

# ASCELIA PHARMA

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**WEBCAST:**  
18 August 2022, 10:00AM CET

Link webcast:  
[Ascelia Pharma Q2 Report 2022  
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## PRESENTATION OF Q2-2022 REPORT

*Present from Ascelia Pharma:*

CEO Magnus Corfitzen | CFO Kristian Borbos  
CMO Carl Bjartmar | CCO Julie Waras Brogren

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# ASCELIA PHARMA – COMPANY HIGHLIGHTS



**TO IMPROVE THE LIFE  
OF PEOPLE LIVING WITH CANCER  
BY OFFERING BETTER  
TREATMENT OPTIONS**

## ADVANCING ORPHAN ONCOLOGY

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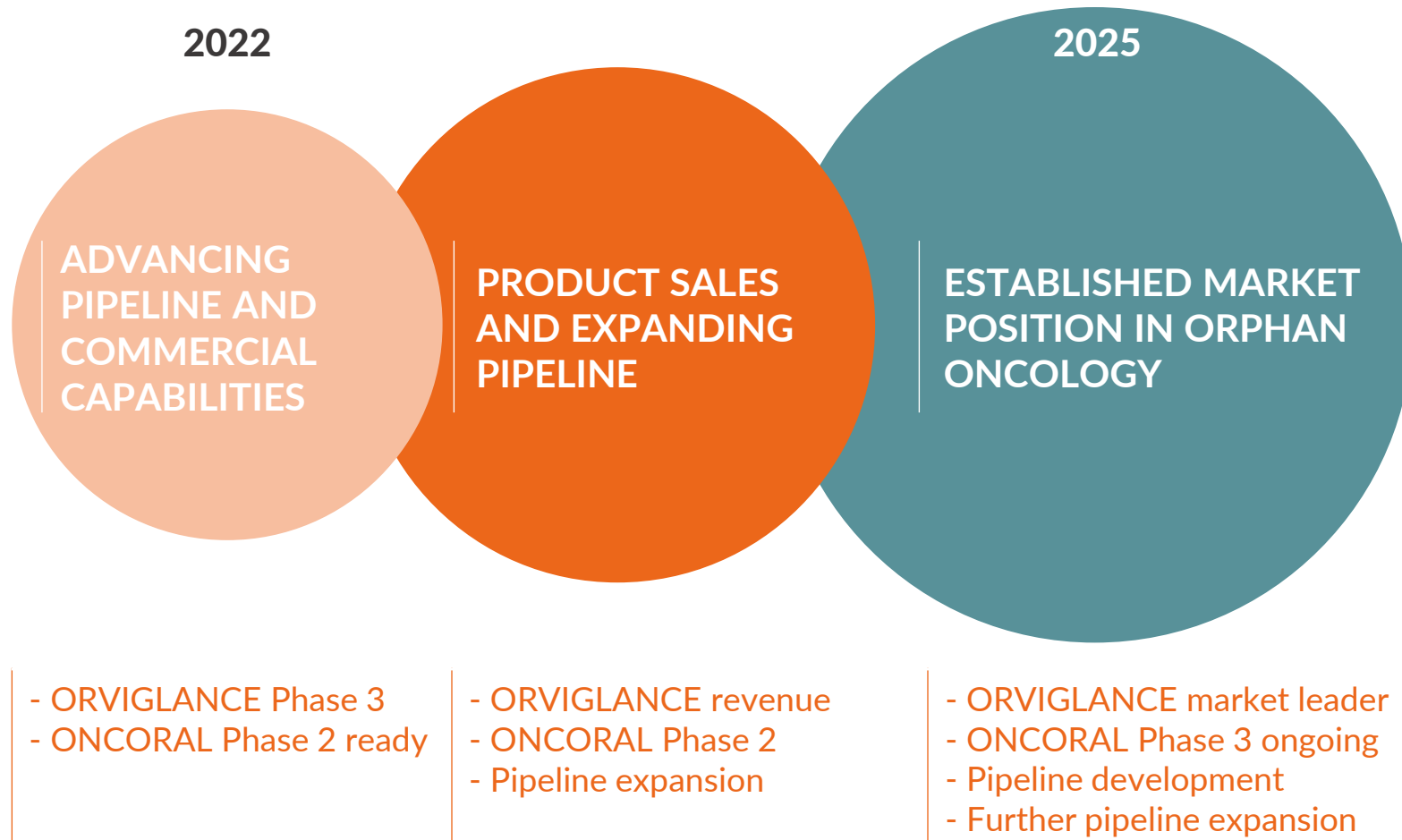
- Identify, develop and commercialize novel drugs that address unmet needs in rare cancers
- Two drugs in advanced clinical development
  - **ORVIGLANCE** – in global Phase 3; FDA Orphan Drug Designation
  - **ONCORAL** – ready for Phase 2

## BUILDING GLOBAL CAPABILITIES

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- Based in Malmö (Sweden), US affiliate in New Jersey (US)
- Solid balance sheet and financed into H2 2023
- Listed on NASDAQ Stockholm (Ticker: ACE)

# BUILDING VALUE AND GROWTH TRAJECTORY





# RECENT KEY EVENTS

## Key events in Q2-2022

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- May** Results from Food Effect Study show strong liver imaging enhancement with Orvigance both with light meal and fasting condition
- May** Notice of allowance for second US patent for Oncoral
- Jun** Orvigance comparison study to gadolinium presented at ESGAR conference
- Jun** Déspina Georgiadou Hedin appointed as new CFO replacing Kristian Borbos

## Key events after Q2-2022

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- Aug** Food Effect Study accepted as an oral presentation at the world's largest radiology conference, RSNA







