



# ASCELIA PHARMA

ADVANCING  
ORPHAN ONCOLOGY

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[www.ascelia.com](http://www.ascelia.com)

## COMPANY PRESENTATION

JANUARY 2021

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# ASCELIA PHARMA – ADVANCING ORPHAN ONCOLOGY



IMPROVING THE LIFE OF PEOPLE  
WITH RARE ONCOLOGY-RELATED  
CONDITIONS

## CURRENT ASSETS

### Mangoral

- Contrast agent for liver MRI in subset of overall population (Orphan Drug)
- Global Phase 3 study (ongoing)

### Oncoral

- Novel daily chemotherapy tablet ready for Phase 2
- Focus on gastric cancer; potential in other cancer types

## CAPABILITIES

### People & Partners

- Strong experienced team
- World class network of global KOLs and advisors

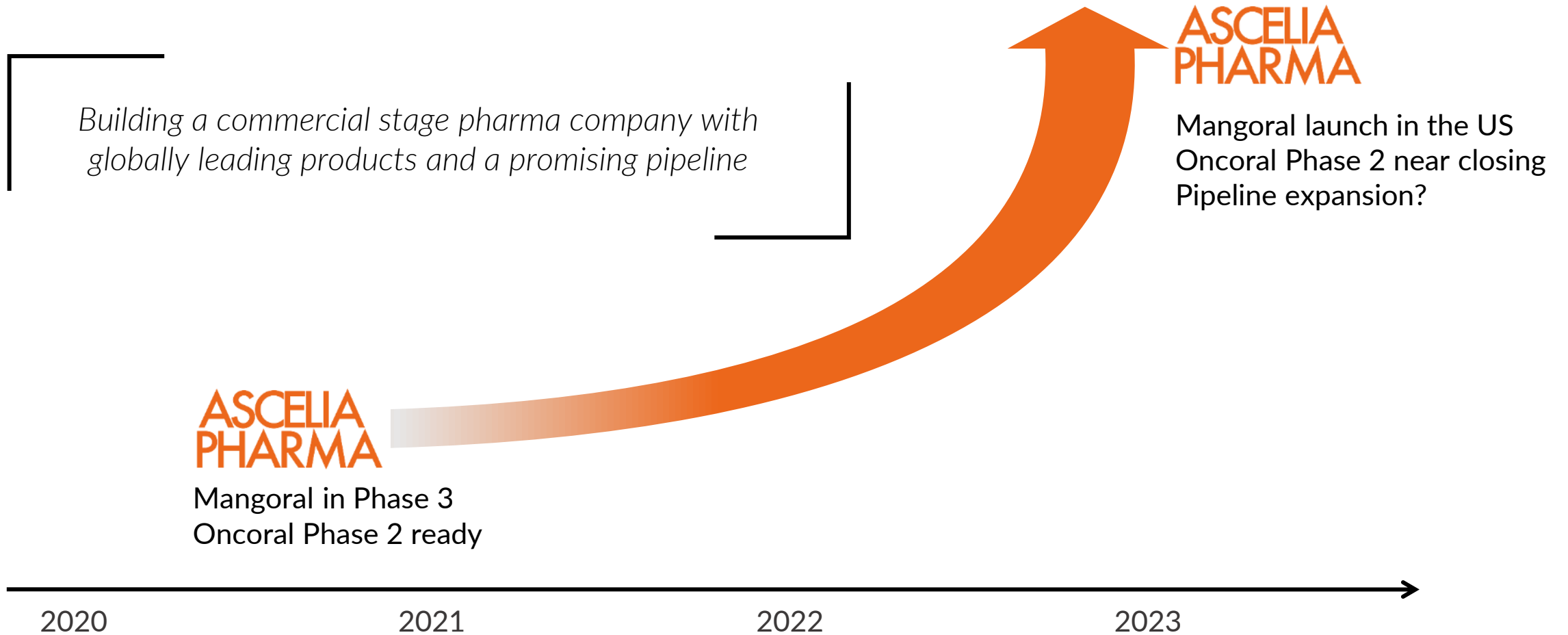
### Financial

- Fully financed to reach key value creating milestones
- Listed on NASDAQ Stockholm

