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**WEBCAST:**

4 November 2021, 10:00AM CET

Link webcast:

[Ascelia Pharma Q3 Report 2021  
\(streamfabriken.com\)](https://streamfabriken.com/Ascelia-Pharma-Q3-Report-2021)

*Dial-in teleconference:*

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# PRESENTATION OF Q3-2021 REPORT

*Present from Ascelia Pharma:*

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

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# INVESTMENT 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** (MANGORAL) – in global Phase 3; FDA Orphan Drug Designation; U.S. launch expected H2 2023
  - **ONCORAL** – starting Phase 2 in Q4 2021\*

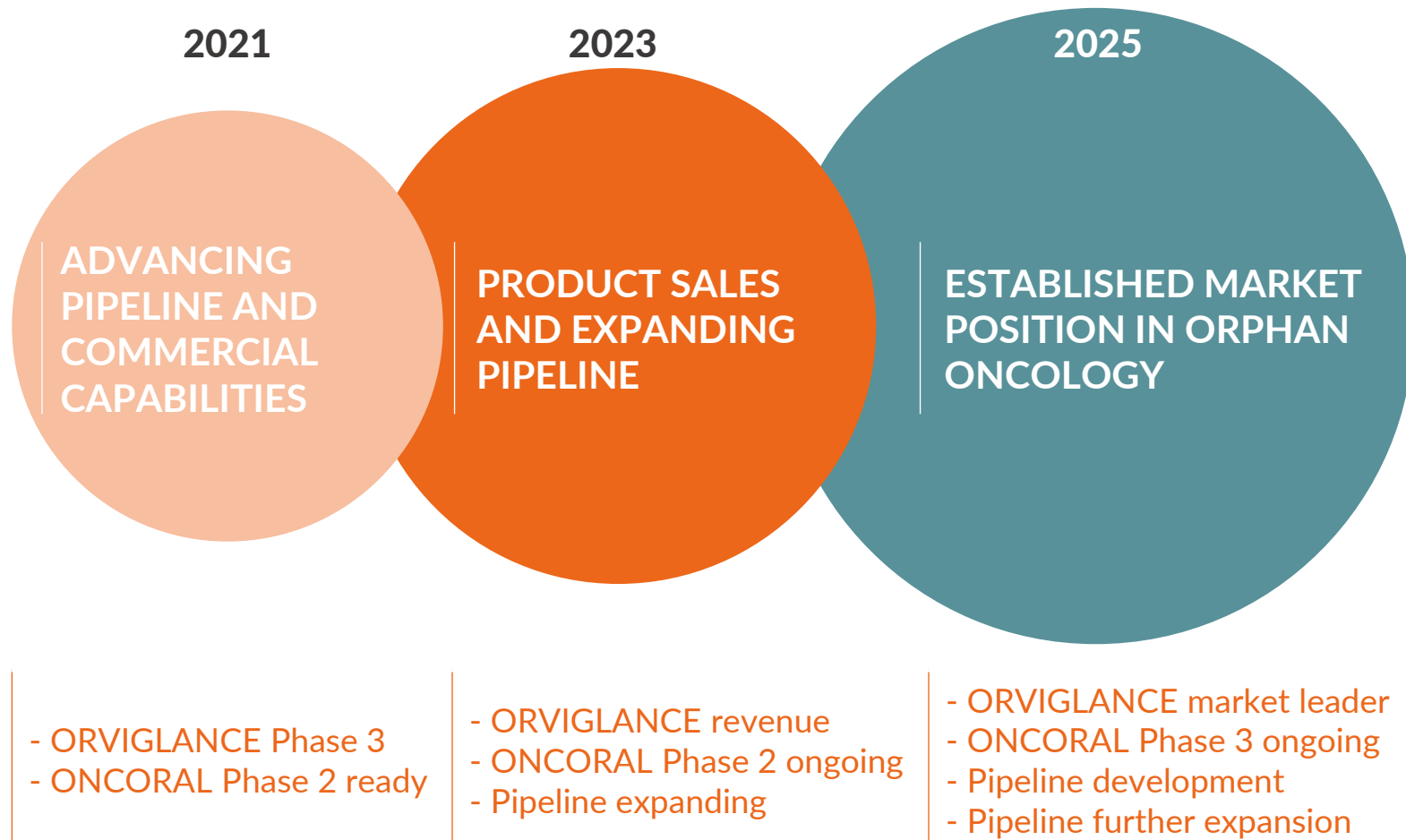
## BUILDING GLOBAL CAPABILITIES

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- Financed well into 2023
- Based in Malmö (Sweden) & Woodbridge, NJ (US)
- Listed on NASDAQ Stockholm (Ticker: ACE)

\*Expected timing for study start approval (IND approval)

# BUILDING VALUE





# RECENT KEY EVENTS

## Key events Q3-2021

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- Aug** FDA conditionally accepted Orvigance® as the brand name for Mangoral
- Aug** Abstract for Orvigance comparison study to gadolinium accepted as an oral paper presentation at the world's largest radiology conference RSNA
- Aug** Guidance for expected recruitment completion for the SPARKLE study moved to H1 2022 (previously H2 2021)
- Sep** Clinical collaboration agreement with Taiho Oncology Inc. for the development of Oncoral in combination with LONSURF®

