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**WEBCAST:**  
19 August 2021, 10:00AM CET

Link webcast:  
[Ascelia Pharma Q2 Report 2021  
\(streamfabriken.com\)](https://streamfabriken.com)

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# PRESENTATION OF Q2-2021 REPORT

*Present from Ascelia Pharma:*

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# ASCELIA PHARMA – ADVANCING ORPHAN ONCOLOGY



## ORVIGLANCE (MANGORAL)

- Diagnostic agent for liver MRI in population subset
- Global Phase 3 study ongoing
- \$500-600M annual addressable as the only gadolinium-free agent

## ONCORAL

- Oral daily chemotherapy – initial focus on gastric cancer
- Phase 2 to start H2-2021

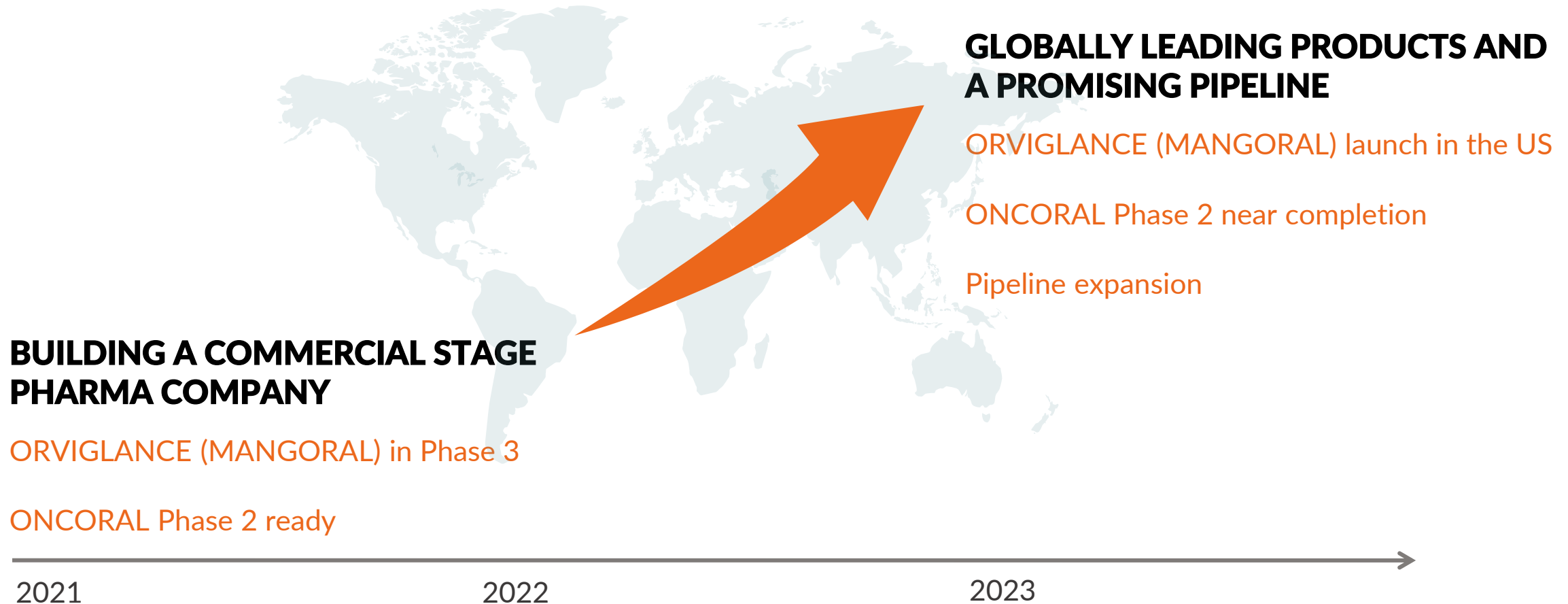
## BUILDING GLOBAL CAPABILITIES

- Global network of KOLs and advisors
- Driving approval and commercialization of Orviglance

## SOLID FINANCIAL POSITION

- Financed to reach key value creating milestones
- Listed on NASDAQ Stockholm

# BUILDING ASCELIA PHARMA AND BUILDING VALUE





# RECENT KEY EVENTS

## Key events Q2-2021

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**Apr** EGM approval of new share issuance

## Key events after the quarter

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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** Covid-19 extends recruitment period for SPARKLE study with up to 6 months into H1 2022 (previously H2 2021)



**ASCELIA  
PHARMA**

# ORVIGLANCE – THE BRAND NAME FOR MANGORAL

## Brand name approved by FDA and EMA

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- In August 2021, the FDA conditionally accepted Orviglance\* as the proposed brand name for manganese chloride tetrahydrate (Mangoral)
- The name Orviglance was developed in accordance with FDA's guidance for the submission and evaluation of proprietary names
- The name selection included a research study of healthcare practitioners across the U.S. to ensure accurate prescription and safety interpretation of the name
- EMA has earlier also approved the brand name



\* Trademark is registered in Europe and several other markets and submitted for registration in the US.