# PORTFOLIO

## ORVIGLANCE

Liver diagnostic drug in ongoing Phase 3

## ONCORAL

Daily oral chemotherapy ready for Phase 2

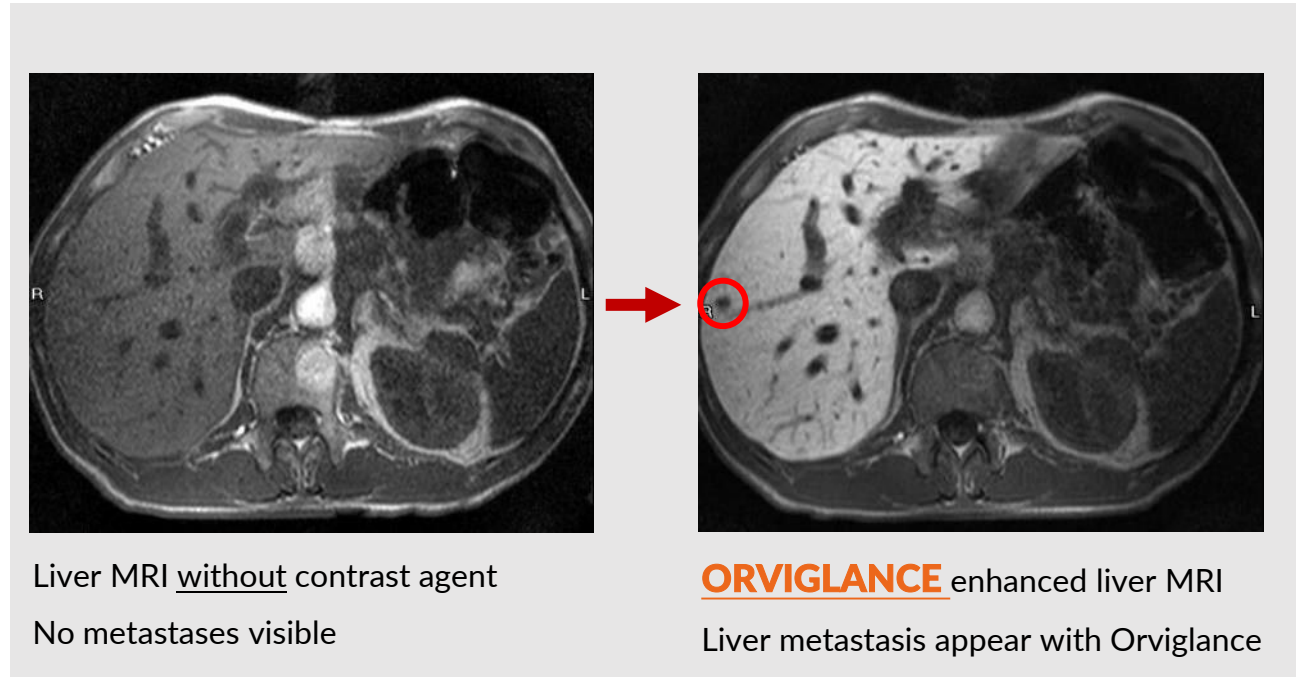
# ORVIGLANCE – PHASE 3 LIVER MRI CONTRAST AGENT

## NOVEL LIVER MRI CONTRAST AGENT

- Diagnostic drug for use in liver MRI scan to detect cancer
- Liver metastases common in many cancer types and often the cause of mortality
- Targeting patients at risk of potentially fatal side-effect from the current contrast agents on the market

## SOLID PROGRESS

- Strong clinical Phase 2 results (p-values <0.0001)
- Ongoing Global Phase 3 study
- Strong results to pivotal program from supportive studies
- Orphan Drug Designation from FDA



# ORVIGLANCE PHASE 1 & 2 RESULTS (6 STUDIES)

## Consistent strong efficacy readout and safety profile



### Blind read study of all images vs. unenhanced MRI (178 persons)

- Significantly improved MRI
- 33% more lesions
- Lesion visualization
  - Delineation (border sharpness): **p-value <0.0001**
  - Conspicuity (contrast vs. background): **p-value <0.0001**

Proceed to Phase 3



# ORVIGLANCE ONGOING PHASE 3 STUDY – SPARKLE

<p>Patients</p> 	<ul style="list-style-type: none"><li>• Global study, 200 patients</li><li>• Known or suspected focal liver lesions and severe renal impairment</li></ul>	<ul style="list-style-type: none"><li>• Around 50 sites in the US, Europe, Latin America</li><li>• Working with active and new sites to accelerate enrollment</li></ul>
<p>Comparator</p> 	<p>Unenhanced MRI + ORVIGLANCE MRI vs. Unenhanced MRI</p>	<p>No randomization – each patient as own control</p>
<p>Endpoint</p> 	<p>Lesion visualization</p> <ul style="list-style-type: none"><li>• Lesion border delineation</li><li>• Conspicuity</li></ul>	<ul style="list-style-type: none"><li>• Same endpoints as in Phase 2</li><li>• Same endpoints as for approved gadolinium agents</li></ul>
<p>Follow-up</p> 	<p>Less than a week</p>	<p>Expected pivotal study patient enrollment: 2022</p>

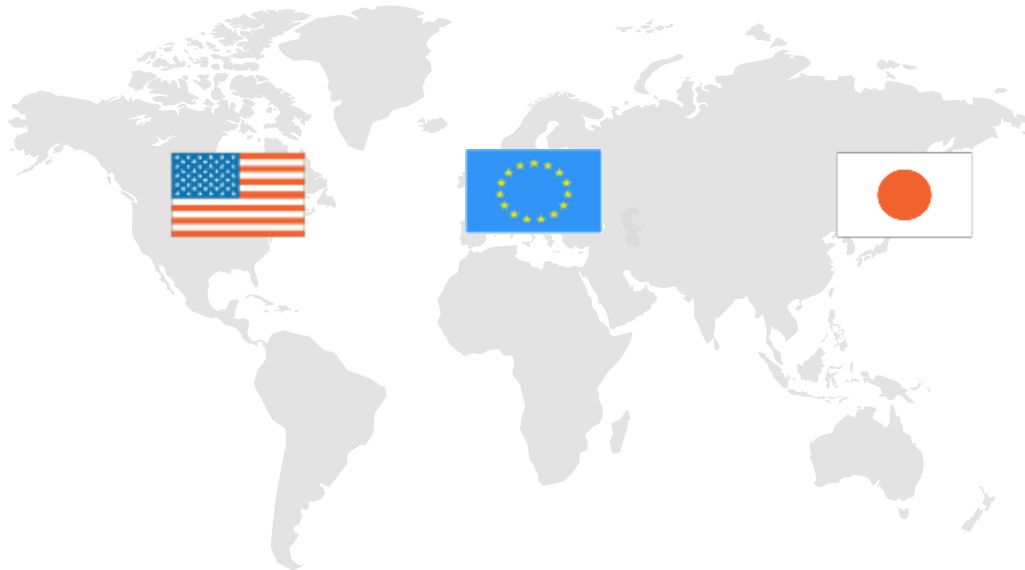
# ORVIGLANCE PIVOTAL PROGRAM – SUPPORTING STUDIES

	Study design	Status and Results
Food Effect Study	<ul style="list-style-type: none"><li>• Crossover study in healthy volunteers</li><li>• Evaluate the impact of food intake on absorption and signal intensity of Orviglance (light meal or full meal vs. fasting condition)</li></ul>	<ul style="list-style-type: none"><li>• Intake of light meal prior to Orviglance MRI provides similar liver image enhancement as Orviglance MRI on fasting condition</li><li>• Robust image enhancement of the liver after Orviglance compared to an MRI without a contrast agent</li></ul>
Hepatic Impairment Study	<ul style="list-style-type: none"><li>• Sequential cohort study in patients with different degrees of hepatic impairment</li><li>• Evaluate the effect of liver impairment on the safety, pharmacokinetics and pharmacodynamics of Orviglance</li></ul>	<ul style="list-style-type: none"><li>• Last Patient Last Visit completed</li><li>• Final results expected in Q3 2022</li></ul>

