ADVANCING
ORPHAN
ONCOLOGY

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Nasdaq Stockholm
www.ascelia.com

# ASCELIA PHARMA

## INVESTOR PRESENTATION

*February, 2023*

ASCELIA
PHARMA

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# ASCELIA PHARMA – COMPANY HIGHLIGHTS


## ADVANCING ORPHAN ONCOLOGY

- 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

- Based in Malmö (Sweden), US affiliate in New Jersey (US)
- Solid balance sheet and financed into Q4 2023
- Listed on NASDAQ Stockholm (Ticker: ACE)

# PORTFOLIO

## ORVIGLANCE

**Liver diagnostic drug in ongoing Phase 3**

## ONCORAL

Daily oral chemotherapy ready for Phase 2



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# ORVIGLANCE – FILLING AN UNMET NEED IN LIVER MRI

## Liver metastases critical in cancer care

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

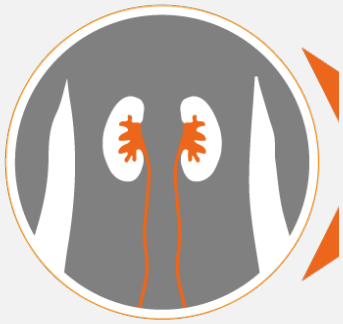
- Colorectal cancer, metastatic breast cancer, gastric cancer

## Contrast enhanced MRI is gold standard

Contrast enhanced MRI

- Detection and visualization
- Surgery or drug treatment planning
- Post-treatment surveillance

## A role for Orviglance in patients with kidney impairment



**Healthy kidneys**
MRI with gadolinium contrast agent

**Severe kidney impairment**

- All gadolinium contrast agents have regulatory Black Box warnings
- Risk of severe side effects, incl. Nephrogenic Systemic Fibrosis

**Orviglance**
aims to be the liver imaging option that doesn't risk safety for cancer patients with poor kidney function


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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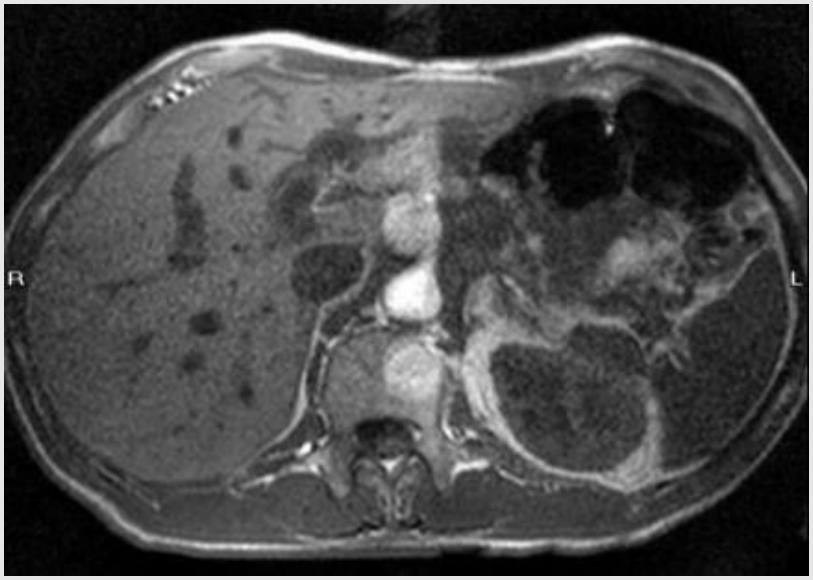
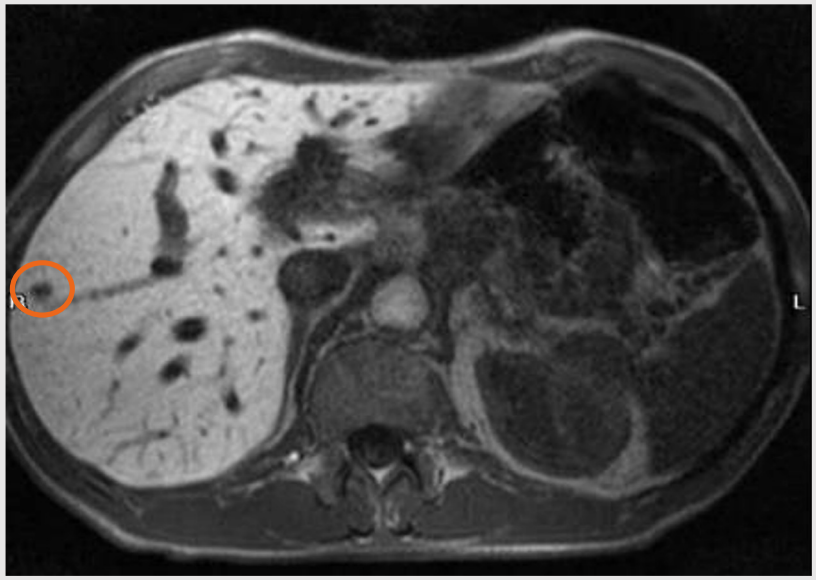
# STRONG LIVER ENHANCEMENT WITH ORVIGLANCE