# A CLINICAL STAGE PORTFOLIO WITH CLEAR MARKET PATHWAY

Drug candidate	Indication	Phase 1	Phase 2	Phase 3	Filing	Market
<b>Mangoral</b> <ul style="list-style-type: none"><li>• Novel manganese-based imaging drug</li><li>• Orphan Drug Designation (FDA)</li><li>• No competing drugs</li><li>• \$500-600M addressable market</li></ul>	Detection and visualization of focal liver lesions	✓	✓	Ongoing 2020 – H2 2021	H1 2022	Q4 2022-H1 2023
<b>Oncoral</b> <ul style="list-style-type: none"><li>• Novel daily chemotherapy (irinotecan)</li><li>• Promising phase 1 study results</li><li>• Gastric cancer orphan disease</li></ul>	Gastric cancer treatment; expansions to other solid cancers	✓	2H 2021-2024			

# BUILDING ASCELIA PHARMA AND BUILDING VALUE





# RECENT KEY EVENTS

## 2020

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- Sep** First commercial scale production of Mangoral
- Oct** Raised estimate for Mangoral addressable market
- Nov** Mangoral eligible for centralized EU regulatory procedure
- Dec** Mangoral is as effective as gadolinium (new study)
- Dec** US patent for second-generation Mangoral

## 2021

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- Jan** Presentation of clinical development plan for Oncoral





# PORTFOLIO

## **MANGORAL**

Liver MRI contrast agent in ongoing Phase 3

## **ONCORAL**

Daily chemotherapy ready for Phase 2

# CLEAR UNMET MEDICAL NEED

## TODAY

NORMAL KIDNEY FUNCTION  
Gadolinium contrast agent

POOR KIDNEY FUNCTION  
NO contrast agent  
(regulatory black box warnings )

Severe and potentially deadly side-effects  
(Nephrogenic Systemic Fibrosis)

## TOMORROW

NORMAL KIDNEY FUNCTION  
Gadolinium contrast agent

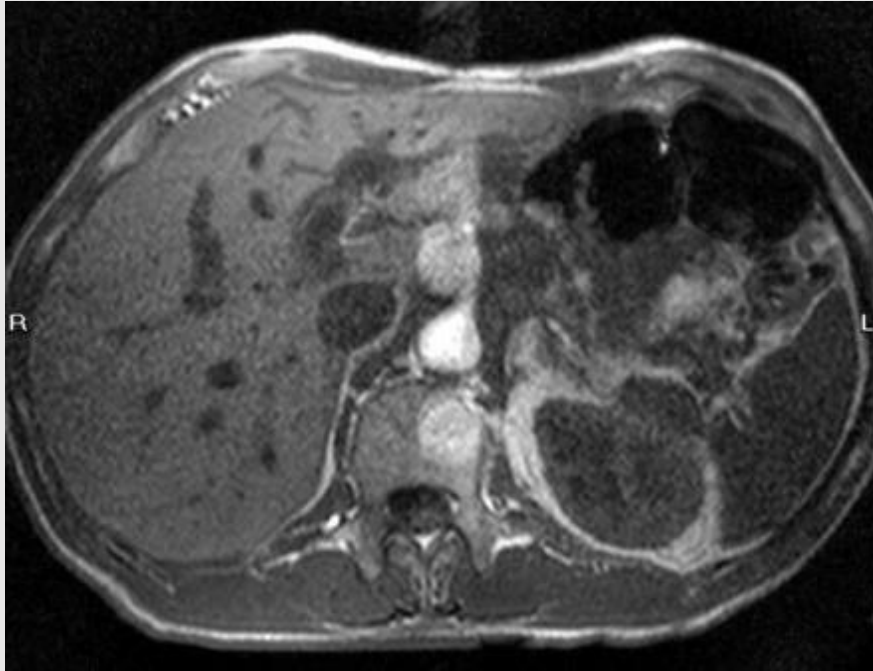
POOR KIDNEY FUNCTION  
**MANGORAL** contrast agent  
(based on manganese)