# ORVIGLANCE MARKET \$500-600 MILLION ANNUALLY

## \$500-600M ADDRESSABLE MARKET IN US, EU AND JAPAN

- Ascelia Pharma to commercialize in the US
- RoW commercialization with partners



### DRIVERS

- Patients with suspected primary liver cancer or liver metastases and severe kidney impairment (~4%)
- Actual imaging procedures (real-world data)<sup>1</sup>
- Payer and expert input (+75 stakeholders)<sup>2</sup>

### UPSIDES

- Other markets, e.g., China
- Annual growth of 4-5%

Sources:

1) Ascelia Pharma market research with Decision Resources Group, 2020

2) Ascelia Pharma market research and analyses with Revenue Reimbursement Solutions and Charles River Associates, 2020

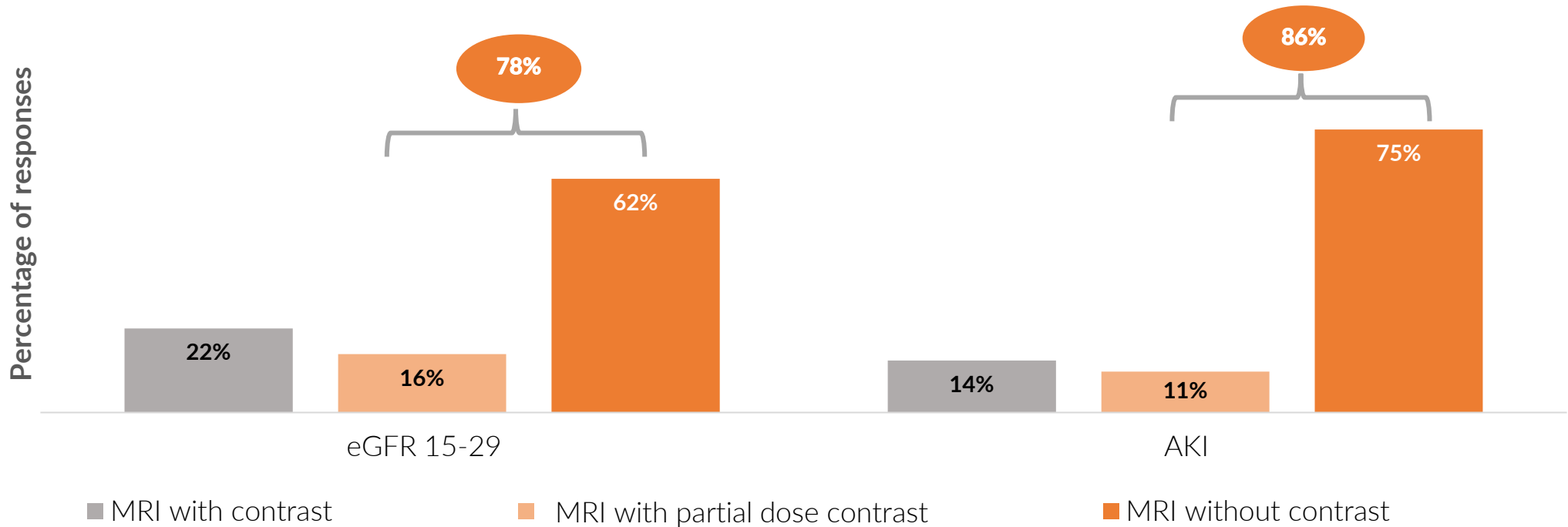


# MARKET RESEARCH MARCH 2022

- FOR ORVIGLANCE TARGET PATIENTS, US HEALTHCARE PROFESSIONALS CURRENTLY PREFER UNENHANCED MRI

78% PREFER MRI WITHOUT OR WITH PARTIAL DOSE CONTRAST FOR PATIENTS WITH LOW eGFR

... EVEN MORE FOR AKI PATIENTS



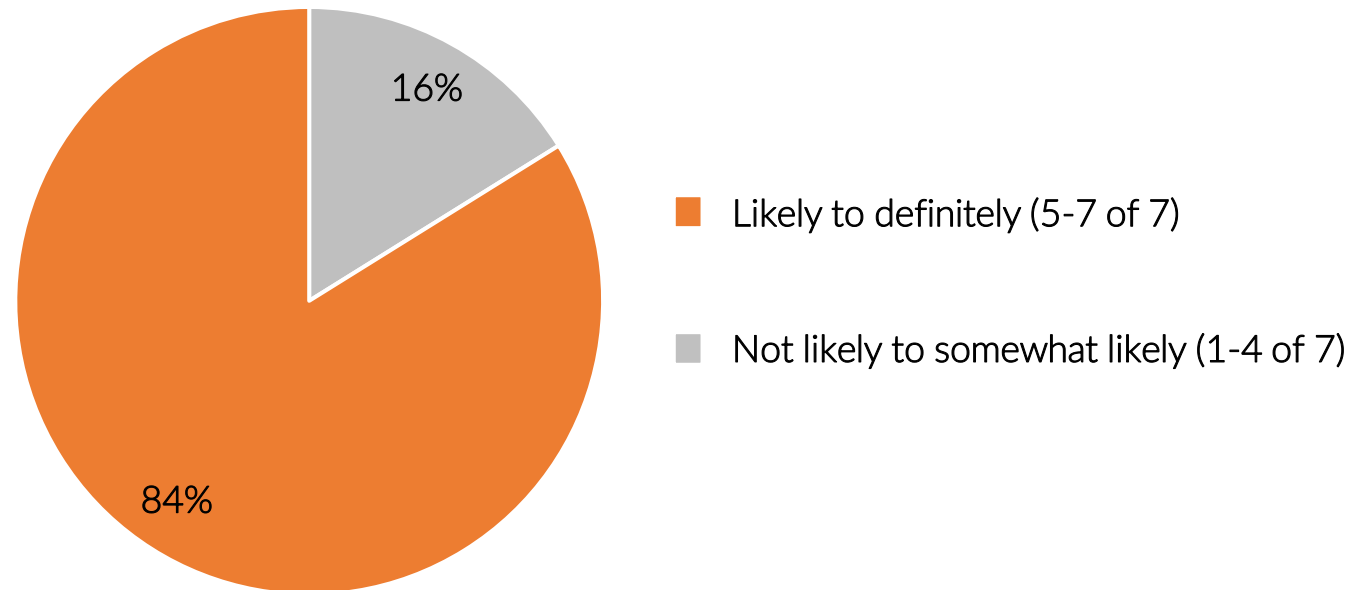
N=103 oncologist, nephrologist, and radiologist responses.