## Key events after the quarter

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- Oct** Food Effect Study with Orvigance successfully completed



# ONCORAL – CLINICAL COLLABORATION WITH TAIHO ONCOLOGY

## DEVELOPMENT OF ONCORAL IN COMBINATION WITH LONSURF®

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- Clinical Phase 2 collaboration with Taiho Oncology Inc. (part of the Otsuka Group)
- Taiho Oncology will supply Lonsurf as well as provide scientific expertise for the study
- Depending on the results, the collaboration may be extended for further development
- Ascelia Pharma retains full development and commercialization rights to Oncoral

### Clinical collaboration with



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

# ORVIGLANCE – FOOD EFFECT STUDY SUCCESSFULLY COMPLETED

## **Last Patient Last Visit in Food Effect Study** (part of the registration package for Orviglance)

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- Crossover study in 24 healthy volunteers in fasting condition versus two fed conditions (snack or full meal)
- Study objectives included food effects on Orviglance PK, PD and safety profile
  - Data will provide guidance on fasting requirements before Orviglance administration
- Preliminary data indicate that Orviglance was well tolerated in the study – final results expected late 2021/early 2022
- A potential removal of the current fasting requirement could further improve the convenience and ease the administration of Orviglance in clinical practice





# PORTFOLIO

## ORVIGLANCE (MANGORAL)

Liver diagnostic drug in ongoing Phase 3

## ONCORAL

Daily oral chemotherapy ready for Phase 2



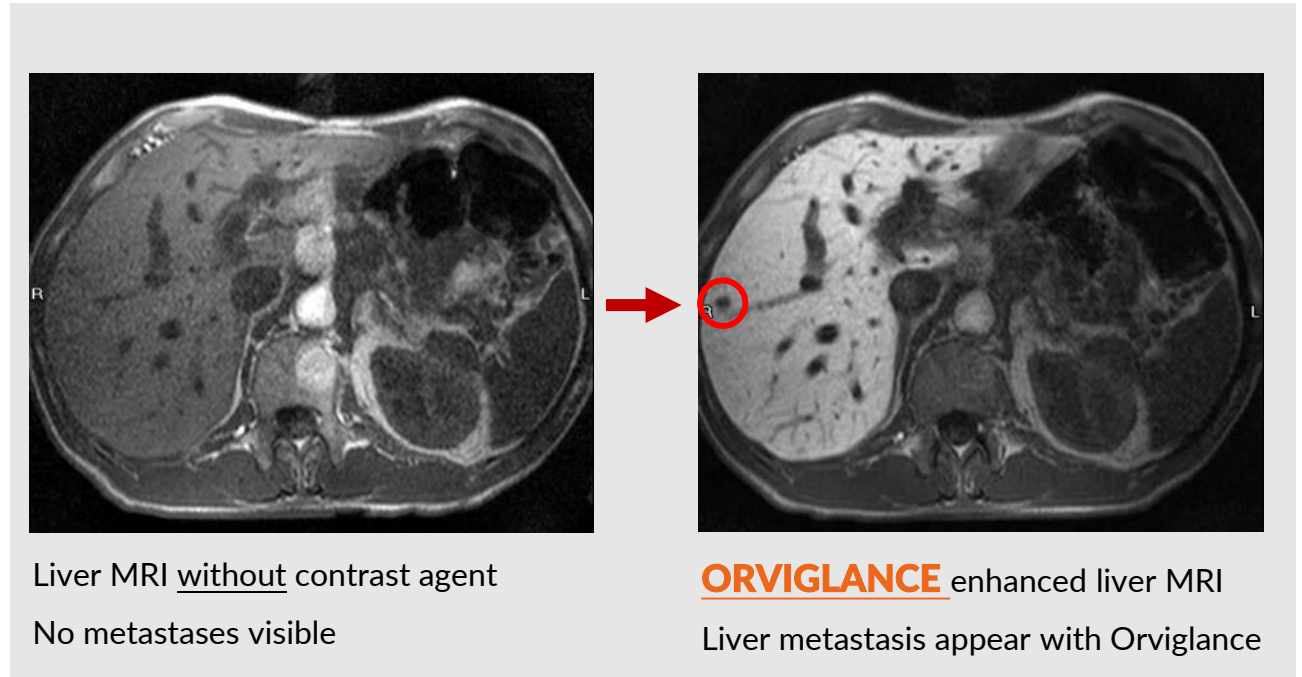
# ORVIGLANCE – PHASE 3 LIVER MRI CONTRAST AGENT

## NOVEL LIVER MRI CONTRAST AGENT

- Diagnostic drug for use in liver MRI scan to detect cancer
- Targeting patients at risk of potentially fatal side-effect from the current contrast agents on the market
- \$500-600 million addressable market with Orviglance as the only gadolinium-free agent

## SOLID PROGRESS

- Strong clinical Phase 2 results (p-values <0.0001)
- Ongoing Global Phase 3 study
- 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**









## Re-read study vs. gadolinium contrast agent (GBCA)

(20 patients)

- ORVIGLANCE lesion visualization as effective as GBCA  
(2 out of 3 readers favoured Orviglance)