Mangoral aims to be the standard of  
care liver MRI contrast agent



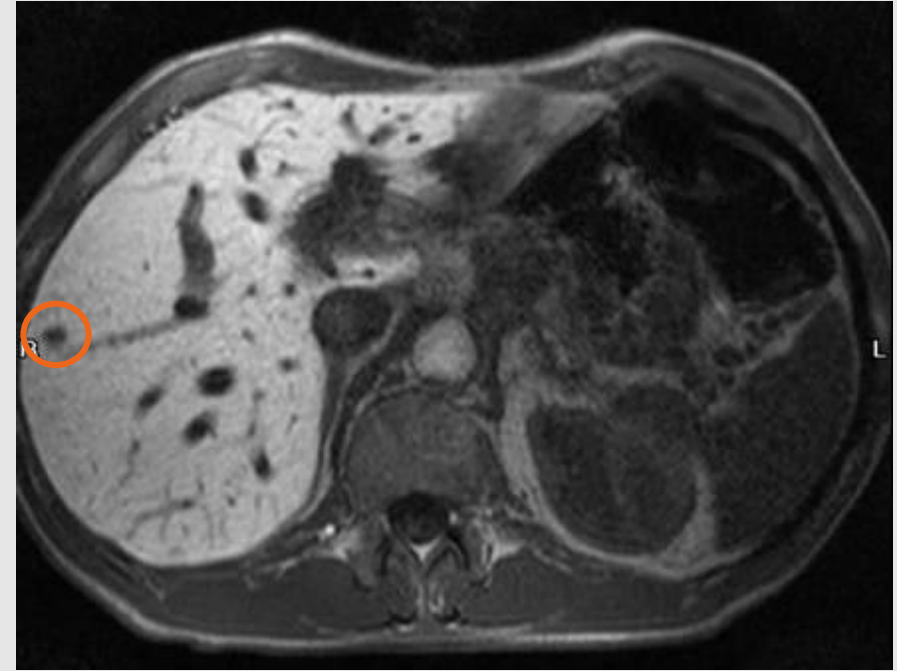
# SAFE AND EFFECTIVE FOR LIVER MRI

## PATIENT EXAMPLE FROM PHASE 2 STUDY



**UNENHANCED** liver MRI

Standard of care today in target population



**MANGORAL** enhanced liver MRI

Liver metastasis appear with Mangoral

# ONGOING PHASE 3 STUDY SPARKLE

## PHASE 1 AND PHASE 2 RESULTS

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  
(20 persons)