Q: Please assign priority to the imaging tests in the sequence or order in which you would recommend or perform them (shown as % split of first priority of MRI options)

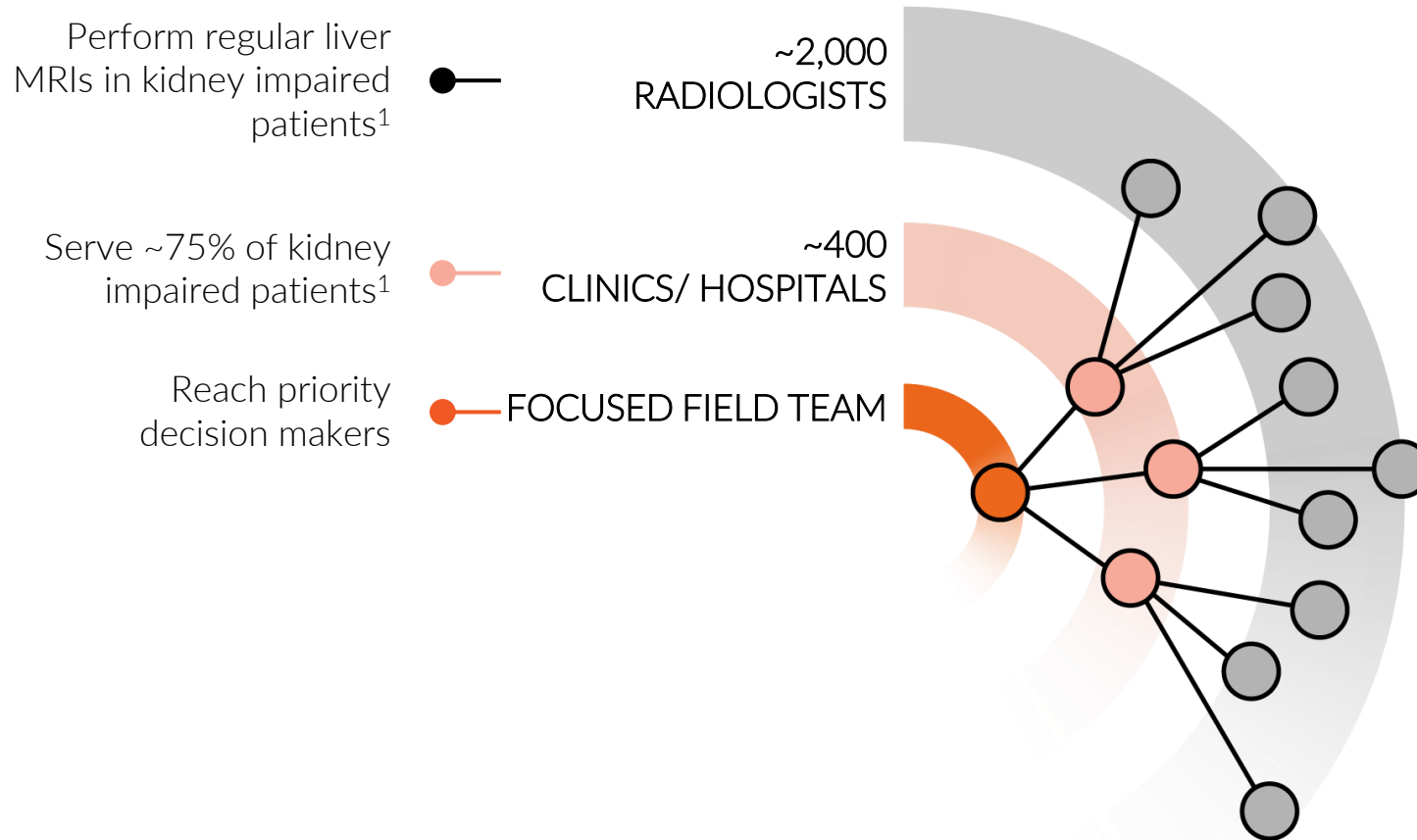
# MARKET RESEARCH FROM MARCH 2022

– 84% US HEALTHCARE PROFESSIONALS SAY THEY WILL USE ORVIGLANCE

## LIKELIHOOD OF USING ORVIGLANCE FOR TARGET PATIENTS



# CAPTURING US MARKET VALUE WITH ASCELIA'S TEAM



## BUILDING ASCELIA U.S. TEAM

New Jersey office (up to 40 FTEs at launch)

Cambrex manufacturing partner in New Jersey

## BUILDING OUT U.S. FOOTPRINT

SPARKLE Phase 3 study at leading US Sites including Stanford, Mass. General, Duke University, UCLA Medical Center

Sources:

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





## PORTFOLIO

### ORVIGLANCE

Liver contrast agent in ongoing Phase 3

### ONCORAL

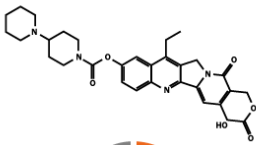
Daily oral chemotherapy ready for Phase 2