**PATIENT EXAMPLE FROM PHASE 2 STUDY**




**UNENHANCED** liver MRI (without contrast agent)




**ORVIGLANCE** contrast enhanced liver MRI

Liver metastasis appear with Orviglance

Source: Patient with colorectal cancer. (Study CMC-P002)



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# EIGHT COMPLETED CLINICAL STUDIES

## - Data presented at major radiology conferences

| | | | |
|---|---|---|---|
| Phase 1 & 2 | Completed (6 studies) | **BLINDED READ STUDY**<br>Safety and efficacy vs. unenhanced in all phase 1 and 2 images (6 studies, including 178 persons and compassionate use) | **Consistent positive results, incl. 33% more lesions**<br>**Delineation** (border sharpness) and **conspicuity** (contrast vs. background): p-value <0.0001 |
| | | **ORVIGLANCE VS. GADOLINIUM CONTRAST AGENT**<br>Orviglance vs. gadolinium (Multihance) and vs. unenhanced (20 persons crossover with 3 independent readers) | **Number of lesions** (3 of 3 higher)<br>**Smaller lesion** detection (3 of 3 higher)<br>**Delineation and conspicuity** (2 of 3 higher) |
| Phase 3 Program | Completed (1 study) | **FOOD EFFECT STUDY**<br>Evaluates the effect of food intake on absorption and signal intensity (23 healthy volunteers) | **Strong liver enhancement both in fasting condition and with light meal**, support intake of light meal |
| | Completed (1 study) | **HEPATIC IMPAIRMENT STUDY**<br>Evaluates the effect of liver impairment on the safety, pharmacokinetics and pharmacodynamics | **Well tolerated in patients with liver impairment**<br>Confirms excretion primarily via the liver and not the kidney |
| | Ongoing (1 study) | **SPARKLE PHASE 3 PIVOTAL STUDY**<br>Evaluates the safety and efficacy in target patient population (enrollment not yet completed | **71 of 80** patients completed 27 Jan, 2023<br>Completion expected **Feb-Mar 2023** |



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# New strong Orviglance data support successful SPARKLE completion with substantially fewer patients

Press Release 06 Dec 2022

## Assumptions for original SPARKLE sample size estimate were conservative

New data with the same image reading methodology as in SPARKLE demonstrate

- Successful re-read study ($p<0.009$) based on 20 patients for lesion visualization (primary endpoint in SPARKLE)
- Two to three times higher effect than originally assumed

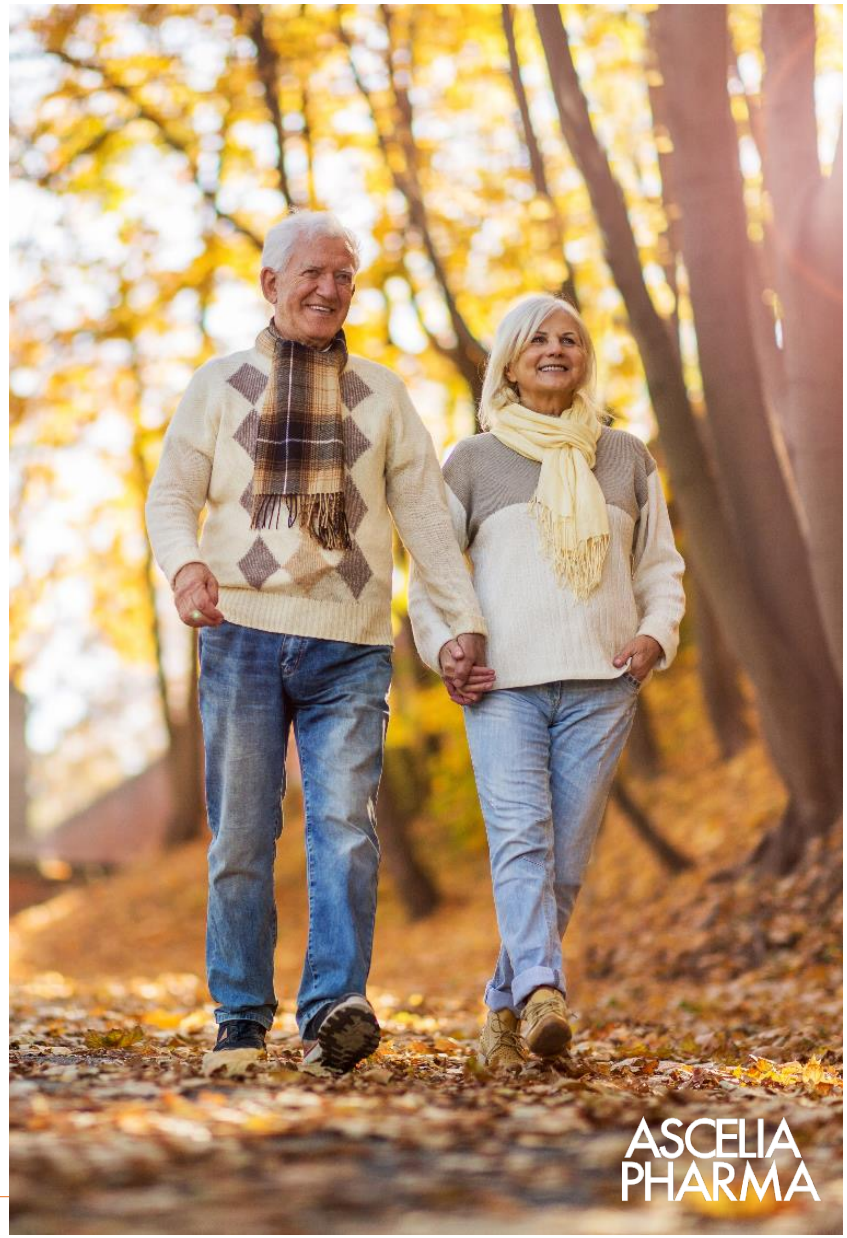
## Statistically significant results can be obtained with substantially fewer patients and strong likelihood of success, while maintaining conservative assumptions