- Mangoral as effective as gadolinium

## 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

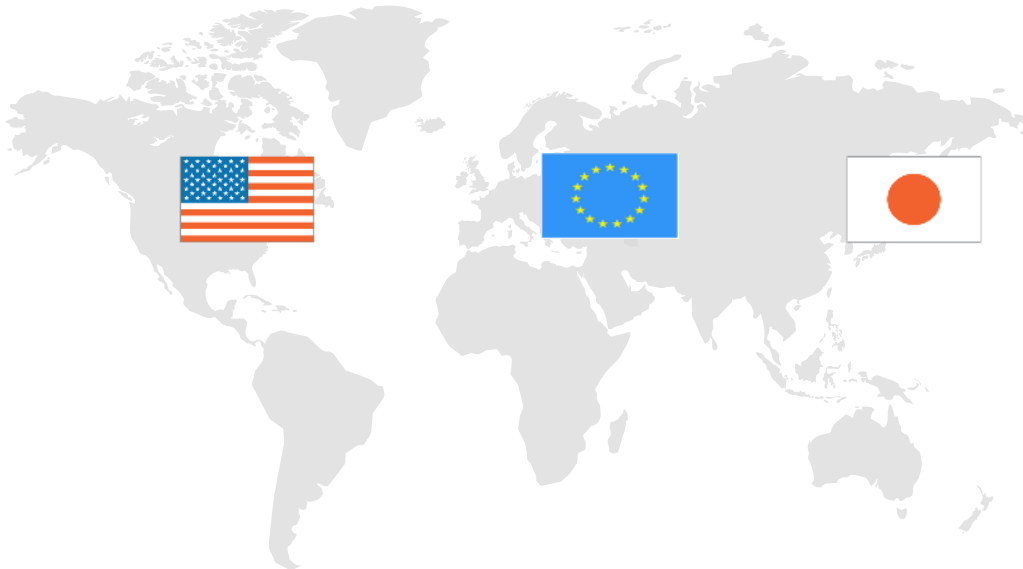


Less than a week

# ADDRESSABLE MARKET OF \$500-600 MILLION

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

- Large markets with mature clinical practices
- Clear regulatory and market access pathway



### ESTIMATE BASED ON:

- Patients with primary liver cancer or liver metastases and severe kidney impairment (4% of total population)
- 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%

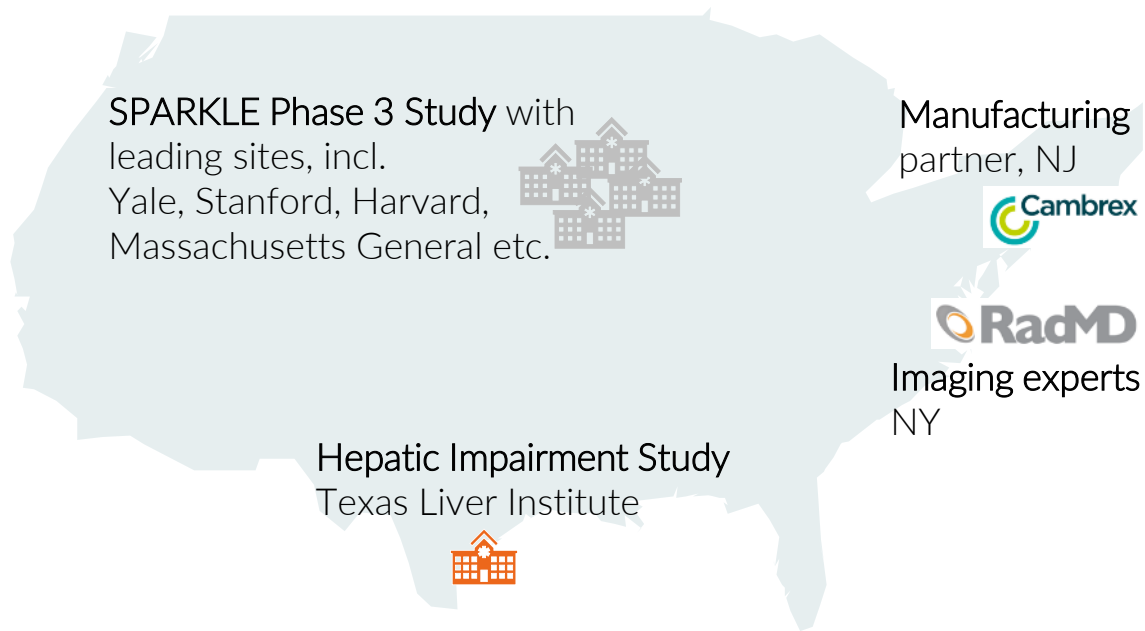
Sources:

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 OWN TEAM

## STRONG FOOTPRINT IN THE US



## BUILDING AN ASCELIA US TEAM

Sales team

~20 FTEs reach priority accounts & physicians

Clinic/  
Hospitals

Reachable hospitals/clinics

~400 serve ~75% of kidney impairment patients<sup>1</sup>

Physicians

Radiologists main clinical decision makers

~2,000 radiologists perform regular liver MRIs in patients with kidney impairment<sup>1</sup>