Proceed into Phase 3

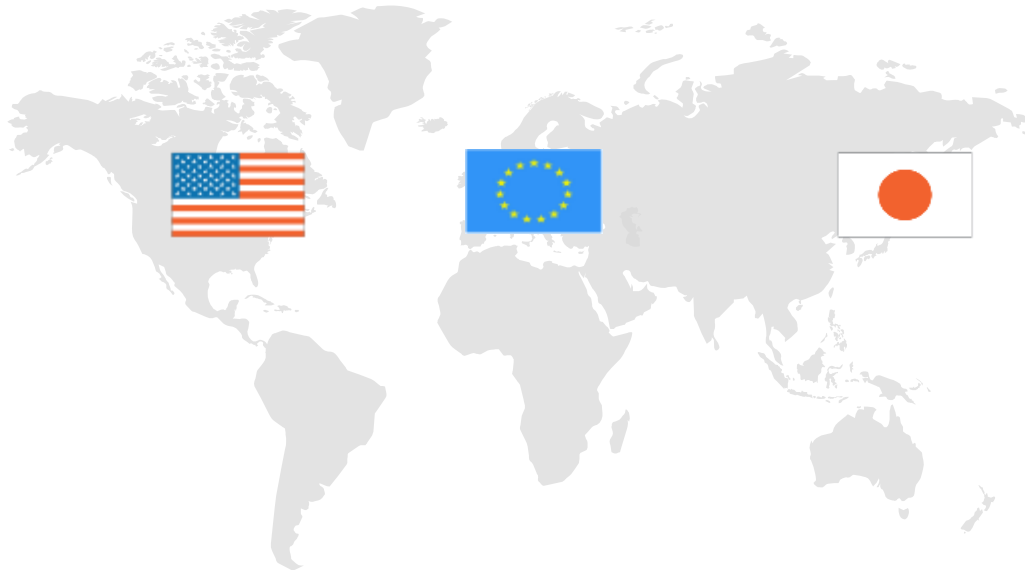
# ORVIGLANCE ONGOING PHASE 3 STUDY – SPARKLE

<b>Patients</b> 	<ul style="list-style-type: none"><li>• Global study, 200 patients</li><li>• Known or suspected focal liver lesions and severe renal impairment</li><li>• No randomization – each patient as own control</li></ul>		<b>Status update: US, Europe, Latin America</b> 40+ sites increasing to over 50 sites
<b>Comparator</b> 	Unenhanced MRI + ORVIGLANCE MRI vs. Unenhanced MRI		Internal control so not randomized
<b>Endpoint</b> 	<b>Lesion visualization</b> <ul style="list-style-type: none"><li>• Lesion border delineation</li><li>• Conspicuity</li></ul>		Same endpoints as Phase 2
<b>Follow-up</b> 	Less than a week		Expected pivotal study patient enrollment: H1 2022

# ORVIGLANCE MARKET \$500-600 MILLION ANNUALLY

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

- Ascelia Pharma to commercialize in the U.S
- 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%

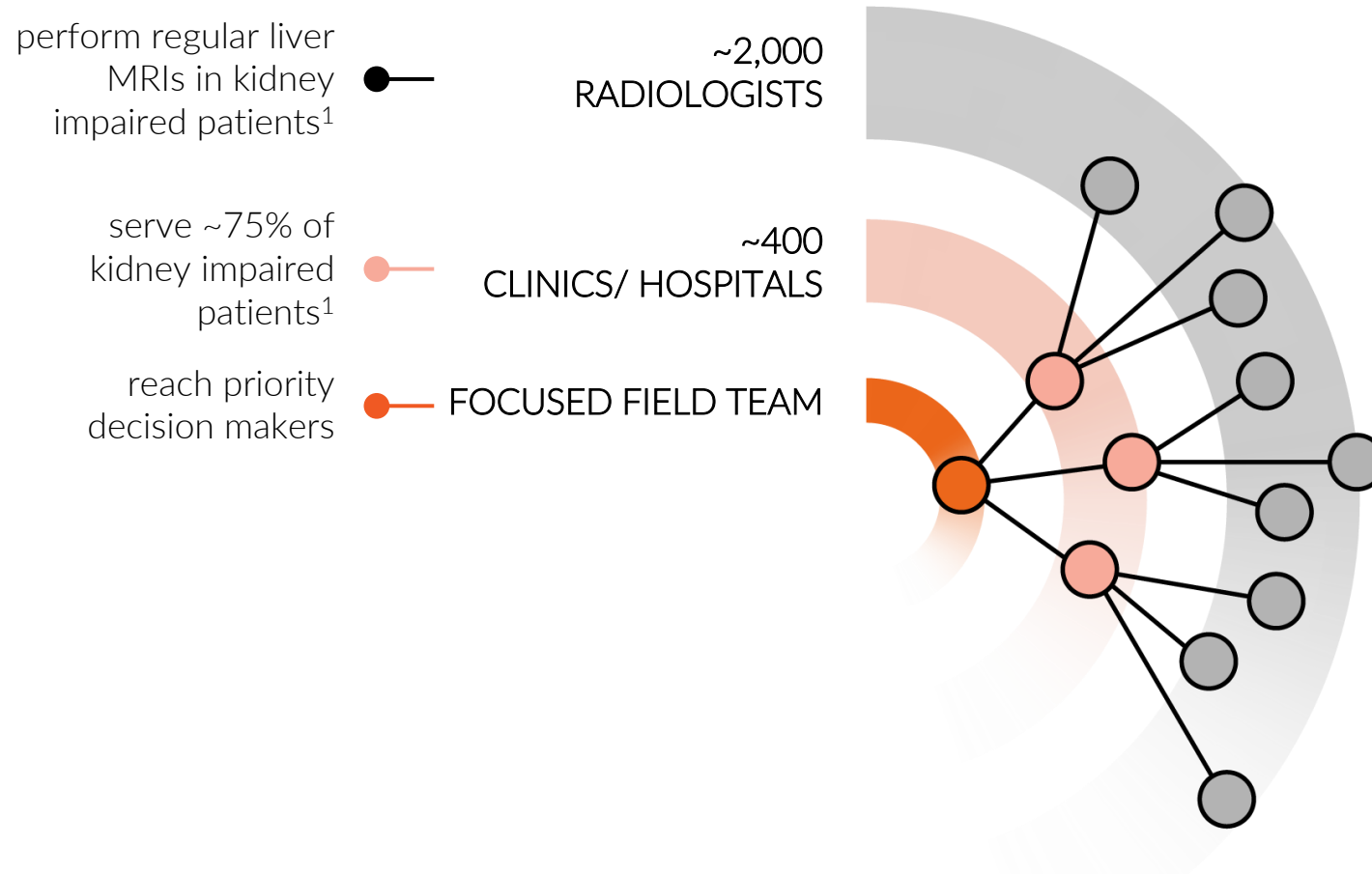
Sources:

1) Market research with Decision Resources Group, 2020

2) Market research and analyses with Revenue Reimbursement Solutions and Charles River Associates, 2020



# 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

## BUILDING OUT U.S. FOOTPRINT

SPARKLE Phase 3 study 13+ US Sites including Yale, Stanford, Mass. General, UCLA Medical Center

Sources:

1) Market research with Decision Resources Group, 2020



# PORTFOLIO

## ORVIGLANCE (MANGORAL)

Liver contrast agent in ongoing Phase 3

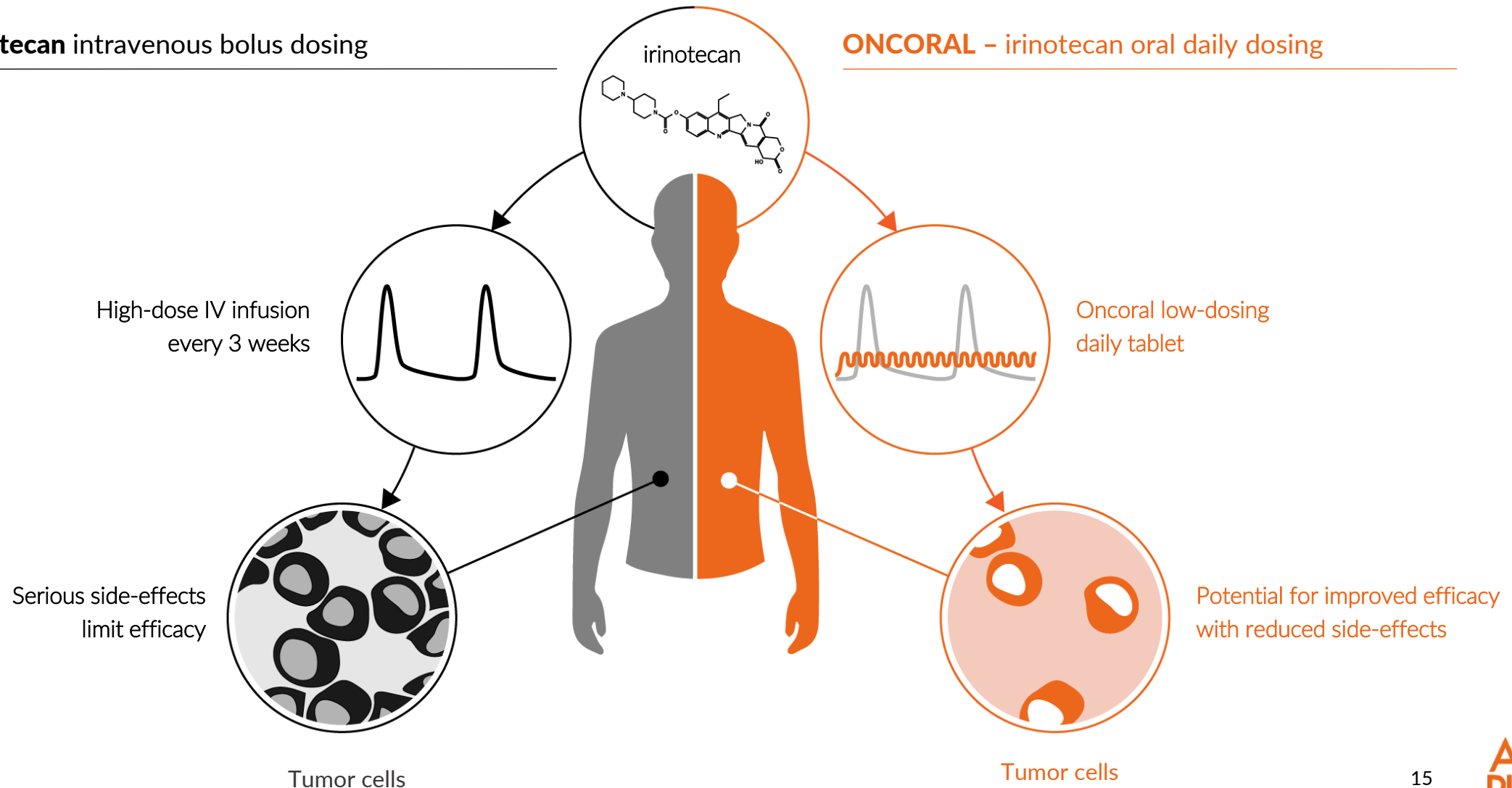
## ONCORAL

Daily oral chemotherapy ready for Phase 2

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

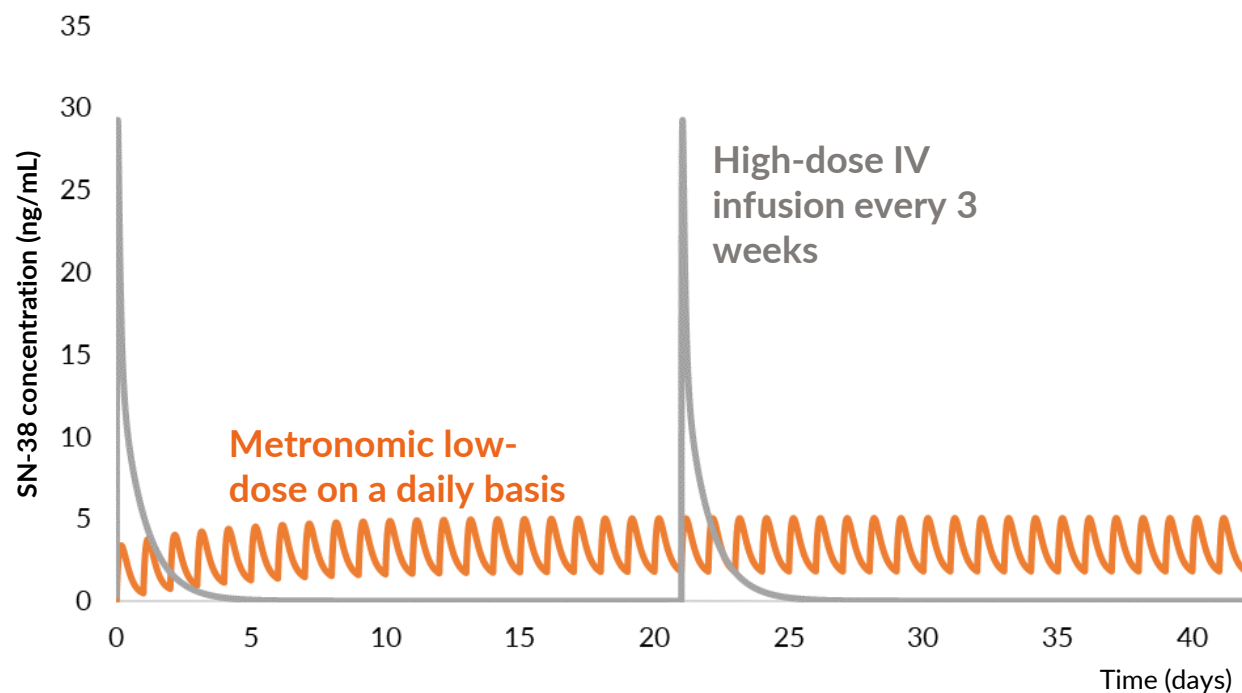
## **Irinotecan** intravenous bolus dosing

