSTANTIAL VALUE CREATION OPPORTUNITIES

	 Advance Orviglance to approval	 Progress Orviglance commercialization readiness	 Develop pipeline potential
Objectives	<p>Timely submission and approval by the US FDA as an orphan drug with an optimal label for the use in the target population</p>	<p>Focused launch for well-defined patient population</p> <p>Global commercialization through partners</p>	<p>Demonstrate Oncoral efficacy and safety in Phase 2</p> <p>Expand Orviglance franchise with 2nd generation</p>
Milestones	<ul style="list-style-type: none"> ▪ Full Clinical Study Report early Q4 2024 ▪ Conclusions from FDA pre-submission meeting by Q1 2025 ▪ NDA submission mid-2025 	<ul style="list-style-type: none"> ▪ Advance launch readiness ▪ Commercialization partnership 	<ul style="list-style-type: none"> ▪ Initiate Oncoral Phase 2 clinical study when financing allows

Mid-2025 refers to the three months from mid-May to mid-August

ASCELIA PHARMA

ascelia.com



[Follow us on LinkedIn](#)