# PORTFOLIO

## MANGORAL

Liver MRI contrast agent in ongoing Phase 3

## ONCORAL

Oral, daily chemotherapy ready for Phase 2

# ONCORAL – IRINOTECAN AS TABLET

## INTRAVENOUS BOLUS INFUSION

TODAY



### IRINOTECAN IV

- Widely used chemotherapy
- Established potent anti-tumor effect

## ONCORAL – ORAL, DAILY DOSING

TOMORROW



### IRINOTECAN TABLET

Solid composition  
Enteric coating

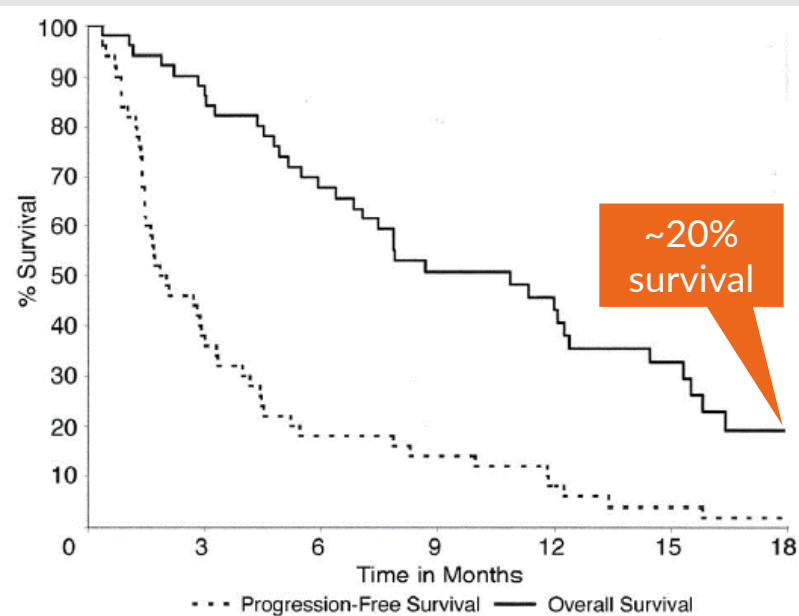
### ONCORAL IRINOTECAN POTENTIAL

- Better efficacy
- Improved safety
- Convenient and cost effective

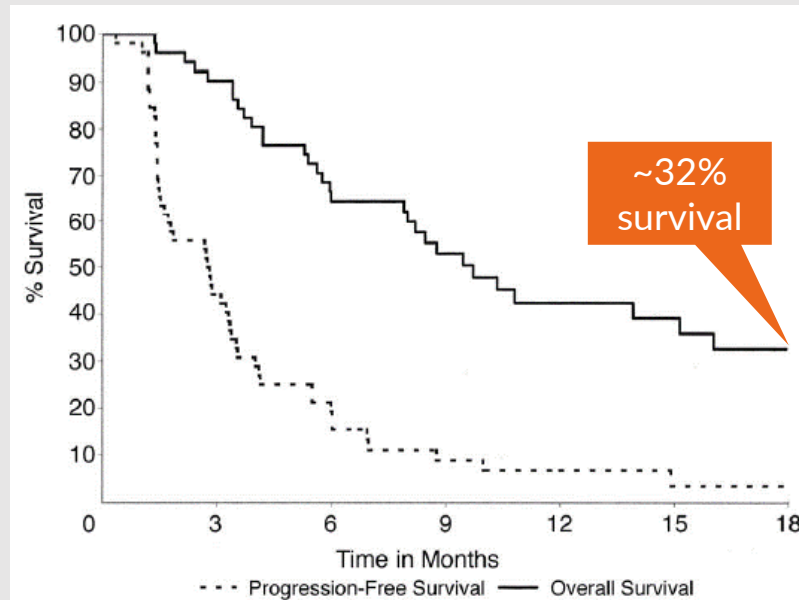
# IMPROVED OVERALL SURVIVAL WITH FREQUENT DOSING

## STUDY: IRINOTECAN DOSING EVERY THIRD WEEK VS. WEEKLY DOSING<sup>1</sup>

Every 3 weeks (IV)



Weekly (IV)



### CONCLUSIONS

Overall survival improved from 20% with irinotecan dosing every 3 weeks to 32% with weekly dosing

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

### ONCORAL PHASE 1

Stable disease even in patients previously treated with IV irinotecan





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 - STUDY IN PREPARATION

## OBJECTIVES OF PHASE 2

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

## 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



A man and a woman are standing in a grassy park. The man is holding a yellow bicycle with a wicker basket on the handlebars. They are both looking towards the right. The background is filled with trees and a bright, hazy sky. A semi-transparent white shape is overlaid on the left side of the image.