## **ONCORAL** – irinotecan oral daily dosing



# ONCORAL PHASE 1 RESULTS

## PLASMA LEVELS OF IRINOTECAN



Source: Simulation of Oncoral vs. IV Camptosar performed by Pkxpert AB

## 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

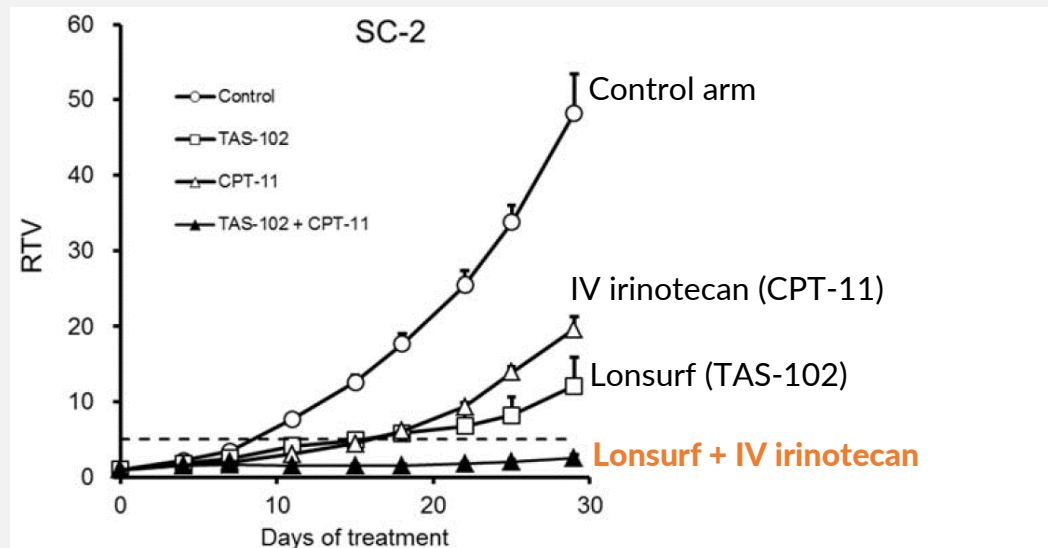


# ONCORAL PHASE 2 IN GASTRIC CANCER

## STRONG RATIONALE FOR GASTRIC CANCER

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

**Efficacy study in an animal model of gastric cancer<sup>1</sup>**  
(Relative Tumor Volume, RTV)



## STUDY DESIGN (ALL-ORAL COMBINATION STUDY)

### Patients



- Around 100 patients
- Metastatic gastric cancer
- Randomized controlled, multicenter, multinational study

### Comparator



Oncoral + Lonsurf  
vs.  
Lonsurf

### Endpoints



**Primary:** Progression Free Survival

**Secondary:** Response rate, PK, Safety and Overall Survival data in a follow up analysis

### Study period

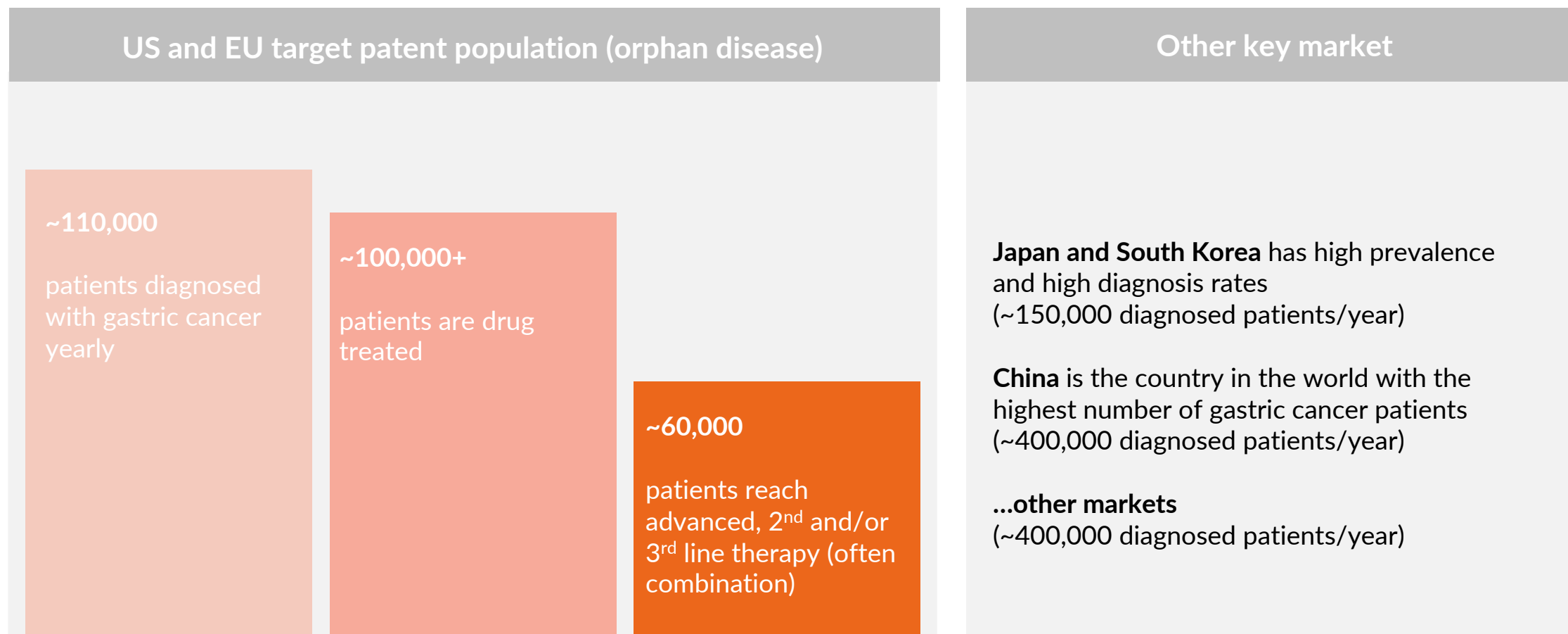


Q4 2021\* - 2024

\*Expected timing for study start approval (IND approval)