# ORVIGLANCE COMPARISON STUDY TO BE PRESENTED AT RSNA

## Upcoming oral paper presentation at RSNA 2021

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- Orviglance comparison study to a gadolinium-based contrast agent accepted as an oral paper presentation at the world's largest radiology conference RSNA
- RSNA conference to be held November 28 – December 3 in Chicago, Illinois
- Endpoints and evaluation criteria in the study same as in the ongoing Phase 3 study SPARKLE



## Results of the comparison study showed that:

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1. Orviglance enhanced MRI was as effective as gadolinium for visualization of focal liver lesions (in fact, 2 out of the 3 independent readers reported higher scores for Orviglance)
  2. Orviglance enhanced MRI provides improved diagnostic efficacy compared to MRI without a contrast agent using identical endpoints as in the ongoing pivotal Phase 3 study SPARKLE
- *Robust evidence of the diagnostic value that Orviglance offers*
- *Strengthens the data package to the regulatory authorities*

# COVID-19 CONTINUES TO AFFECT RECRUITMENT IN SPARKLE

## **Covid-19 impact**

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- Covid-19 has continued to be a challenge for clinical research globally since the outbreak early 2020
- Continued high infection rates in countries where SPARKLE is ongoing → negatively impacting study sites' ability and recruitment pace for clinical research
- Initiatives introduced to mitigate the impact from Covid-19 including the addition of clinical study sites

## **Extending estimated recruitment timeline**

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- In the context of the Covid-19 situation, the estimated recruitment timeline is extended up to 6 months into H1 2022 (previously H2-2021)





