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**WEBCAST:**

12 May 2021, 10:00AM CET

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# PRESENTATION OF Q1-2021 REPORT

*Present from Ascelia Pharma:*

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

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# ASCELIA PHARMA – ADVANCING ORPHAN ONCOLOGY



## MANGORAL

- Diagnostic drug for liver MRI in population subset
- Global Phase 3 study ongoing
- \$500-600M annual addressable market with no competition

## 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 Mangoral

## SOLID FINANCIAL POSITION

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

# BUILDING ASCELIA PHARMA AND BUILDING VALUE

## BUILDING A COMMERCIAL STAGE PHARMA COMPANY

MANGORAL in Phase 3

ONCORAL Phase 2 ready

## WITH GLOBALLY LEADING PRODUCTS AND A PROMISING PIPELINE

MANGORAL launch in the US

ONCORAL phase 2 near completion

Pipeline expansion?

2021

2022

2023



# RECENT KEY EVENTS

## Q1-2020

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- Jan** Presentation of clinical development plan for Oncoral
- Mar** Opening of US office in preparation for Mangoral launch
- Mar** Capital raise of 200 MSEK (gross proceeds) to be used for: Oncoral Phase 2 and Mangoral pre-launch activities





# PORTFOLIO

## MANGORAL

Liver diagnostic drug in ongoing Phase 3

## ONCORAL

Daily oral chemotherapy ready for Phase 2



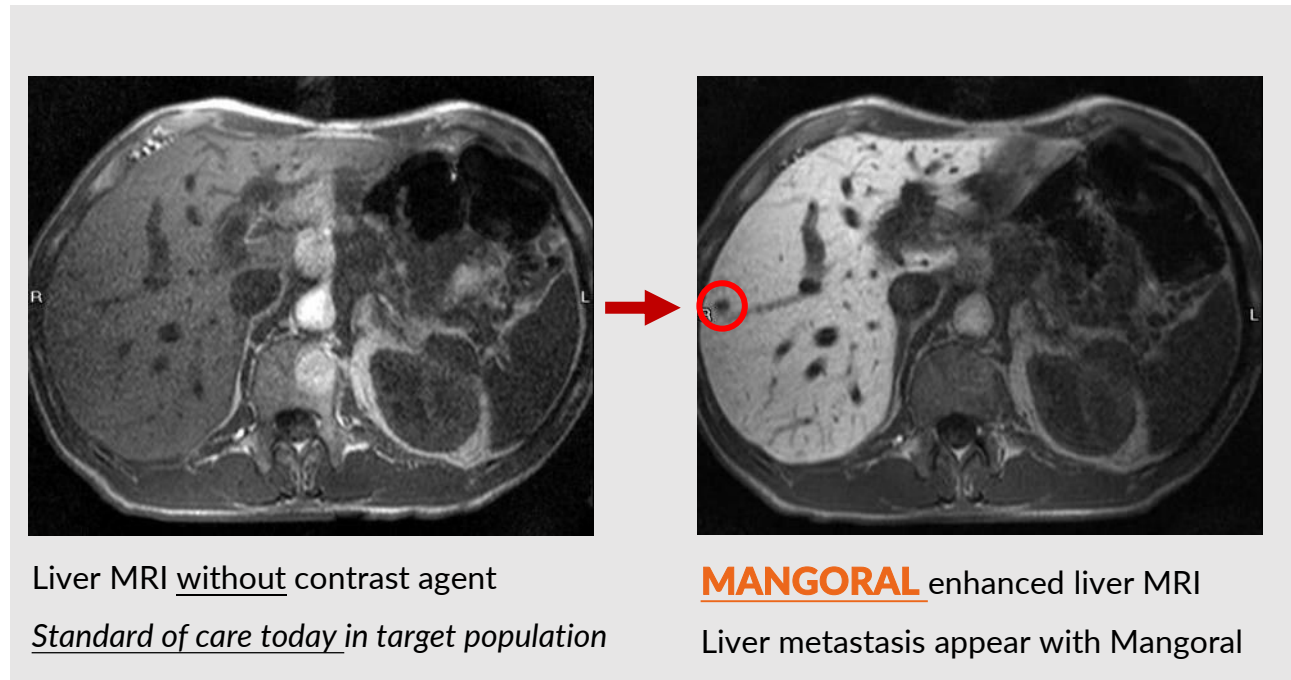
# MANGORAL – LIVER MRI CONTRAST AGENT IN PHASE 3