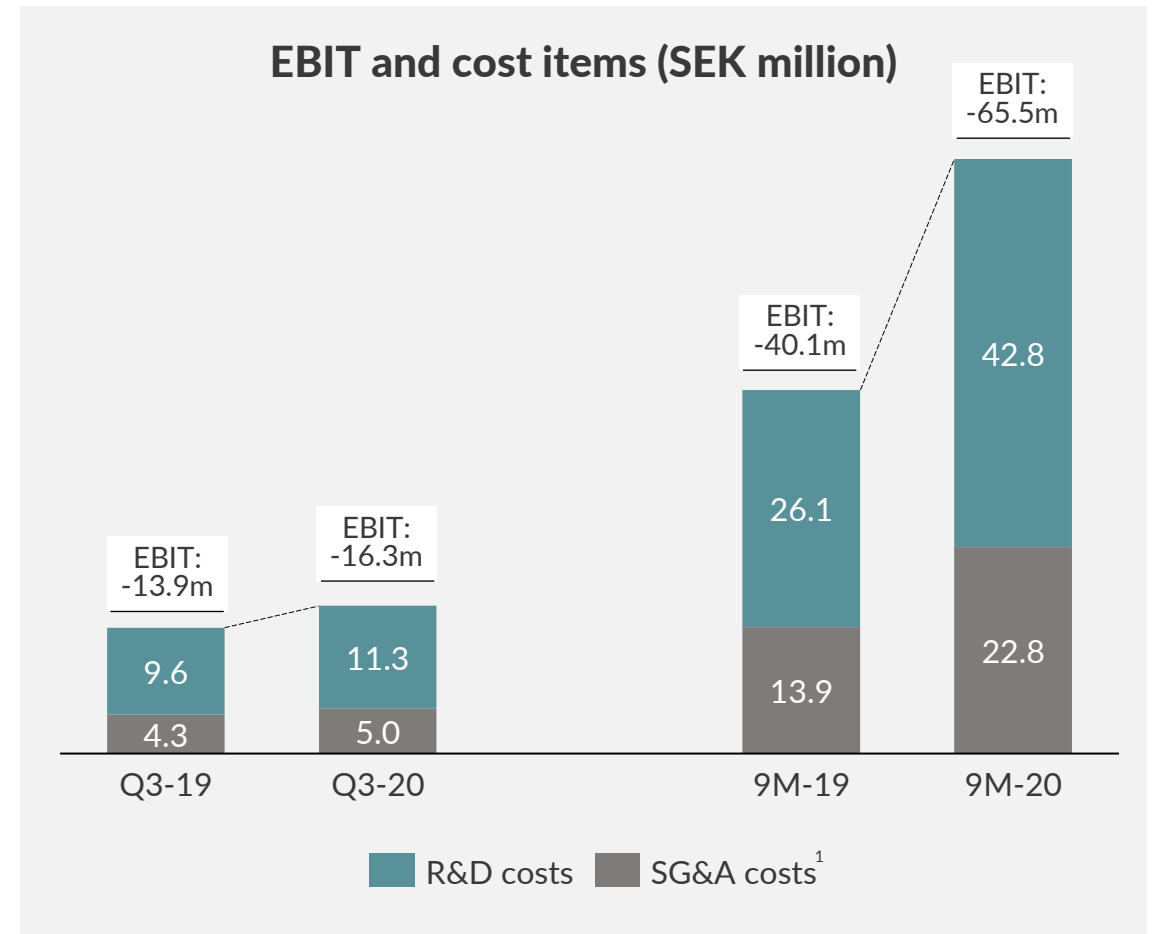
# FINANCIALS

# FINANCIAL HIGHLIGHTS Q3 2020 – 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

... And higher costs for commercial preparations for Mangoral (forming part of Selling, General & Administrative costs)