# PORTFOLIO

## ORVIGLANCE (MANGORAL)

Liver diagnostic drug in ongoing Phase 3

## ONCORAL

Daily oral chemotherapy ready for Phase 2

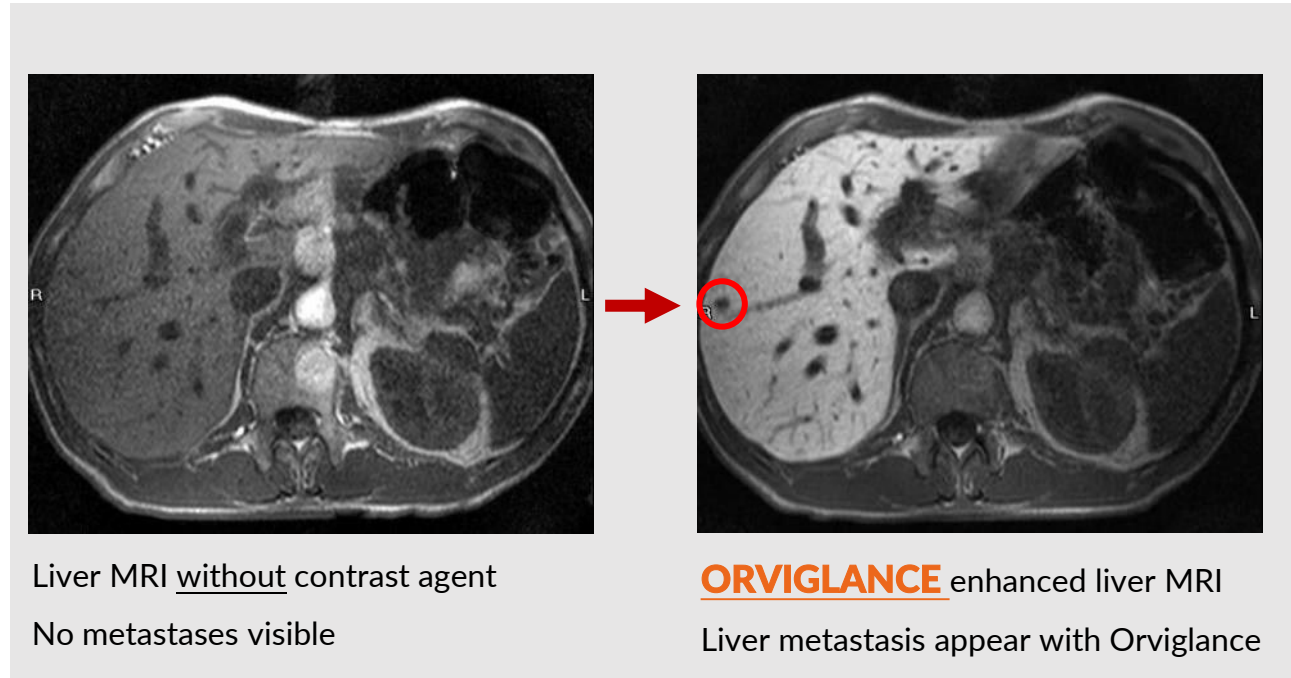
# ORVIGLANCE (MANGORAL)– PHASE 3 LIVER MRI CONTRAST AGENT

## 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



# ONGOING PHASE 3 STUDY SPARKLE

## PHASE 1 AND PHASE 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

## PHASE 3 STUDY (ONGOING)

Patients



Global study, 200 patients  
No randomisation – each patient as own control

Comparator



Unenhanced MRI + Orviglance MRI  
vs.  
Unenhanced MRI

Endpoint



**Lesion visualization**

- Lesion border delineation
- Conspicuity

Follow-up



Less than a week





## COMMERCIAL OUTLOOK ORVIGLANCE

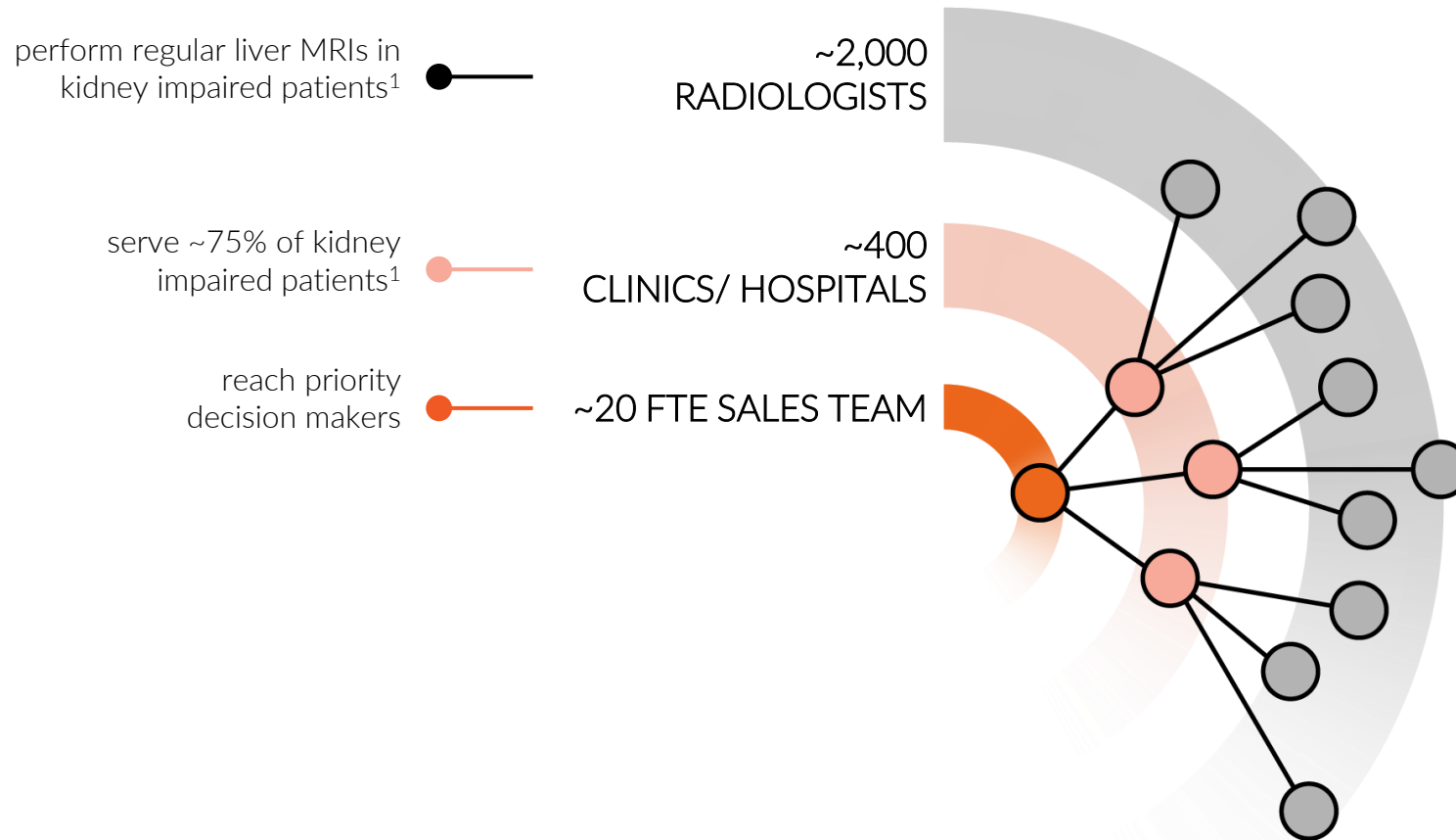
## ORVIGLANCE MARKET OPPORTUNITY AND COMMERCIAL PREPARATIONS

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- **Addressable market** of **\$500-600 million**
- Decision makers **understand the value** that Orviglance provides
- **Launch preparations progress** with a strong case for own commercialization in the US
- **US office** opened in New Jersey
- **US patent** for second-generation Orviglance provides patent protection to **year 2040**