We have thoroughly analyzed the new data and original assumptions with statisticians and regulatory experts to validate this important finding

Based on discussions with the FDA, Ascelia Pharma has decided to change the patient enrollment target of SPARKLE to 80 patients

As of 27 January, 71 patients were enrolled in SPARKLE.

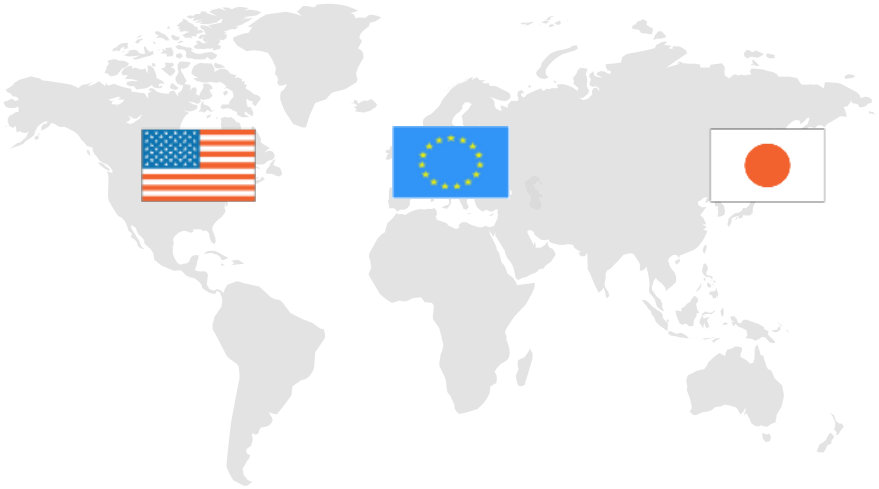
## Our confidence in SPARKLE outcomes and commercial potential remains strong as we prepare for the next steps for Orviglance and Ascelia Pharma