Notes:

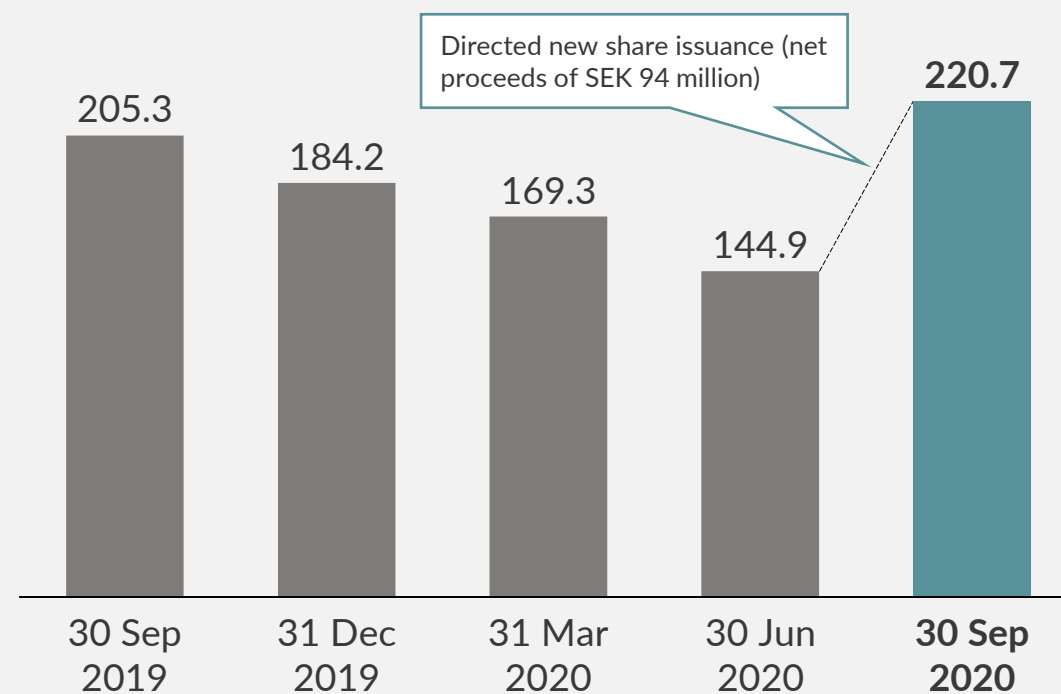
1) Other operating income and other operating costs included into SG&A

# FINANCIAL HIGHLIGHTS Q3 2020 – LIQUIDITY POSITION

## Solid liquidity position:

- Liquid assets incl. marketable securities of SEK 221 million per 30 September 2020
- Liquidity strengthened by the directed share issue (funds received in July 2020)
- Liquidity mainly to be used for Mangoral clinical Phase 3 and pre-commercial activities
- The liquidity position will take Ascelia Pharma into 2022 and consequently beyond the clinical milestone with topline Phase 3 from SPARKLE, which is expected in H2-2021