## 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
- \$500-600M addressable market with no competition

## SOLID PROGRESS

- Strong clinical Phase 2 results (p-values <0.0001)
- Ongoing Phase 3 – expected completion H2-2021
- 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)

- Mangoral 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 + Mangoral MRI  
vs.  
Unenhanced MRI

Endpoint



**Lesion visualization**

- Lesion border delineation
- Conspicuity

Follow-up



Less than a week





## COMMERCIAL OUTLOOK MANGORAL

### MANGORAL MARKET OPPORTUNITY AND COMMERCIAL PREPARATIONS

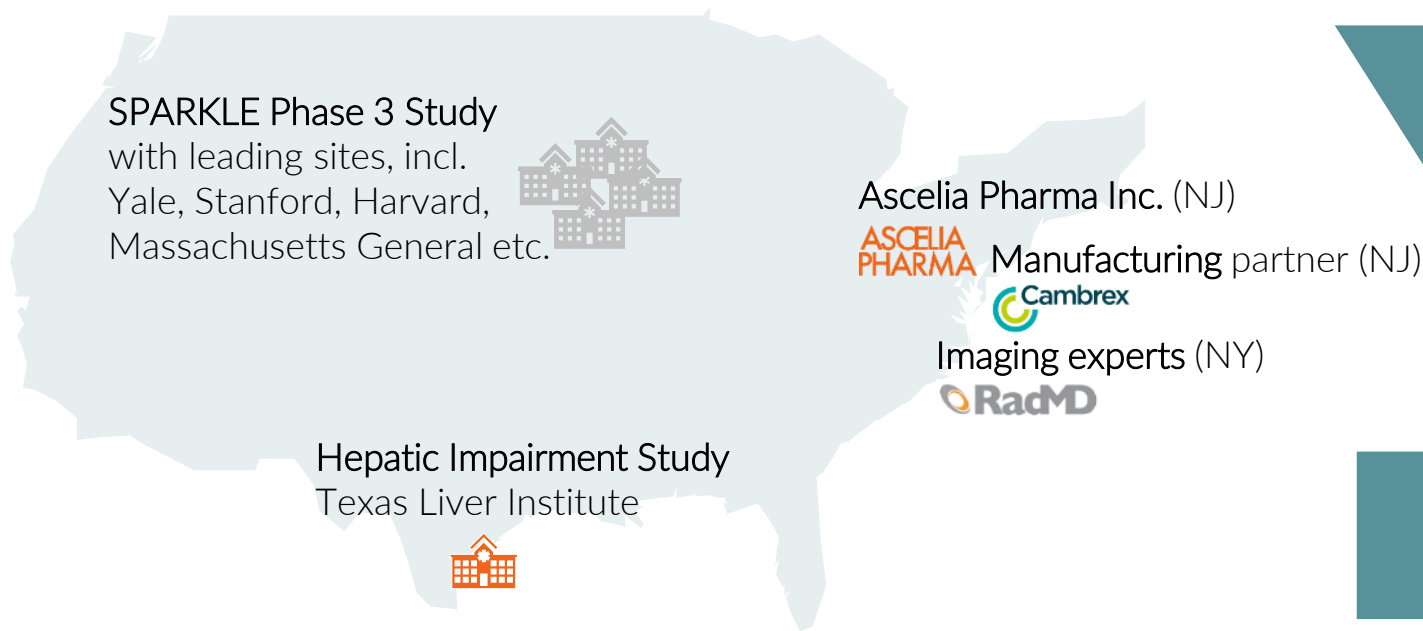
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- **Addressable market** of **\$500-600 million** with no competition
- Decision makers **understand the value** that Mangoral provides
- **Launch preparations progress** with a strong case for own commercialization in the US
- **US office** opened in New Jersey
- **US patent** for second-generation Mangoral provides patent protection to **year 2040**