# IMPROVING IRINOTECAN EFFICACY and TOLERABILITY

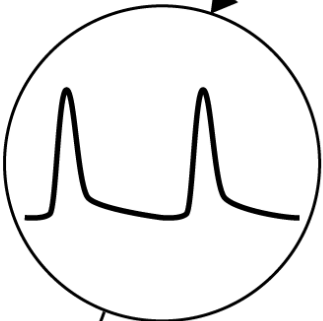
**Irinotecan** intravenous bolus dosing

**ONCORAL** – irinotecan oral daily dosing

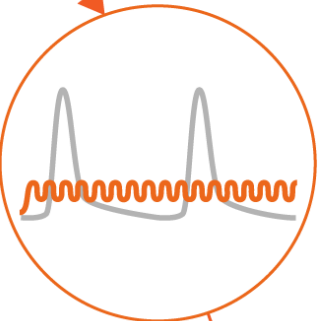
irinotecan



High-dose IV infusion every 3 weeks



Oncoral low-dosing daily tablet



Serious side-effects limit efficacy



Tumor cells

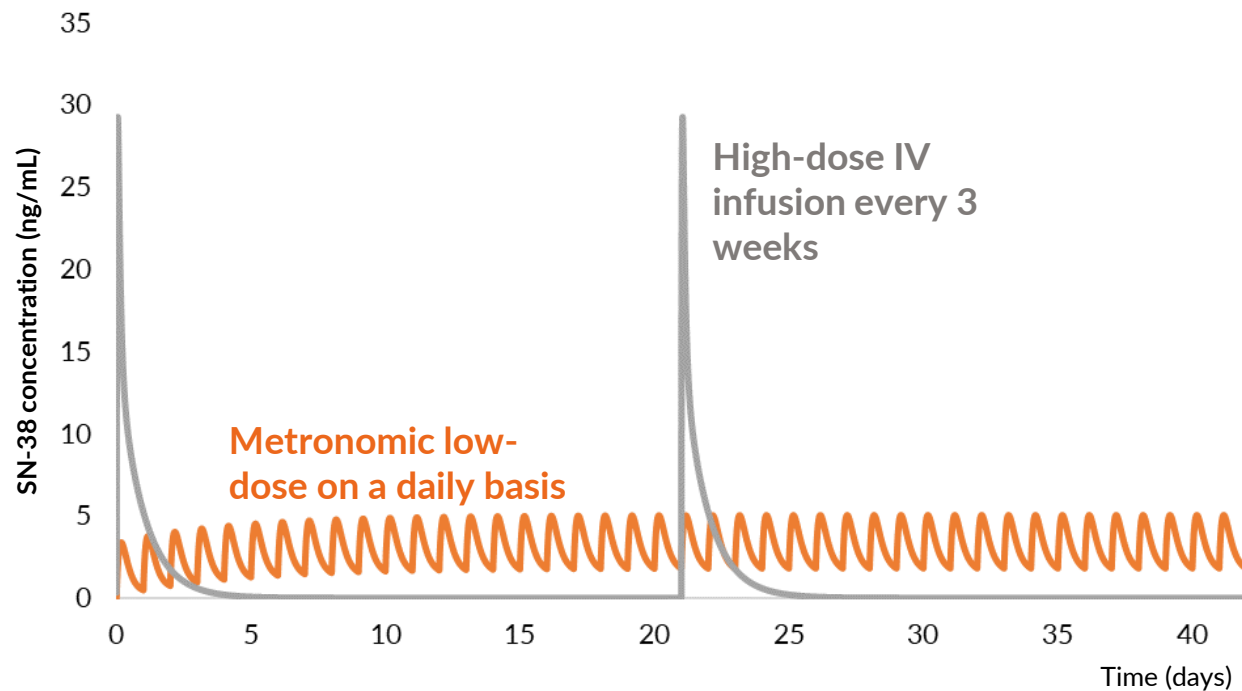
Potential for improved efficacy with reduced side-effects



Tumor cells

# ONCORAL PHASE 1: ENCOURAGING SAFETY PROFILE

## PLASMA LEVELS OF IRINOTECAN



Source: Simulation of Oncoral vs. IV Camptosar

## Oncoral Phase 1 results

- Well tolerated, no unexpected side-effects
- Hematological toxicities mild-moderate (grade 1 or 2)<sup>4</sup>
- Efficacy: Stable disease even in patients previously treated with IV irinotecan

### Infrequent high-dose IV irinotecan

Gastrointestinal and hematological side effects, ~30% severe or life-threatening (grade 3 or 4)<sup>1</sup>

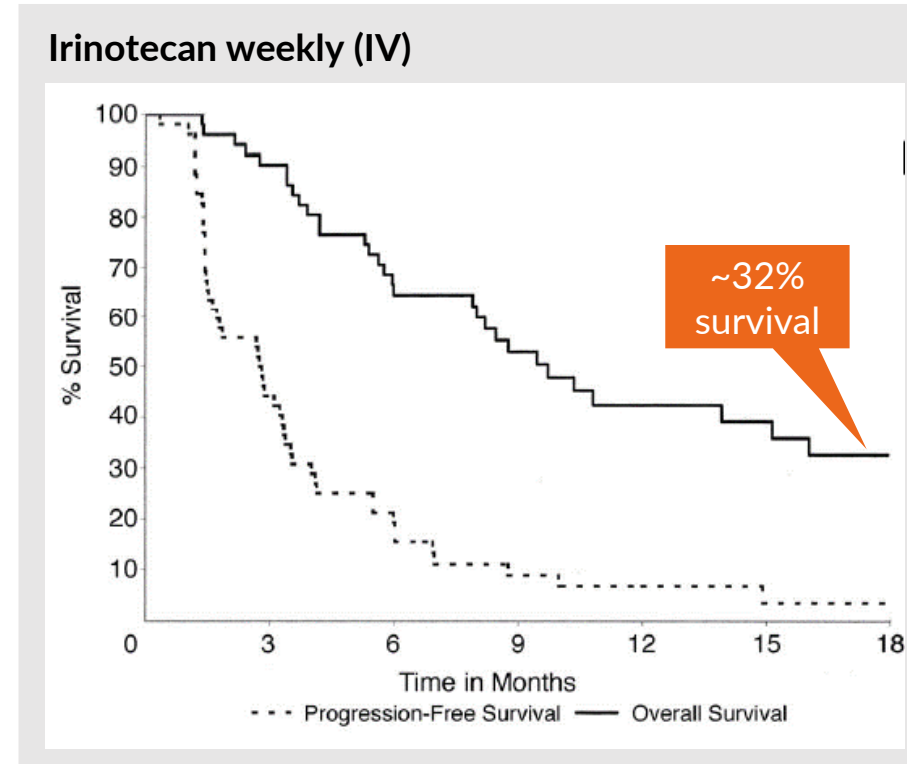
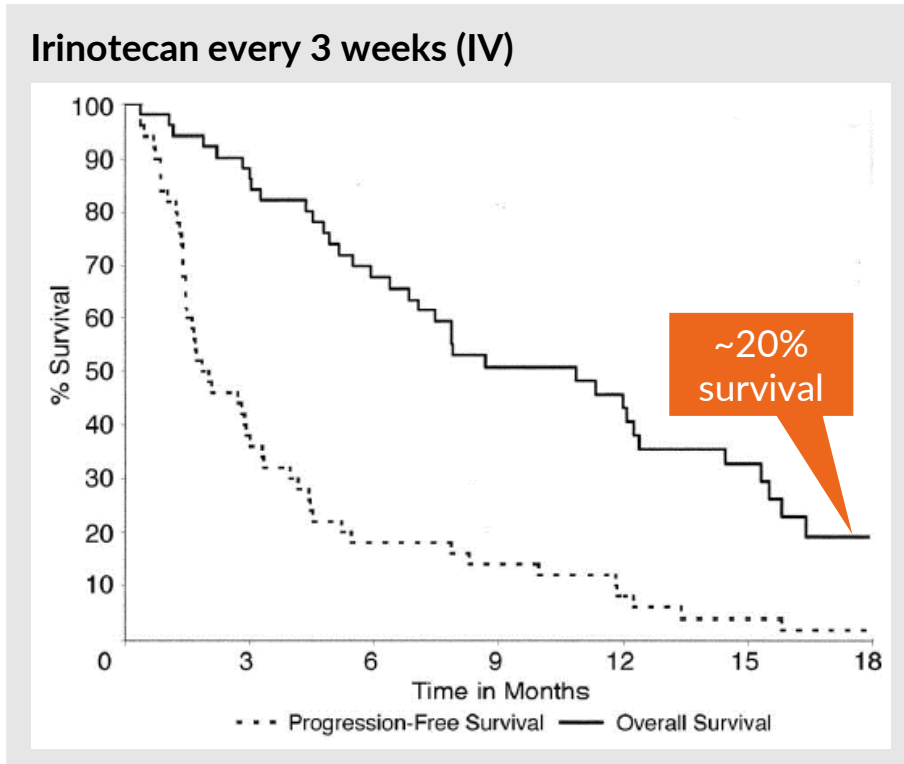
### Frequent (metronomic) low-dose irinotecan