# 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)[1]
- Payer and expert input (+75 stakeholders)[2]

### 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



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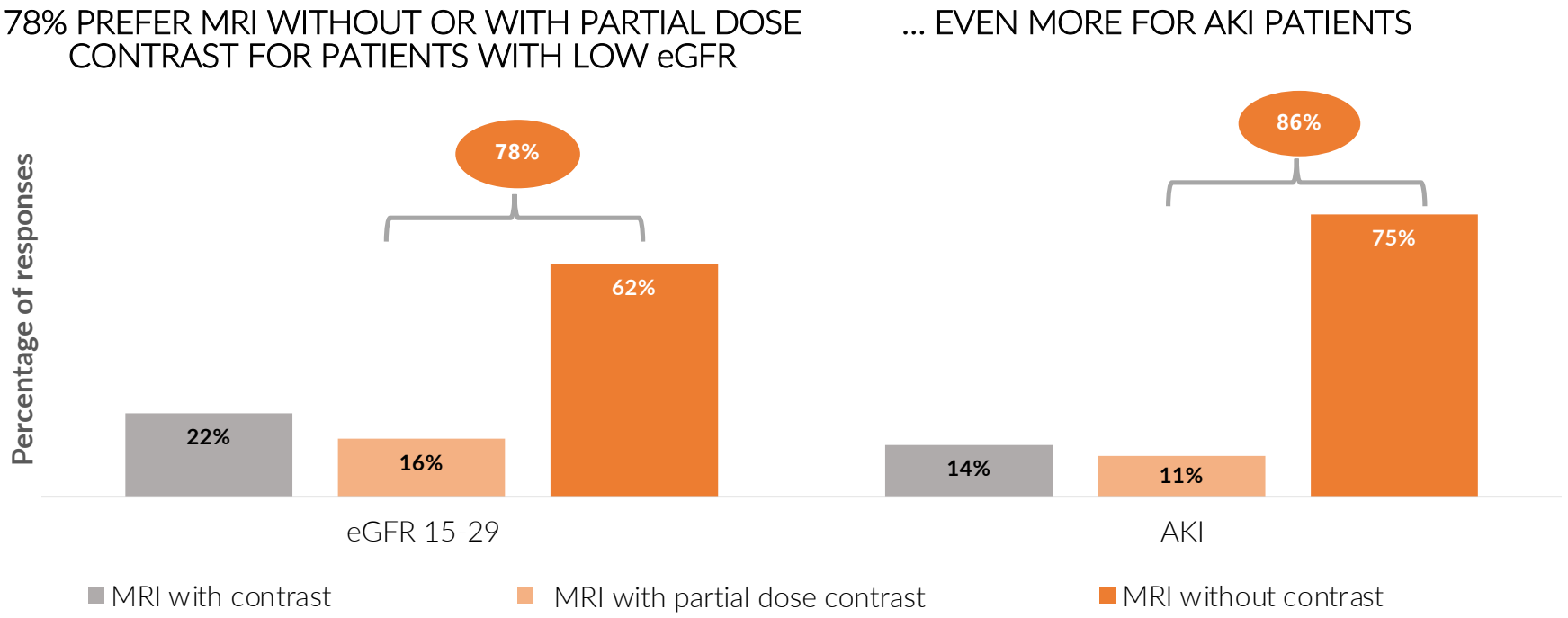
# US PHYSICIANS PREFER UNENHANCED MRI FOR TARGT PATIENTS




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)



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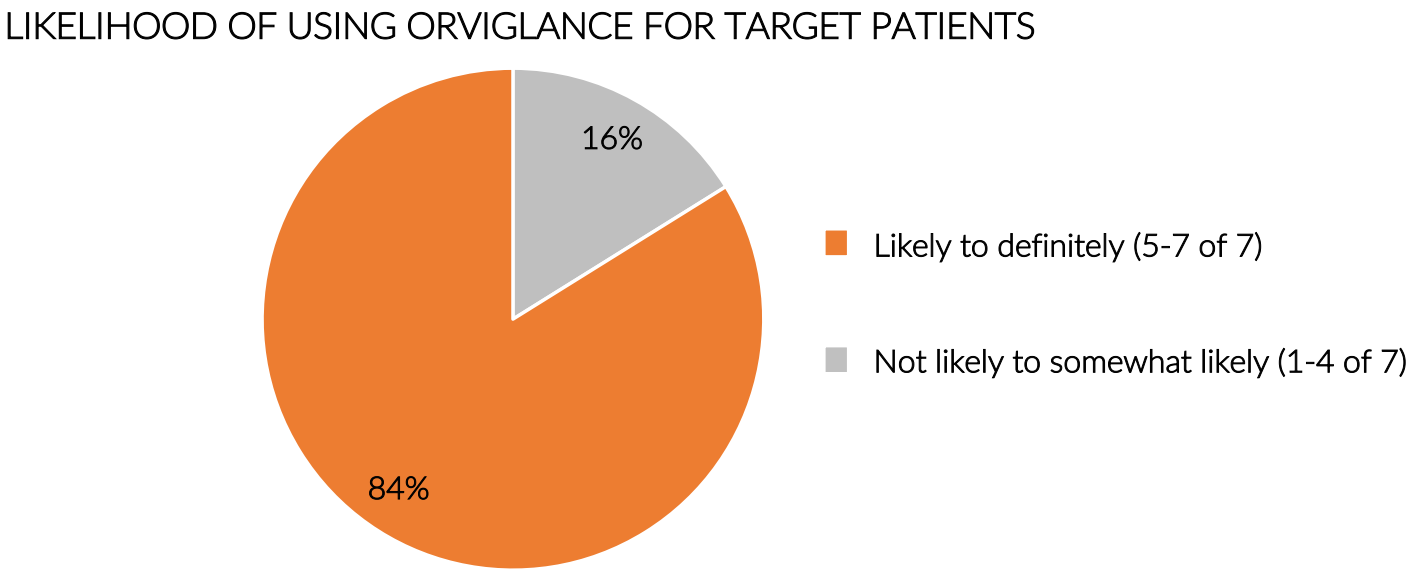
# US PHYSICIANS SAY THEY WILL USE ORVIGLANCE




Market research for Ascelia Pharma conducted in Q4 2021/Q1 2022 by Two Labs Pharma Services
N =254 oncologist, nephrologist, and radiologist responses
Q: On a scale of 1 (not at all likely) to 7 (definitely), how likely are you to use or suggest using Orviglance for your patients?