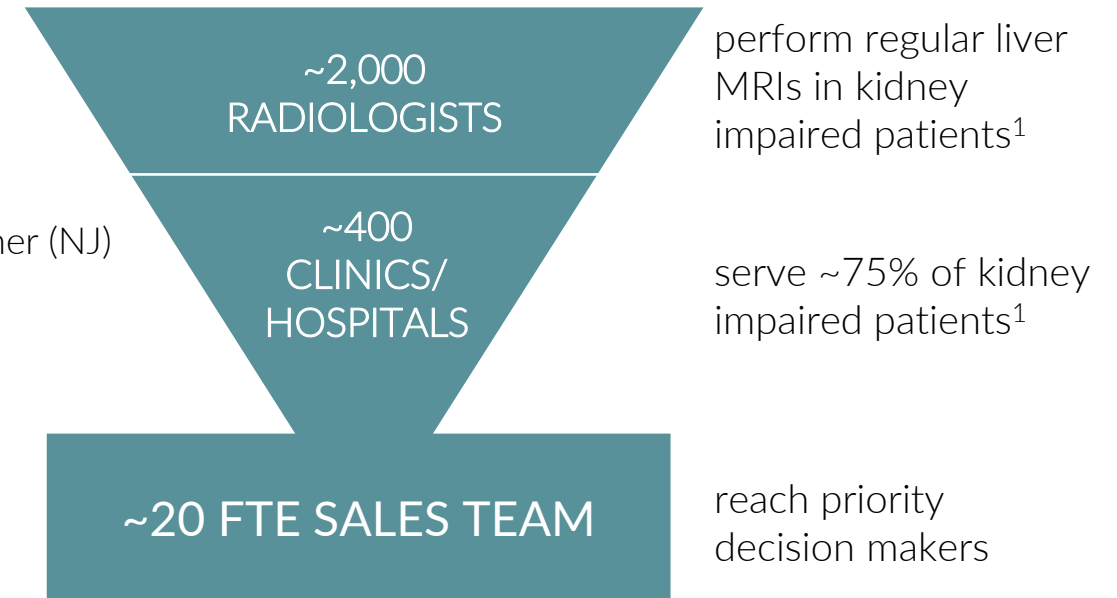
# CAPTURING US MARKET VALUE WITH OWN TEAM