- Several studies show improved tolerability<sup>2,3</sup>
- Daily dosing – adjust quickly if acute toxicity



# IMPROVING IRINOTECAN **EFFICACY** BY FREQUENT LOW DOSING

Overall survival: Improved from 20% (dosing every third week) to 32% (weekly dosing)<sup>1</sup>



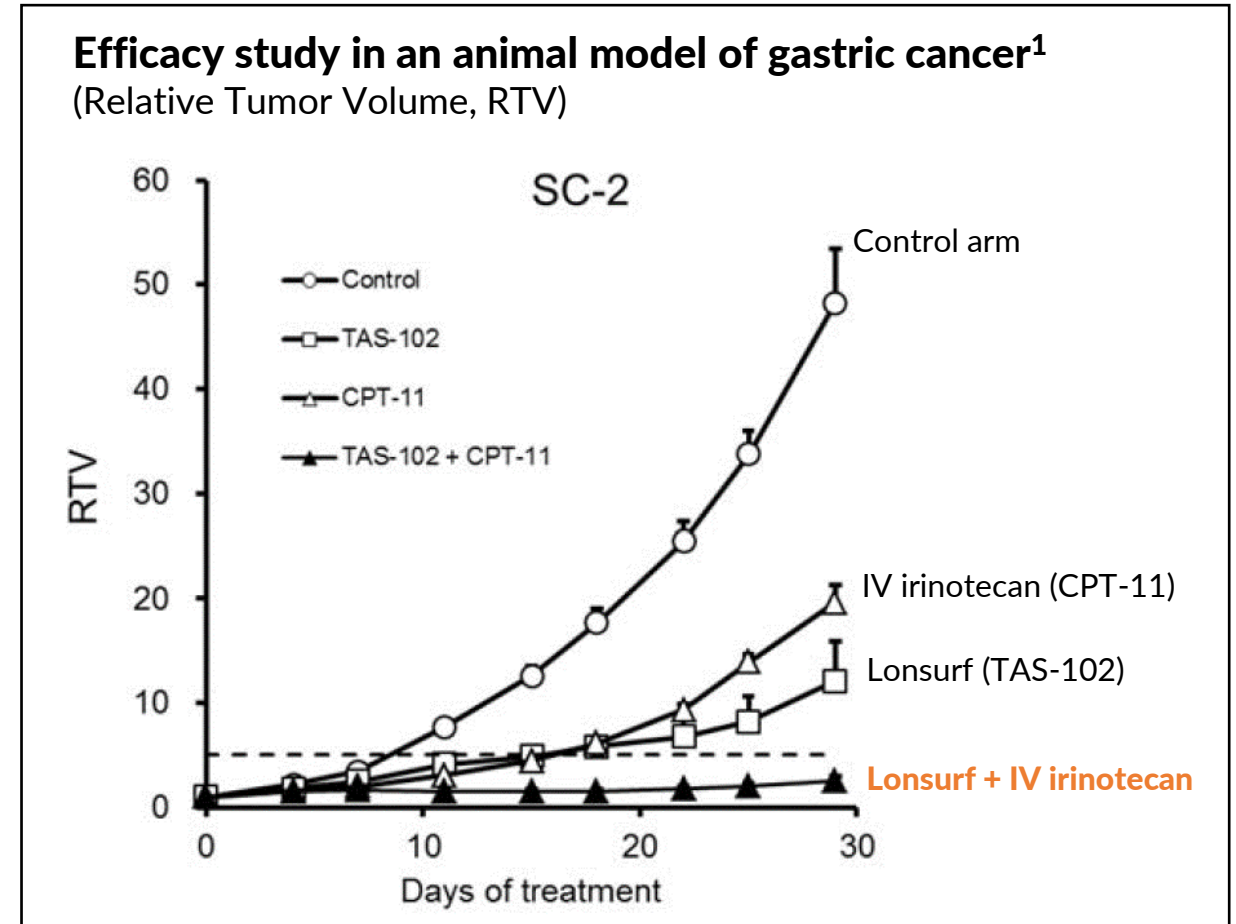
Study in patients with metastatic refractory breast cancer, N=103

1) Perez et al. J Clin Oncol 2004: Randomized Phase II Study of Two Irinotecan Schedules for Patients With Metastatic Breast Cancer Refractory to an Anthracycline, a Taxane, or Both

# ONCORAL PHASE 2 IN GASTRIC CANCER




## STRONG RATIONALE FOR GASTRIC CANCER

- Clinical guidelines support efficacy of irinotecan
- Potential for Orphan Drug Designation
- Potential for synergistic effect between Lonsurf and irinotecan