# CAPTURING US MARKET VALUE WITH OWN TEAM



## BUILDING AN ASCELIA U.S. TEAM

NJ offices

Cambrex manufacturing partner  
RadMD imaging experts

## BUILDING OUT U.S. FOOTPRINT

Sparkle Phase 3 study  
at leading US sites



## PORTFOLIO

ORVIGLANCE (MANGORAL)

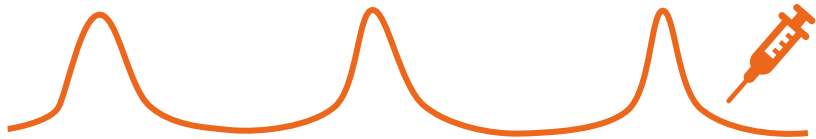
Liver contrast agent in ongoing Phase 3

**ONCORAL**

Daily oral chemotherapy ready for Phase 2

# ONCORAL – IRINOTECAN CHEMOTHERAPY AS TABLET

## **TODAY** – IV BOLUS INFUSION



- Widely used chemotherapy
- Established potent anti-tumour effect

### **UNMET NEEDS**

- Toxicity and gastrointestinal side-effects common
- Sub-optimal compromise between tolerability and efficacy

## **TOMORROW** – ONCORAL (ORAL, DAILY)



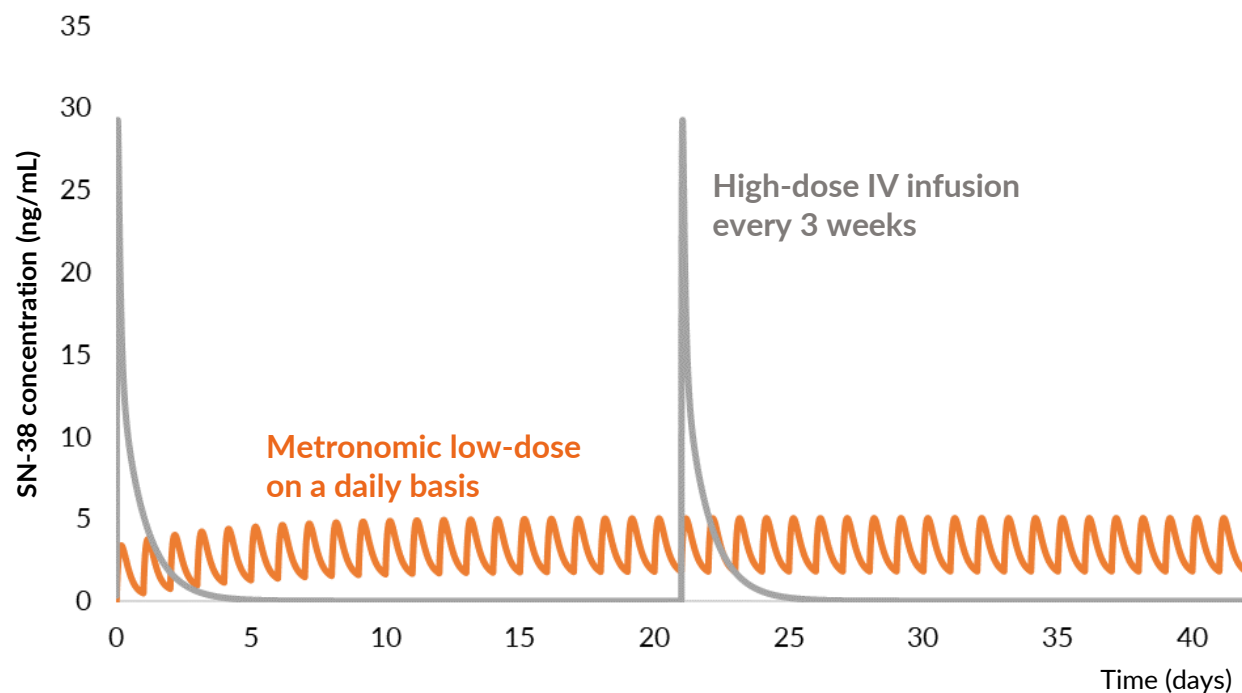
- Novel tablet formulation
- Enteric coating of active ingredient

### **POTENTIAL**

- Improved efficacy driven by pharmacokinetic/dynamic profile
- Improved tolerability due to lower peak exposure with less severe side effects and manageable toxicity with flexible dosing

# ONCORAL – WELL TOLERATED SAFETY PROFILE

## 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
- Haematological 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 haematological side effects, ~30% severe or life-threatening (grade 3 or 4)<sup>1</sup>