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

# GASTRIC CANCER – A \$3BN+ MARKET OPPORTUNITY

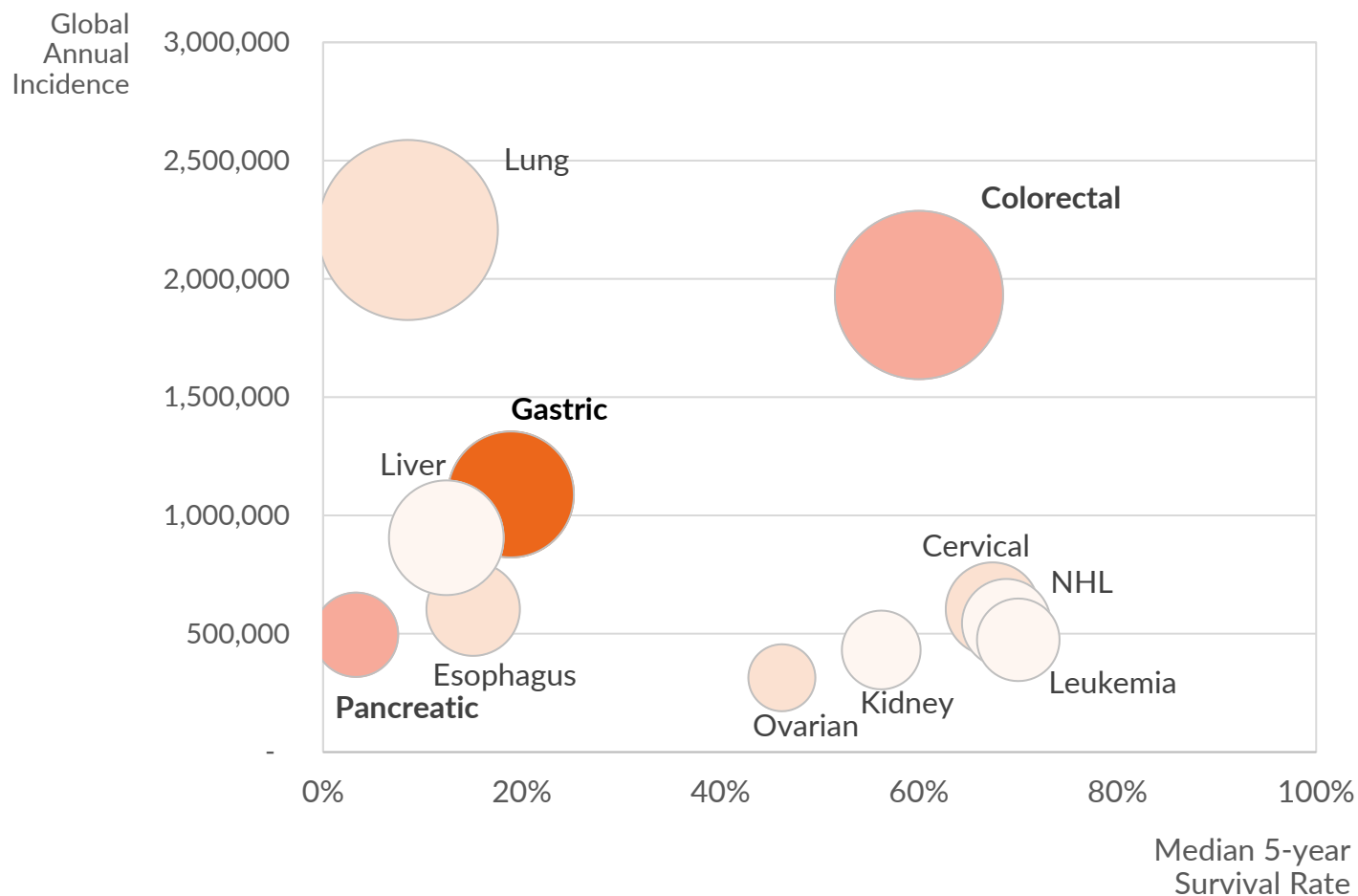


## Sources:

International Agency for Research on Cancer (IARC, 2021, input from key opinion leaders and Ascelia analysis)  
GlobalData - Gastric and Gastroesophageal Junction Adenocarcinoma – Global Drug Forecast and Market Analysis to 2024