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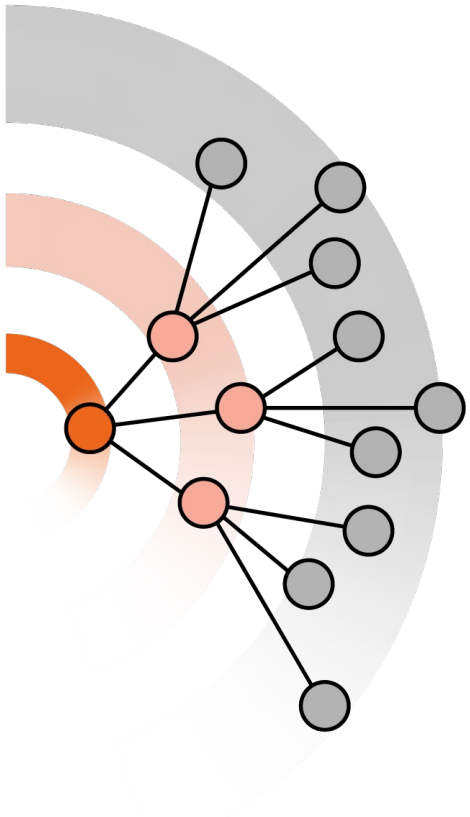
# CAPTURING US MARKET VALUE WITH ASCELIA'S TEAM

Perform regular liver MRIs in kidney impaired patients[1]

Serve ~75% of kidney impaired patients[1]

Reach priority decision makers




## 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 Mayo Clinic, Mass. General, Stanford

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**



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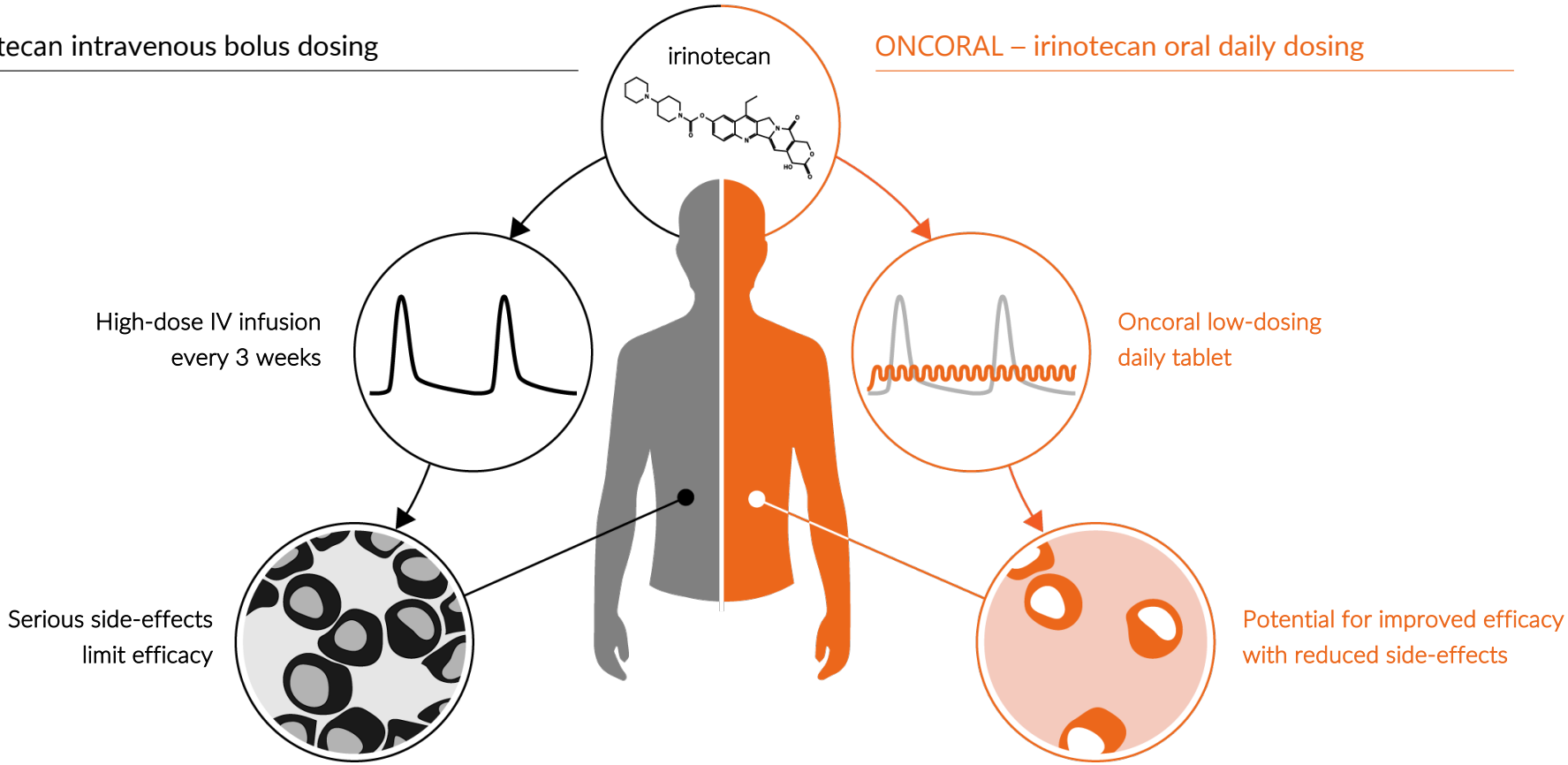
# IMPROVING IRINOTECAN **EFFICACY** and **TOLERABILITY**