## STRONG FOOTPRINT IN THE US



## BUILDING AN ASCELIA US TEAM





## PORTFOLIO

### MANGORAL

Liver contrast agent in ongoing Phase 3

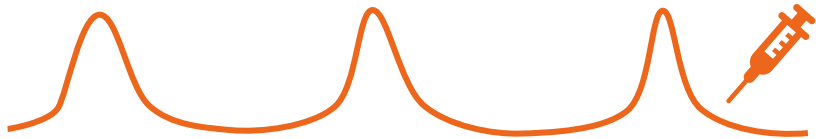
### ONCORAL

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

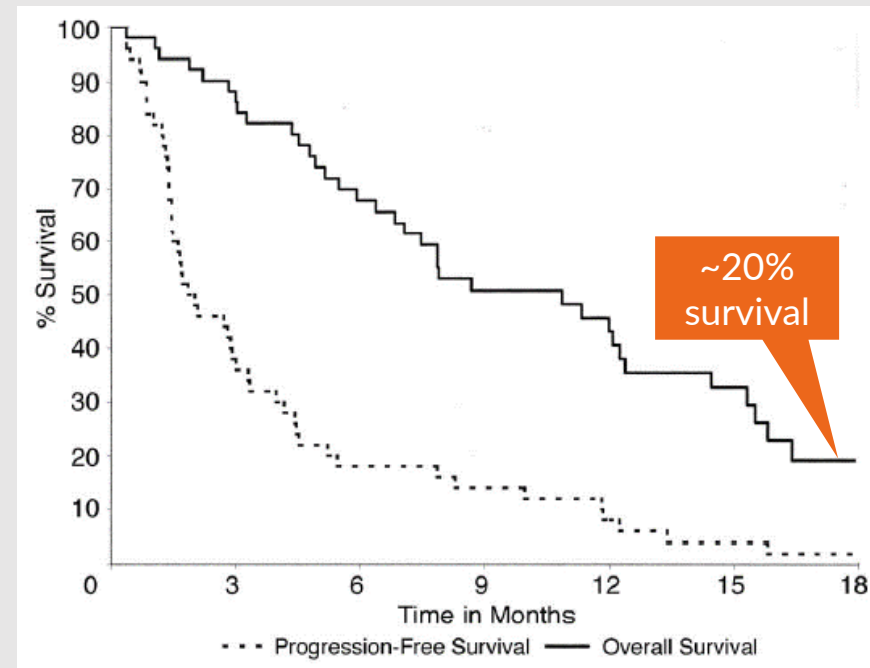
### **POTENTIAL**

- 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

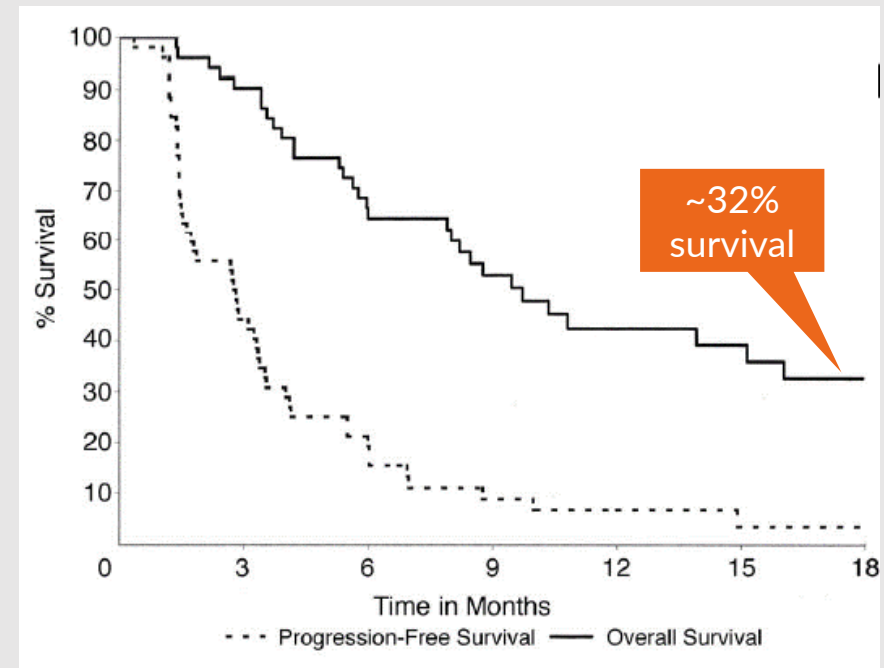
# IMPROVING IRINOTECAN **EFFICACY** BY FREQUENT LOW DOSING

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

Irinotecan every 3 weeks (IV)



Irinotecan weekly (IV)