## Liquid assets including marketable securities (SEK million)



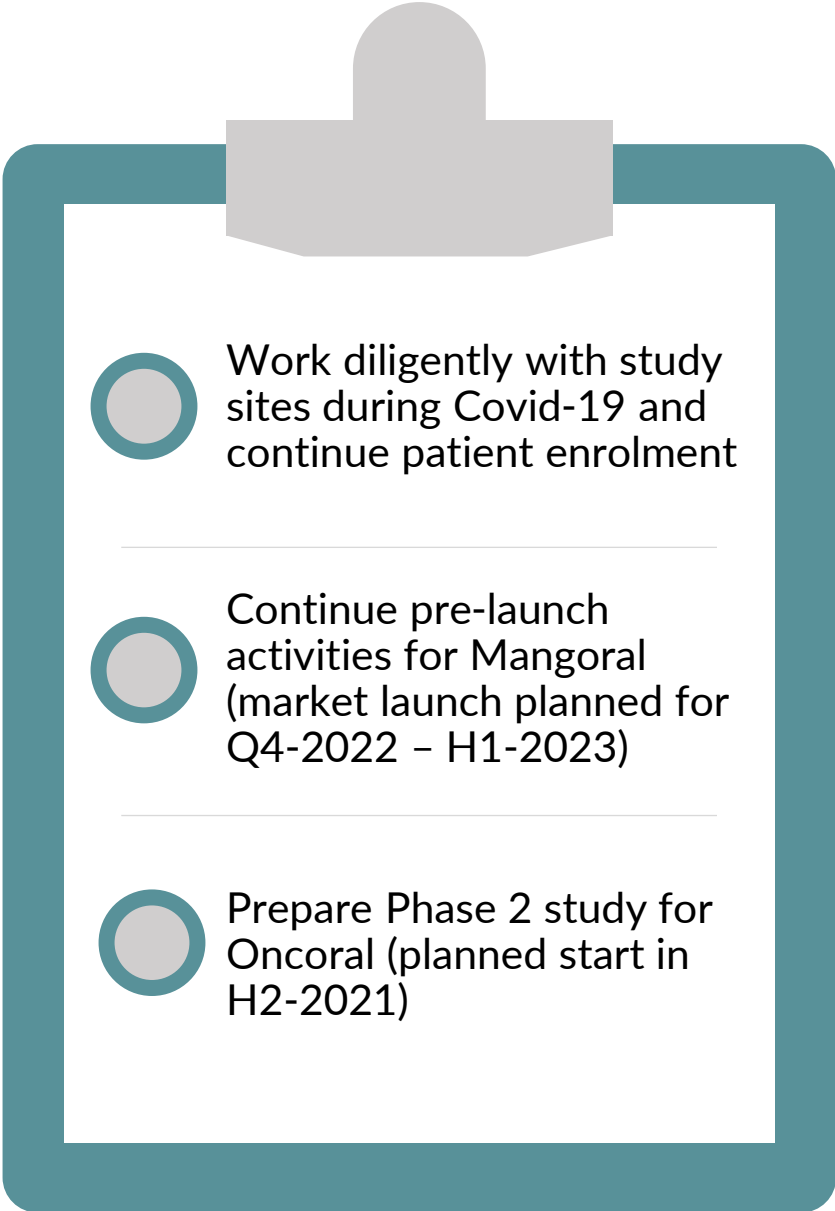





# **PRIORITIES AND SUMMARY**



# Priorities

- 
- Work diligently with study sites during Covid-19 and continue patient enrolment
  - Continue pre-launch activities for Mangoral (market launch planned for Q4-2022 – H1-2023)
  - Prepare Phase 2 study for Oncoral (planned start in H2-2021)

# ASCELIA PHARMA IN SUMMARY



## **Ascelia Pharma (ticker: ACE) – Advancing orphan oncology**

- Drugs with a clear development and market pathway
- Commercial stage capabilities underway
- Solid financial position

## **MANGORAL – Phase 3 non-gadolinium liver imaging drug**

- \$500-600 million annual addressable market with no competing drugs
- Orphan Drug Designation
- Ongoing Phase 3 – results expected H2-2021

## **ONCORAL – Phase 2 ready oral chemotherapy**

- Oral daily dosing of irinotecan chemotherapy
- Potential for better patient outcomes and safety
- Phase 2 in gastric cancer

# ASCELIA PHARMA

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# ASCELIA PHARMA – ADVANCING ORPHAN ONCOLOGY

IMPROVING THE LIFE OF PEOPLE WITH RARE ONCOLOGY-RELATED CONDITIONS

**ASCELIA PHARMA –**  
Develop and commercialize drugs with:

**MANGORAL –**  
Advancing liver imaging

**ONCORAL –**  
Advancing chemotherapy

Unmet medical need



Niche/orphan indication



Known mode of action



Clear development & market pathway



Potential for global leadership