## Frequent (metronomic) low-dose irinotecan

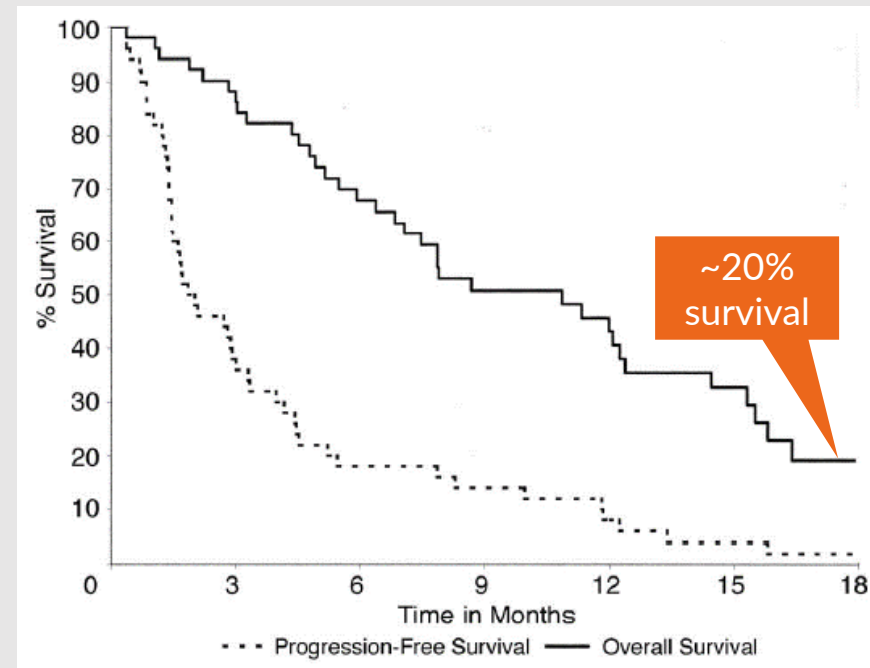
- Several studies show improved tolerability<sup>2,3</sup>
- Daily dosing – adjust quickly if acute toxicity