Irinotecan intravenous bolus dosing

ONCORAL – irinotecan oral daily dosing



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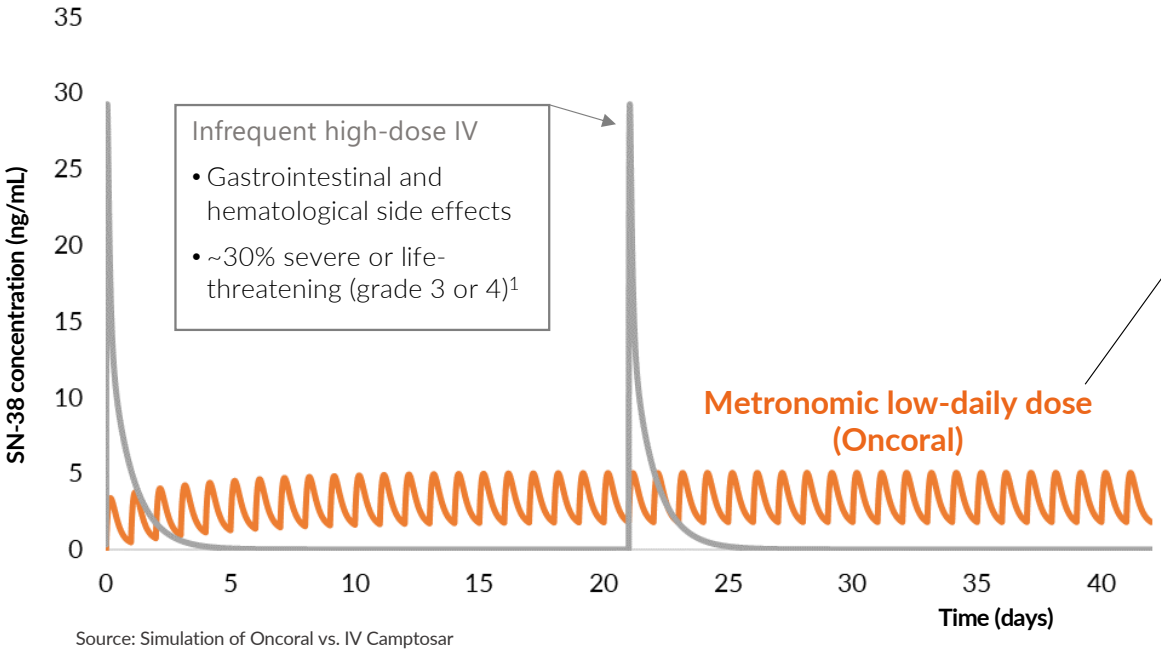
# ONCORAL PHASE 1: ENCOURAGING SAFETY PROFILE

**PLASMA LEVELS OF IRINOTECAN**




Source: Simulation of Oncoral vs. IV Camptosar

## Frequent (metronomic) low-dose irinotecan

- Several studies show improved tolerability[2,3]
- Daily dosing – adjust quickly if acute toxicity

## Oncoral Phase 1 results

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

1) Camptosar prescribing information 2) Furman et al 1999 3) Perez et al 2004 4) Kumler et al 2018