# PHASE 2 STUDY DESIGN

## STUDY DESIGN (ALL-ORAL COMBINATION STUDY)

<b>Patients</b> 	<ul style="list-style-type: none"><li>• Around 100 patients</li><li>• Metastatic gastric cancer</li><li>• Randomized controlled, multicenter/multinational</li></ul>
<b>Comparator</b> 	Oncoral + Lonsurf vs. Lonsurf
<b>Endpoints</b> 	<b>Primary:</b> Progression Free Survival <b>Secondary:</b> Response rate, PK, Safety and Overall Survival data in a follow up analysis

### Clinical collaboration with



**TAIHO ONCOLOGY**

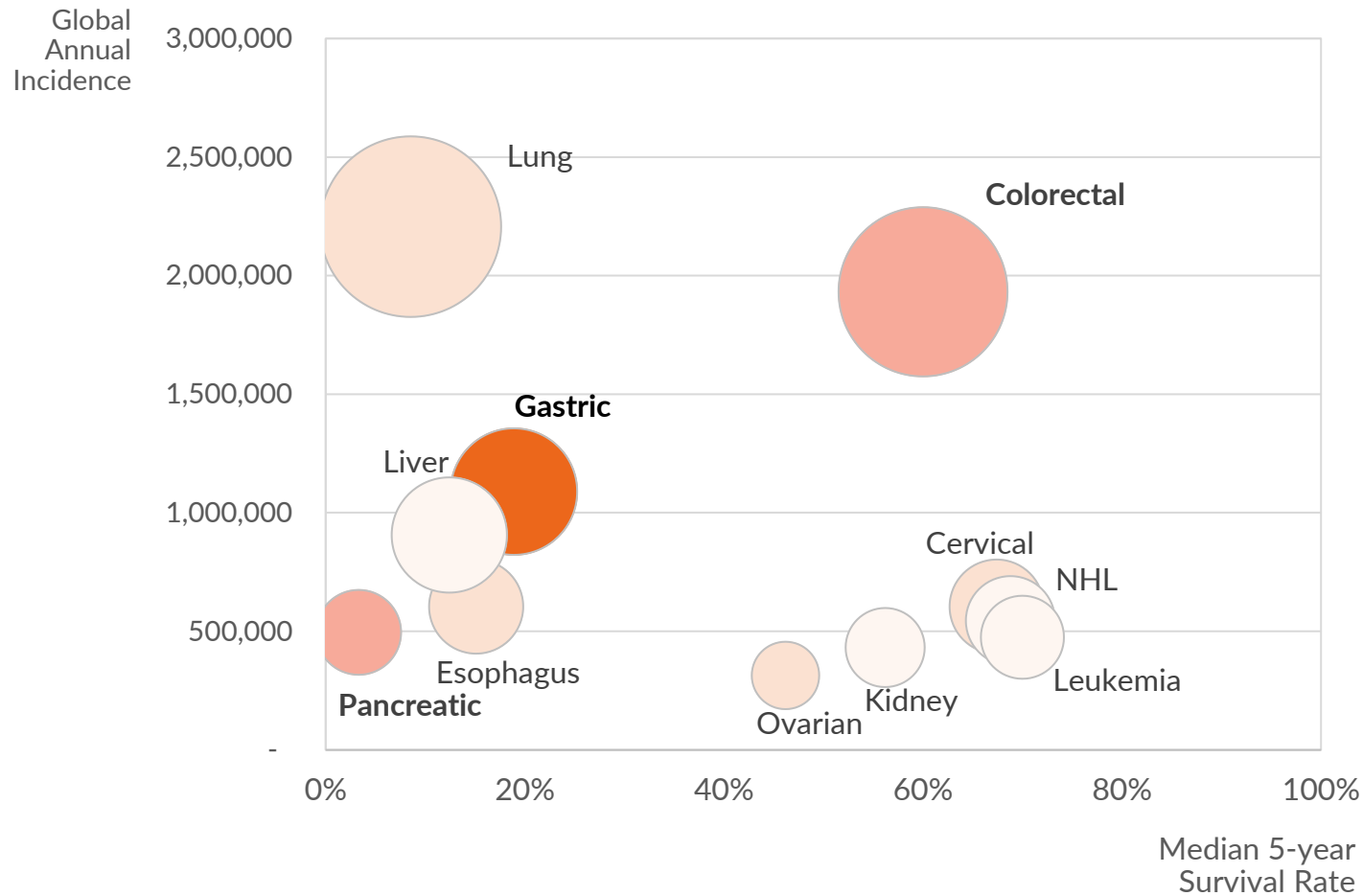
LONSURF is approved for treatment of metastatic gastric cancer and metastatic colorectal cancer

## PHASE 2 READY – AWAITING START TO FOCUS ON ORVIGLANCE

- Continued very strong belief in Oncoral as a novel oral chemotherapy
- Study start approval (IND) gained in the US in December 2021
- Study start approval gained in the UK and Spain in H1 2022
- In May 2022, US Patent and Trademark Office issued a notice of allowance for a second Oncoral patent application for the method of use of Oncoral
- To focus all resources on Orviglance, patient enrollment is not initiated until it can be done effectively

# HIGH VALUE OPPORTUNITY IN GASTRIC CANCER AND EXPANSION

## POTENTIAL FOR ORAL, DAILY DOSING OF IRINOTECAN<sup>3</sup>



- **Current focus: Gastric cancer**
  - 3<sup>rd</sup> highest cancer deaths<sup>1</sup>
  - Orphan opportunity (U.S. and EU)
  - \$3-4bn market<sup>2</sup>
- Approved indications for IV irinotecan infusions
- Indications for which IV irinotecan infusions are clinically demonstrated & NCCN recognized
- Indications for which IV irinotecan infusions 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 AND PRIORITIES