# 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



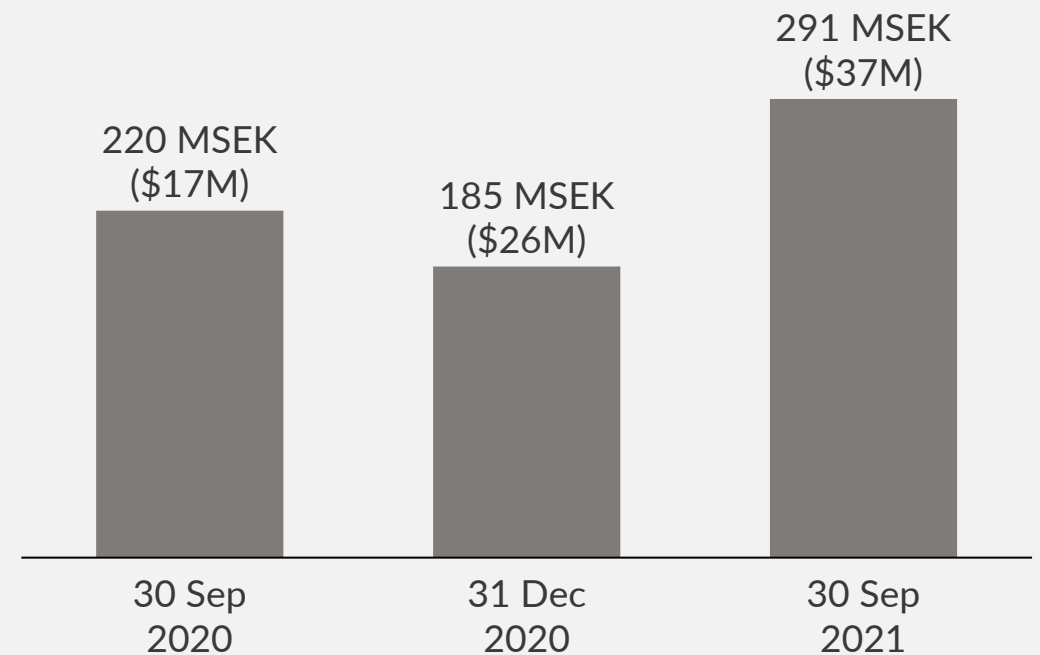


# FINANCIAL HIGHLIGHTS Q3 2021 – LIQUIDITY POSITION

## Solid liquidity position:

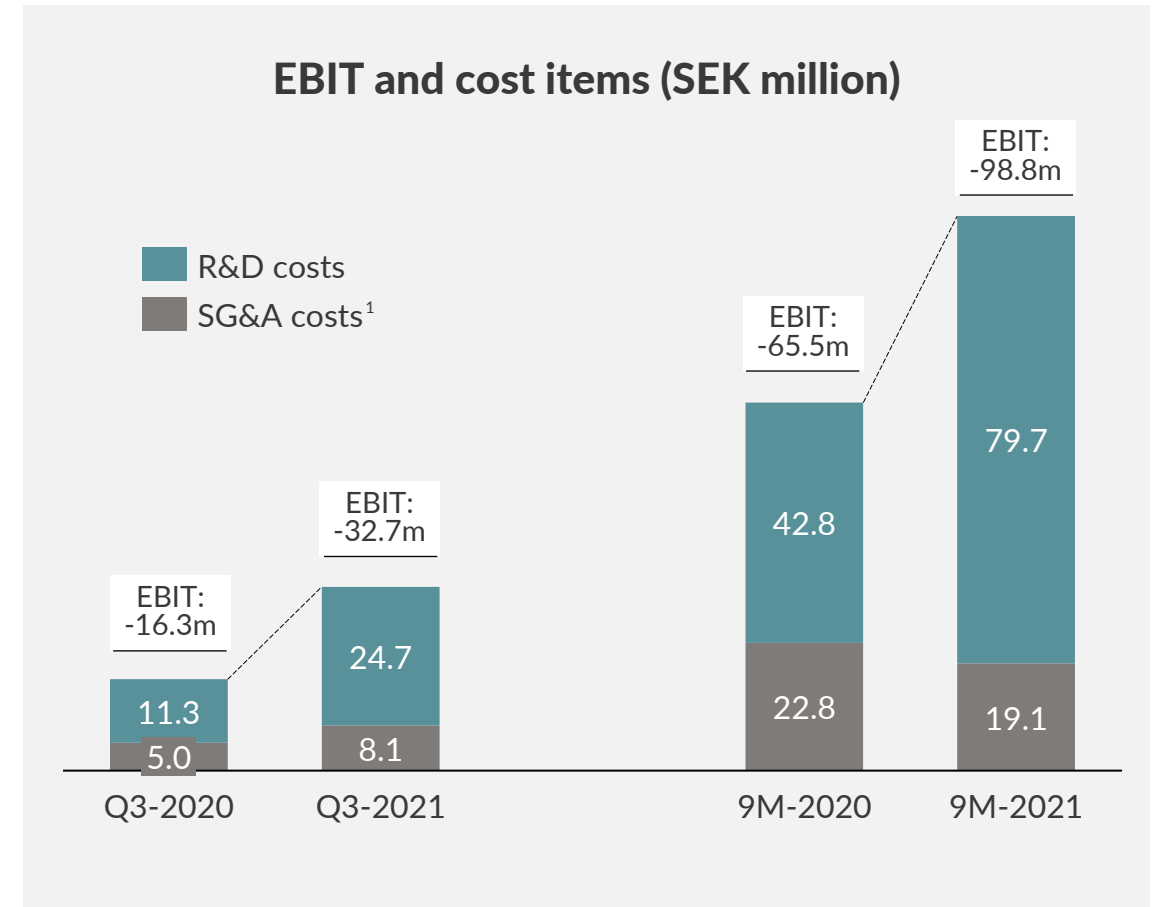
- Liquid assets of 291 MSEK (\$34 million) by 30 Sep 2021
- Quarterly burn rate in 9M 2021 of ~30 MSEK (\$3-4 million)
- Current cash position provides financing well into 2023

## Liquid assets including marketable securities (million)



# FINANCIAL HIGHLIGHTS Q3 2021 – OPERATING RESULTS

- Increased operating loss y/y mainly driven by higher R&D activity for Orvigance Phase 3 study:
  - Clinical development
  - Manufacturing preparations
- Also higher R&D costs y/y due to Oncoral Phase 2 preparations



Notes:

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



## PRIORITIES AND KEY MILESTONES

- 1 Initiate Phase 2 study for Oncoral  
(study approval start, IND, expected Q4-2021)<sup>1</sup>
- 2 Complete Orvigance Phase 3 patient enrollment  
(expected H1-2022)<sup>1</sup>
- 3 Prepare Orvigance launch  
(planned for H2-2023)<sup>1</sup>

1) Timelines incorporate the currently assessed impact from Covid-19.  
An extended Covid-19 situation may further affect timelines.

# ASCELIA PHARMA

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