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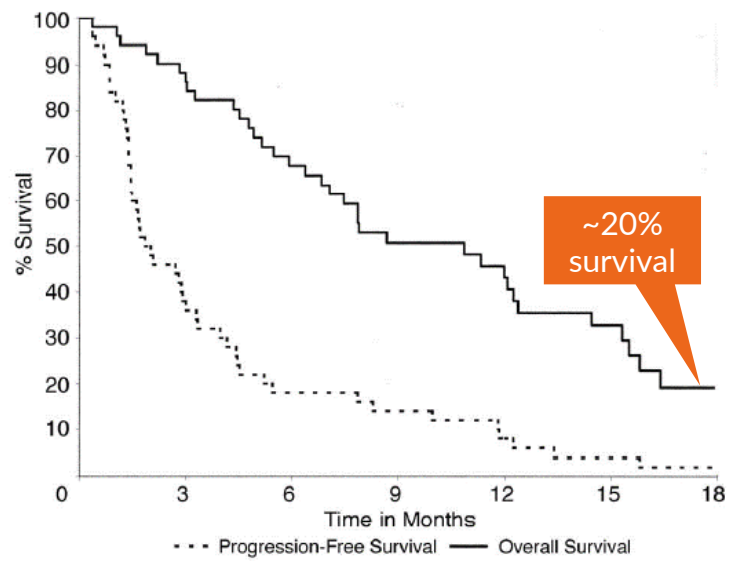
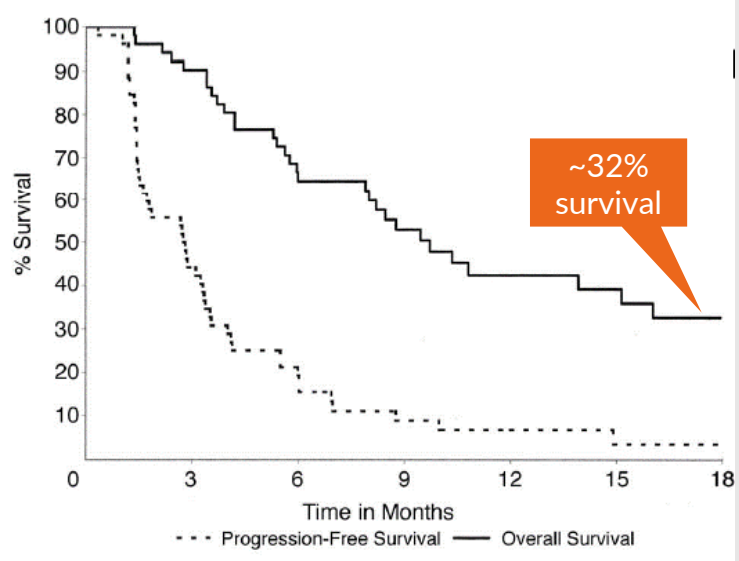
# IMPROVING IRINOTECAN **EFFICACY** BY FREQUENT LOW DOSING

Overall survival: Improved from 20% (dosing every third week) to 32% (weekly dosing)[1]

**Irinotecan every 3 weeks (IV)**




**Irinotecan weekly (IV)**




*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



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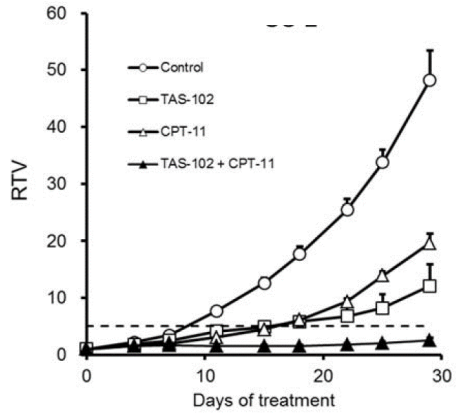
# ONCORAL PHASE 2 IN GASTRIC CANCER