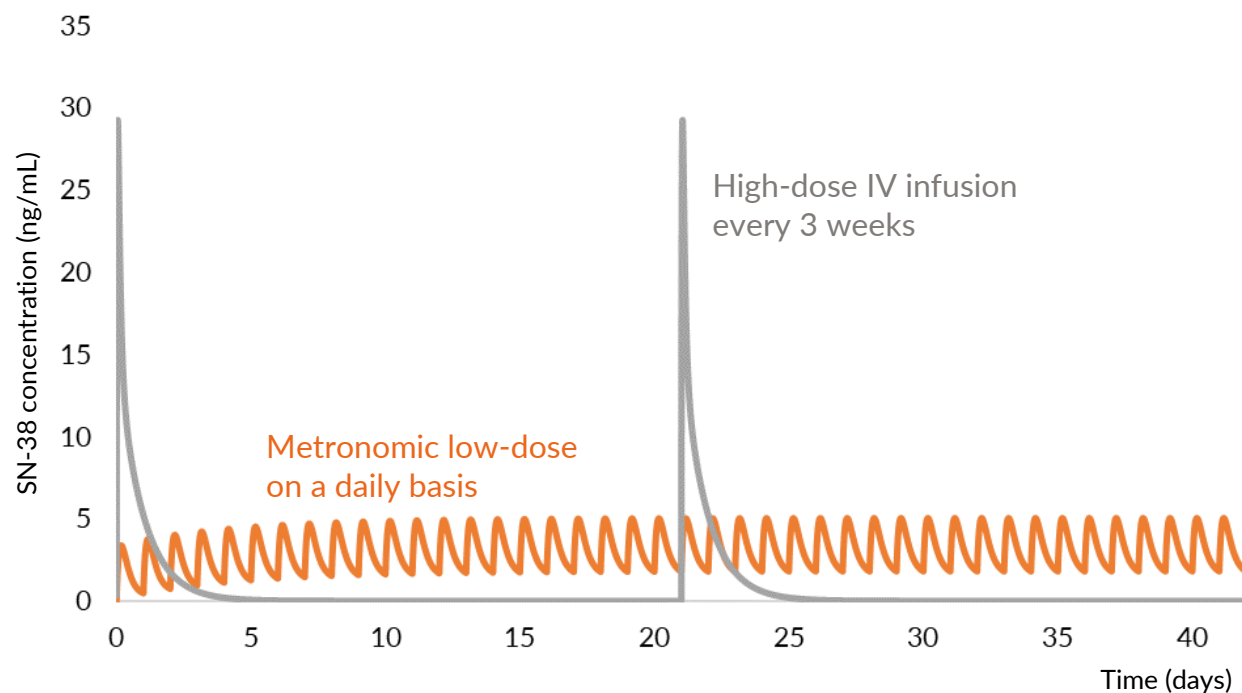


Study in patients with metastatic refractory breast cancer, N=103

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

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

## PLASMA LEVELS OF IRINOTECAN



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

### 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

### 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



# ONCORAL SCIENTIFIC ADVISORY BOARD

## Prof Josep Tabernero, MD, PhD

Head of the Medical Oncology Department at the Vall d'Hebron Barcelona Hospital Campus, Director of the Vall d'Hebron Institute of Oncology (VHIO), and Professor of Medicine

President (2018 – 2019) of ESMO and an Executive Board and Council Member



## Prof Eric Van Cutsem, MD, PhD

Professor and Division Head of Digestive Oncology at University of Leuven (KUL) and University Hospitals Gasthuisberg, Leuven, Belgium

Co-founded ESMO GI/World Congress on GI Cancer. Serves/served on the board/ committee of ESMO, ASCO, ENET, EORTC, ECCO, ESDO



## Prof Jaffer A Ajani, MD

Department of Gastrointestinal Medical Oncology, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, Houston, USA

Chairs the NCCN committee for gastroesophageal cancers



## Prof Jeff Evans, MD

Professor of Translational Cancer Research and Clinical Lead of the Institute of Cancer Sciences, University of Glasgow

Member of the NCRN Upper GI Cancer Pancreatic Cancer and Gastro-Oesophageal Cancer sub-groups



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*Joint view that Oncoral would be an important treatment option for cancer patients, especially in later disease stages*





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# PHASE 2 – STUDY IN PREPARATION