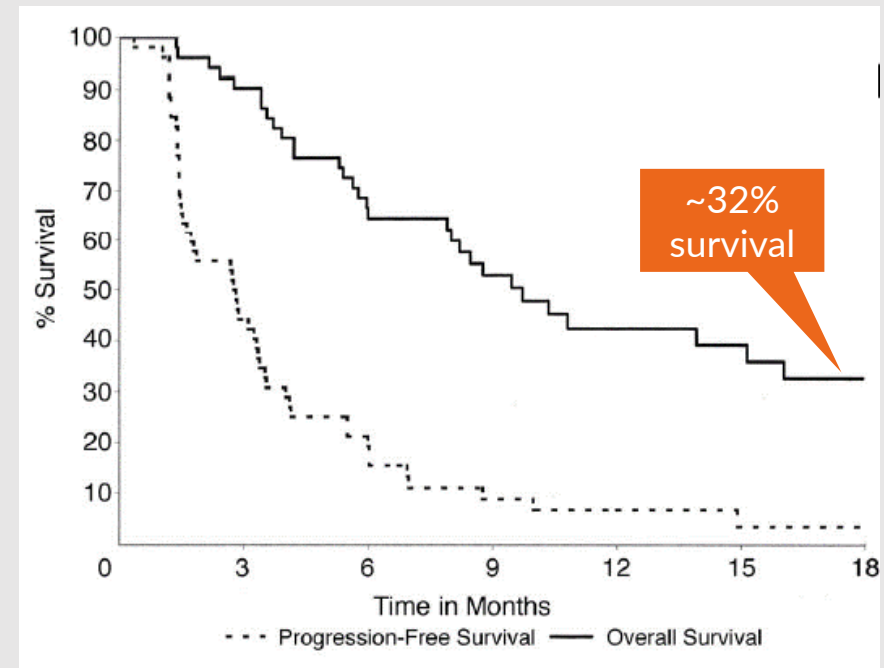
# IMPROVING IRINOTECAN **EFFICACY** BY FREQUENT LOW DOSING

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

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

# PHASE 2 – PREPARING TO START

## OBJECTIVES OF PHASE 2

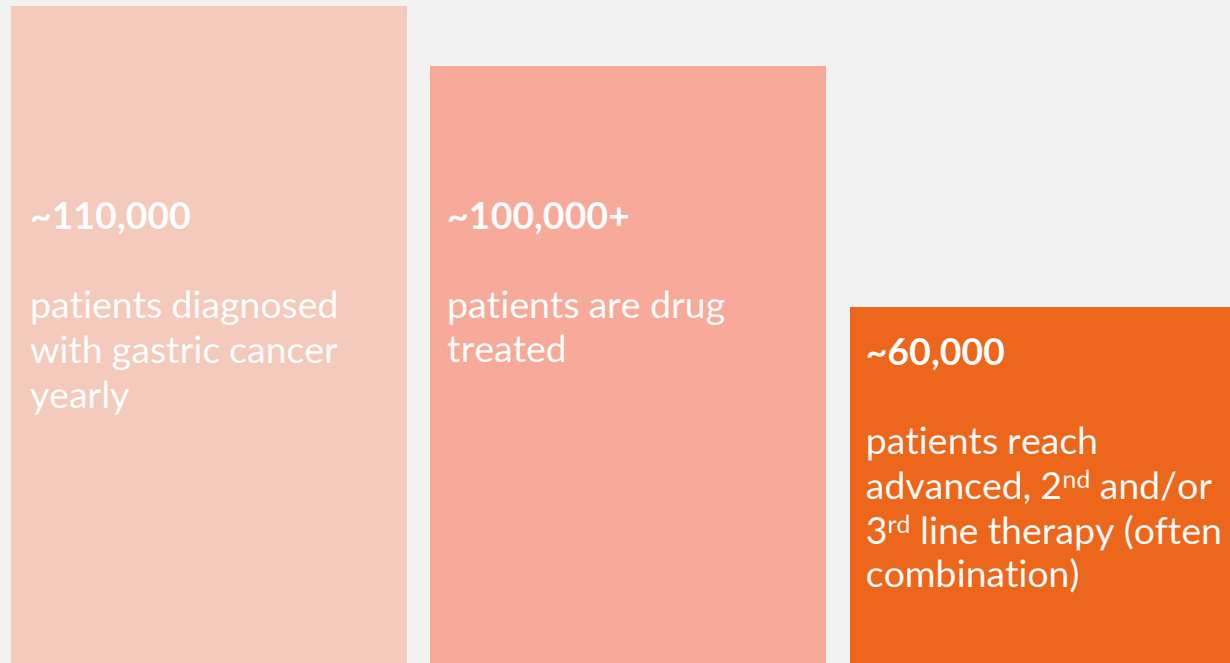
- Clinical proof-of-concept in metastatic gastric cancer
  - Potential orphan drug designation
  - Clinical guidelines support efficacy of irinotecan
- Compelling Phase 2 data package for further development
  - Potential for subsequent label expansion to other solid tumor indications

## STUDY DESIGN

Type of study 	Randomized controlled, multicentre, multinational study: Oncoral + Standard of Care <u>vs.</u> Standard of Care