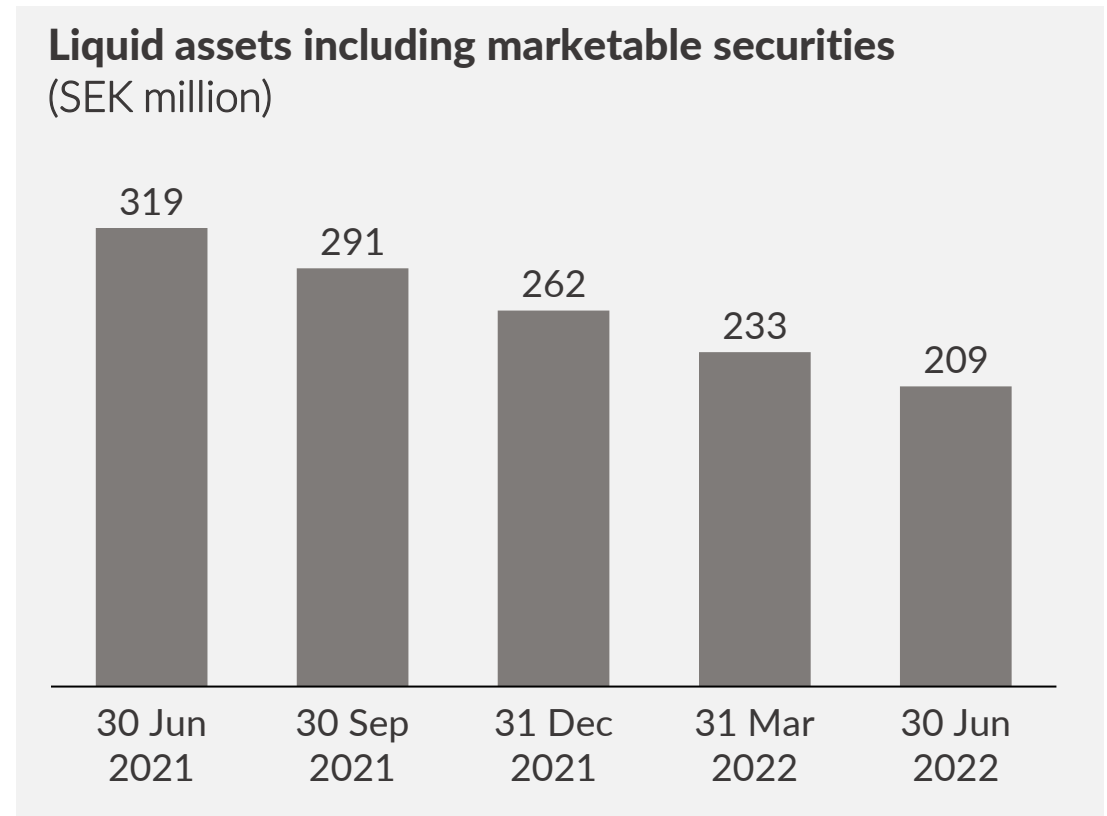




# FINANCIAL HIGHLIGHTS Q2 2022 – LIQUIDITY POSITION

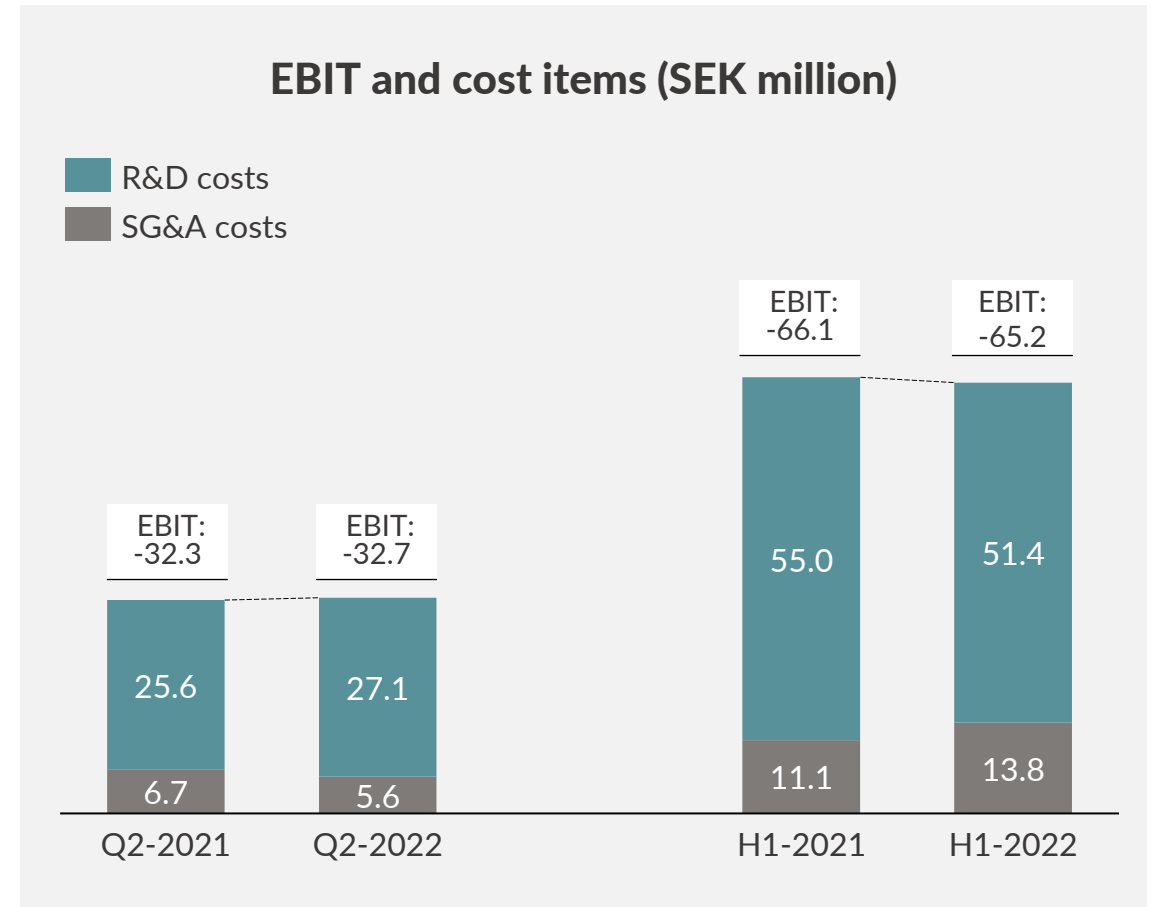
## Solid liquidity position:

- Liquid assets of 209 MSEK (\$20 million) by 30 Jun 2022
- Current cash position provides financing into H2 2023



# FINANCIAL HIGHLIGHTS Q2 2022 – OPERATING RESULTS

- Operating loss in Q2 2022 was largely unchanged compared to Q2 2021
- Same y/y development in operating loss for H1-2022 vs. H1-2021



Notes:

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



## PRIORITIES 2022

 Complete Orvigance Phase 3 patient enrollment

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 Prepare Orvigance launch

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# ASCELIA PHARMA

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