## 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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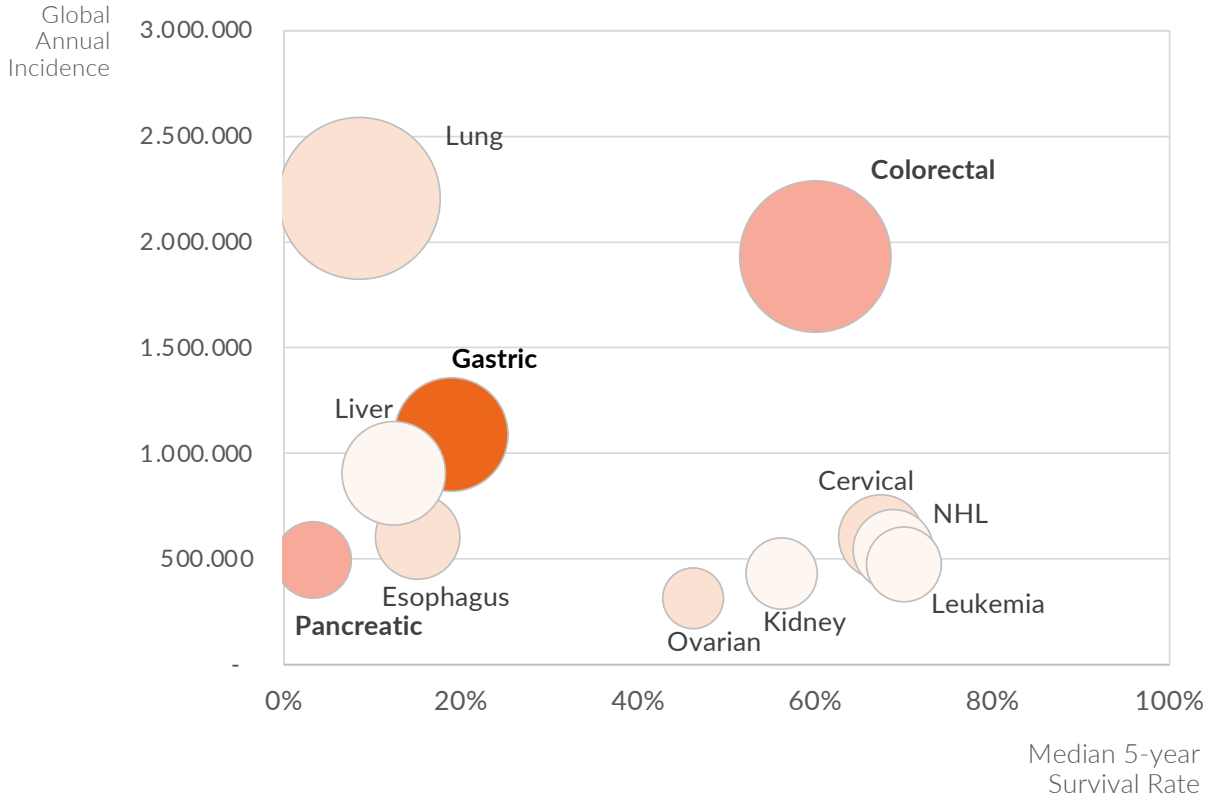
# HIGH VALUE OPPORTUNITY IN GASTRIC CANCER AND EXPANSION

## 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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# PRIORITIES, FINANCIALS AND SUMMARY

# PRIORITIES 2023

- Complete Orviglance Phase 3 patient enrollment
- Generate SPARKLE headline results
- Prepare Orviglance launch



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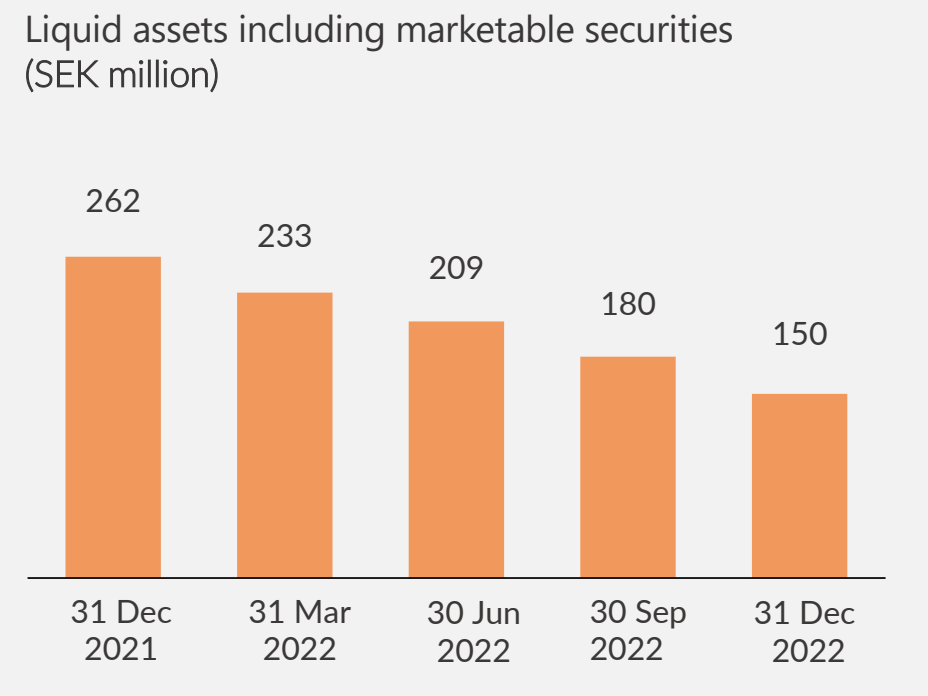
# FINANCIAL HIGHLIGHTS Q4 2022 – LIQUIDITY POSITION

Liquidity position:

- Liquid assets of 150 MSEK ($14.3 million) by 31 Dec 2022
- Current cash position provides financing into Q4 2023
- Quarterly burn rate in FY 2022 of 36-37 MSEK ($3.5 million)



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