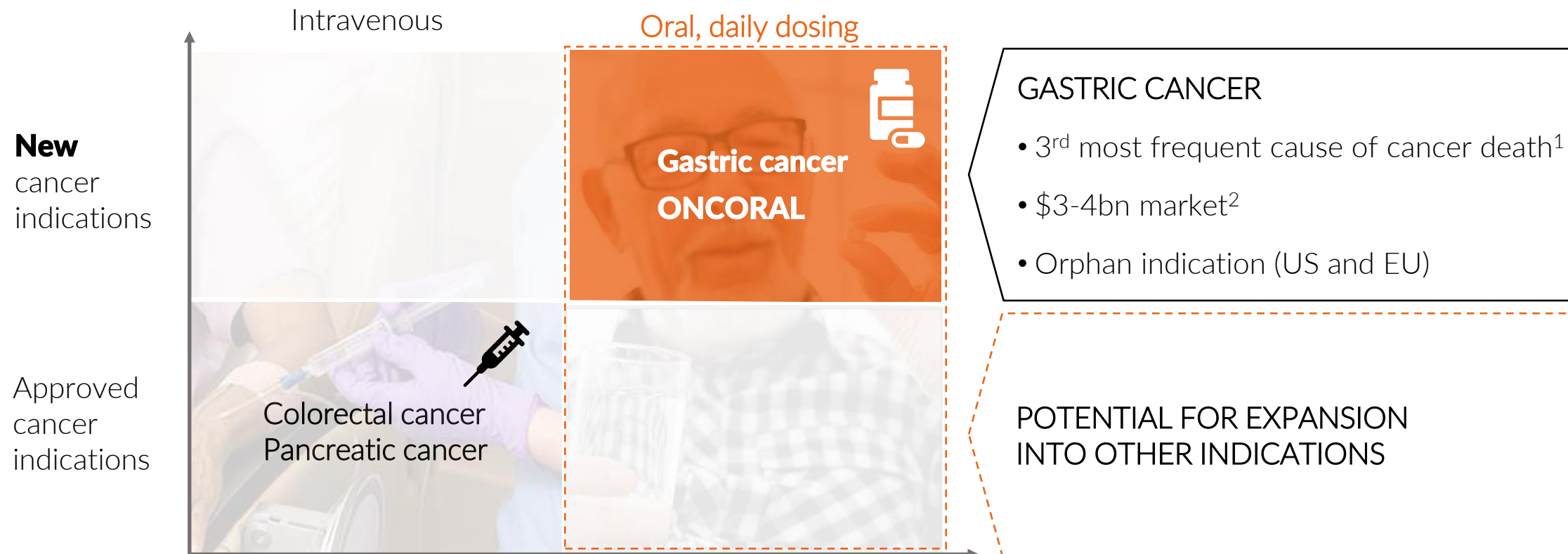
## OBJECTIVES OF PHASE 2

- 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

# HIGH VALUE OPPORTUNITY IN GASTRIC CANCER



1) International Agency for Research on Cancer (IARC, 2021)

2) GlobalData - Gastric and Gastroesophageal Junction Adenocarcinoma – Global Drug Forecast and Market Analysis to 2024



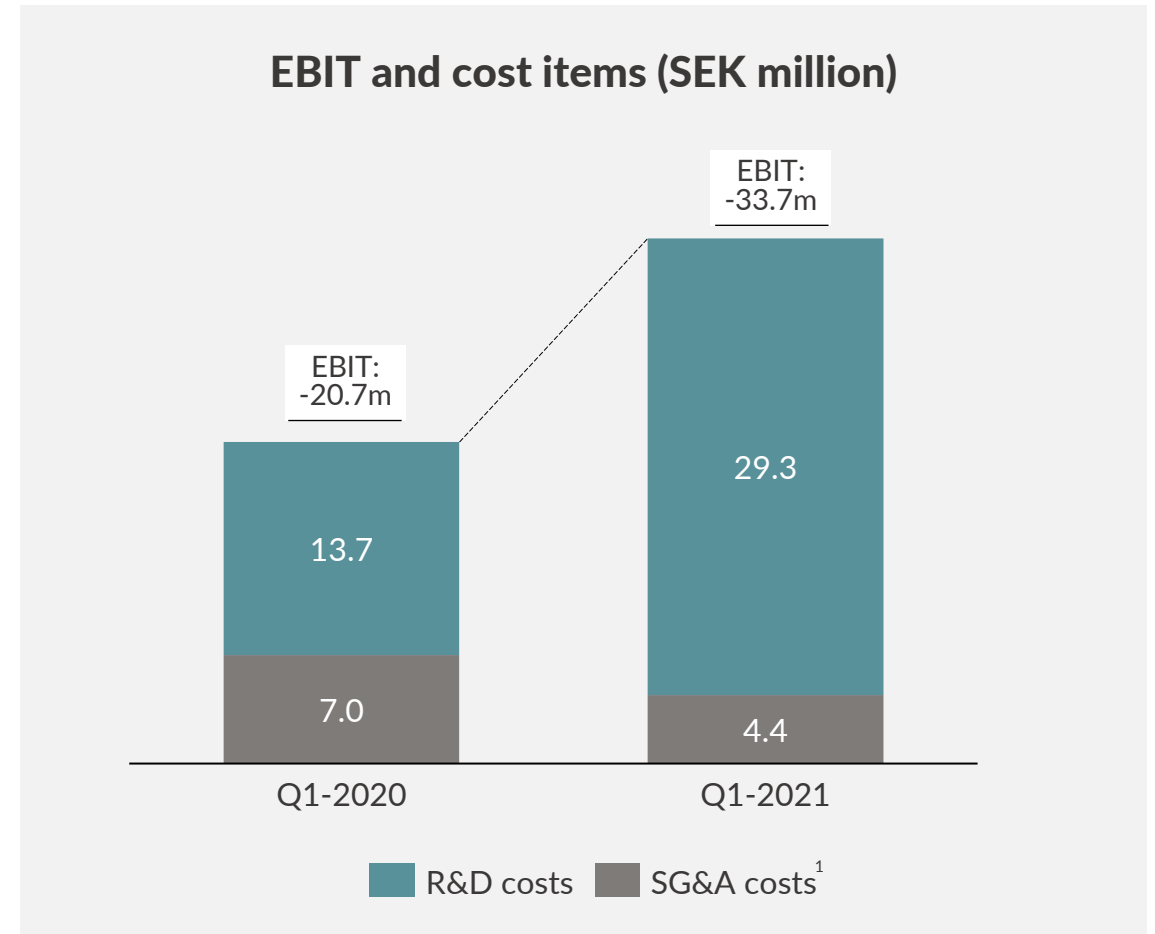
# **FINANCIALS AND PRIORITIES 2021**



# FINANCIAL HIGHLIGHTS Q1 2021 – OPERATING RESULTS

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

- Clinical development
- Manufacturing preparations
- Regulatory preparations



Notes:

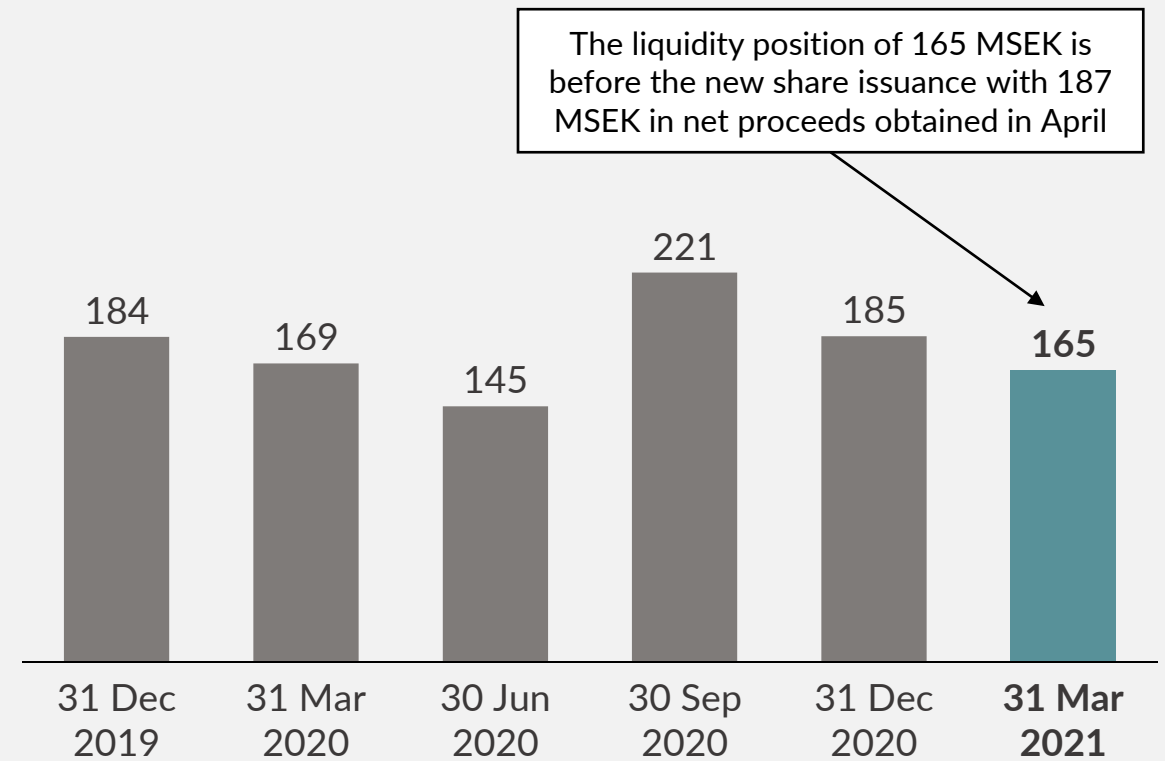
1) Other operating income and other operating costs included into SG&A

# FINANCIAL HIGHLIGHTS Q1 2021 – LIQUIDITY POSITION

## Solid liquidity position:

- Liquid assets of 165 MSEK by 31 March 2021
- New share issuance of 200 MSEK with net proceeds of 187 MSEK obtained in April 2021 following EGM approval
- Liquidity mainly to be used for Mangoral clinical Phase 3 and pre-commercial activities as well as Oncoral Phase 2

## Liquid assets including marketable securities (SEK million)







## PRIORITIES 2021

- Complete Mangoral Phase 3 patient enrolment (top line results planned for 2H 2021)<sup>1</sup>

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- Prepare for Mangoral launch (planned Q4-2022 – H1-2023)<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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