Endpoints 	<b>Primary:</b> Progression Free Survival <b>Secondary:</b> Response rate, PK, Safety and Overall Survival data in a follow up analysis
No. of patients 	Approximately 100 patients
Study period 	H2 2021 – 2024

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

## US and EU target patent population (orphan disease)



## Other key market

**Japan and South Korea** has high prevalence and high diagnosis rates  
(~150,000 diagnosed patients/year)

**China** is the country in the world with the highest number of gastric cancer patients  
(~400,000 diagnosed patients/year)

**...other markets**  
(~400,000 diagnosed patients/year)

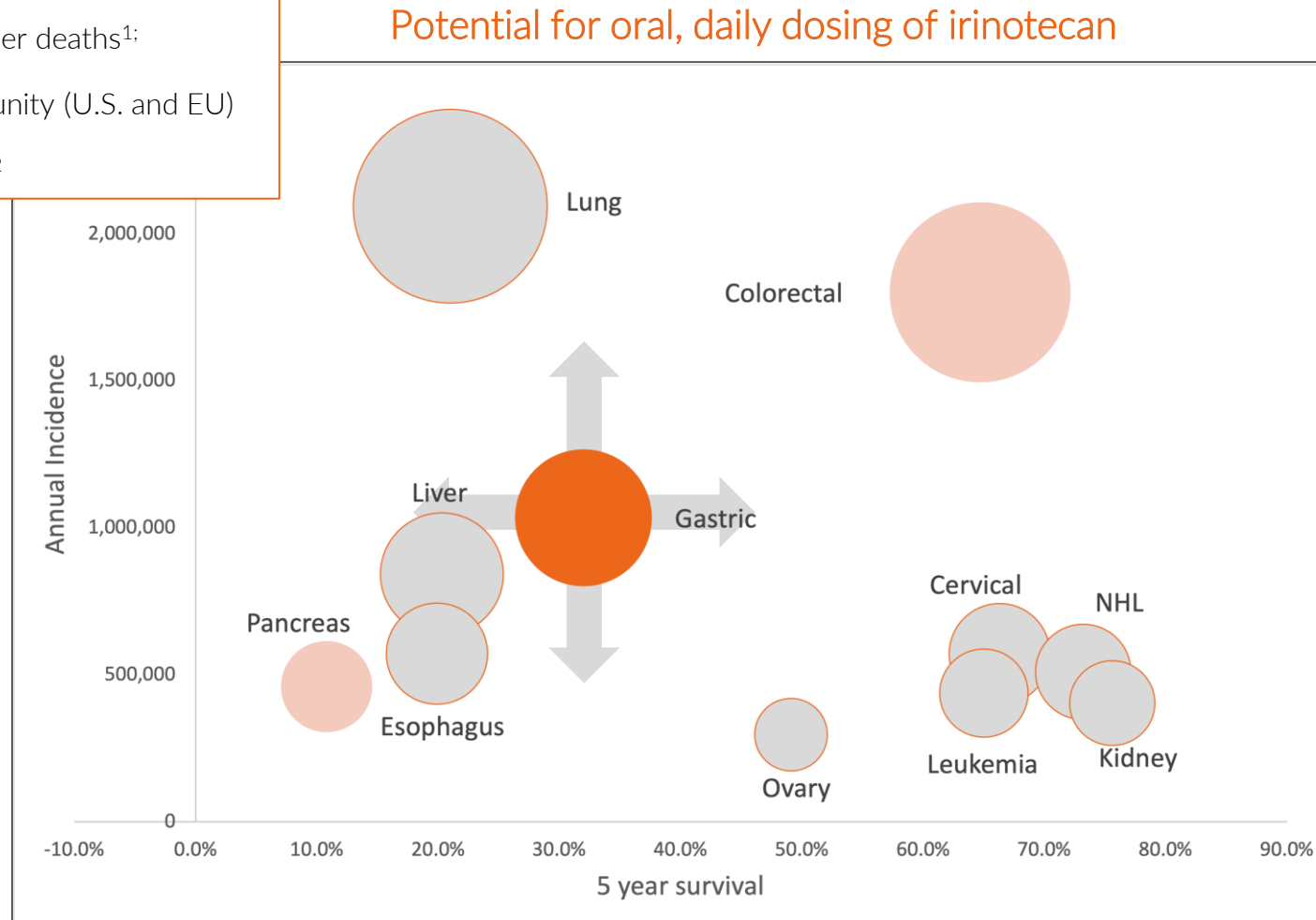
### Sources:

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

## GASTRIC CANCER

- 3<sup>rd</sup> highest cancer deaths<sup>1</sup>;
- Orphan opportunity (U.S. and EU)
- \$3-4bn market<sup>2</sup>



	Clinically demonstrated	NCCN recognized
Lung	✓	✓
Colorectal	Approved	
<b>Gastric</b>	✓	✓
Liver	✓	
Esophagus	✓	✓
NHL	✓	
Pancreatic	Approved	
Leukemia	✓	
Kidney	✓	
Cervical	✓	✓
Ovarian	✓	✓
Bone, Bile duct, CNS	✓	✓

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



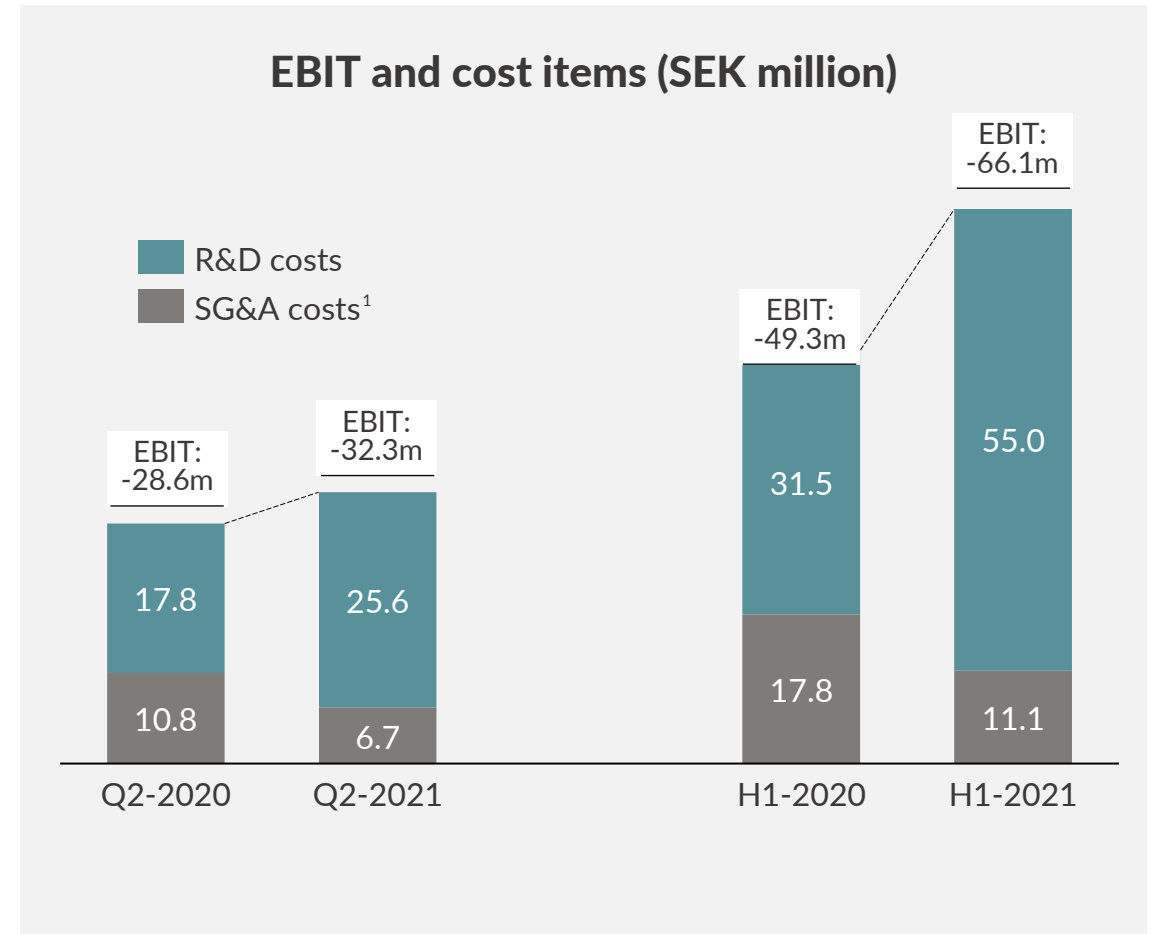
# FINANCIALS AND PRIORITIES



# FINANCIAL HIGHLIGHTS Q2 2021 – OPERATING RESULTS

Increased operating loss y/y mainly driven by higher R&D activity for Orviglance Phase 3 study:

- Clinical development
- Manufacturing preparations
- Regulatory preparations
- Also higher R&D costs y/y due to Oncoral Phase 2 preparations



Notes:

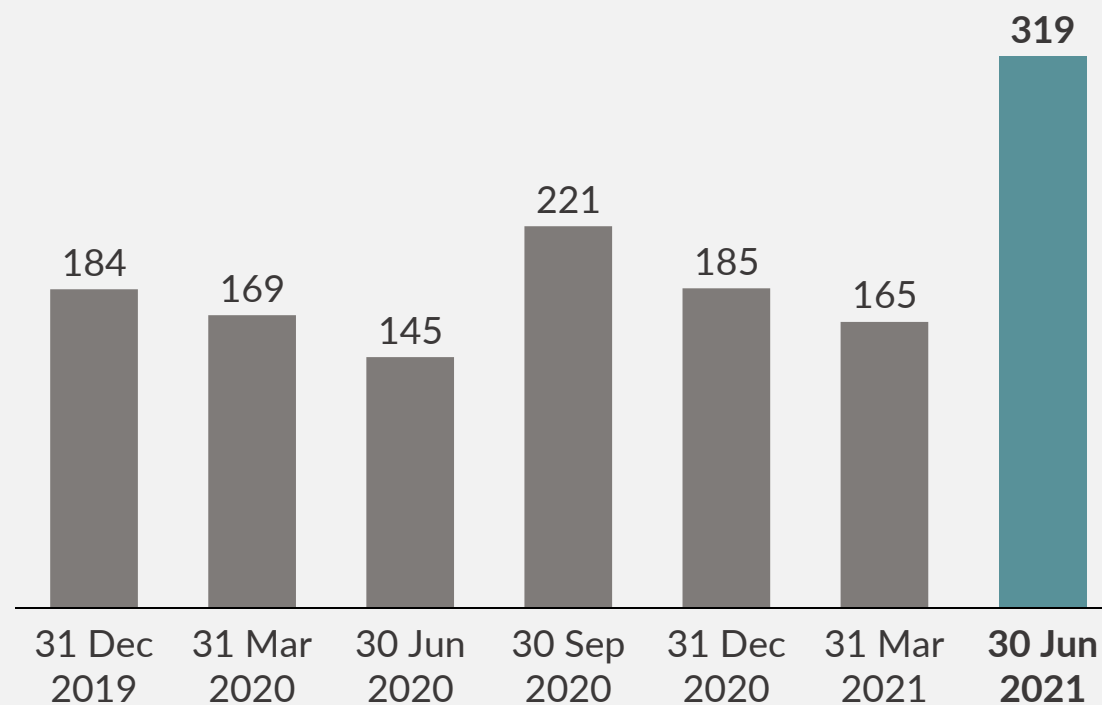
1) Other operating income and other operating costs added to SG&A

# FINANCIAL HIGHLIGHTS Q2 2021 – LIQUIDITY POSITION

## Solid liquidity position:

- Liquid assets of 319 MSEK by 30 June 2021
- Liquidity strengthened in Q2-2021 from new share issuance of 200 MSEK (net proceeds of 187 MSEK)
- Current cash position provides financing well into 2023
- Liquidity mainly to be used for ongoing Phase 3 study SPARKLE, pre-commercial activities as well as Oncoral Phase 2 study

## Liquid assets including marketable securities (SEK million)





## PRIORITIES

- Complete Orvigance Phase 3 patient enrolment (timeline could be extended into H1-2022)<sup>1</sup>

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- Prepare for Orvigance launch (planned for H2-2023)<sup>1</sup>

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- Initiate Phase 2 study for Oncoral (planned start in H2-2021)

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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