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# PRESENTATION OF YEAR-END REPORT JULY-DECEMBER 2019

*AUDIO CONFERENCE  
14 FEBRUARY 2020, AT 10:00 CET*

*Present from Ascelia Pharma:*

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

*Direct link audiocast:*

<https://tv.streamfabriken.com/ascelia-pharma-year-end-report-2019>

*Teleconference dial-in:*

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# ASCELIA PHARMA IN BRIEF



Orphan oncology-dedicated drug development company



We develop drugs which target unmet medical needs, have an established mode of action and a relatively low development risk



*Mangoral®*

Phase III novel liver MRI contrast agent with no competition



*Oncoral*

Phase II ready novel tablet chemotherapy for gastric cancer



Founded in 2000 and headquartered in Malmö, Sweden



Listed on Nasdaq Stockholm in 2019 (ticker: ACE)

# KEY EVENTS IN THE PERIOD

## Summary of key events in October-December 2019

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- Appointment of Julie Waras Brogren as Chief Commercial Officer
- Preparatory work for opening of study sites for Mangoral's Phase III study SPARKLE
- Four sites opened for patient enrolment in December in Mangoral Phase III study and one additional site opened in January 2020

# OUR PORTFOLIO AND UPCOMING KEY MILESTONES

Drug candidate	Indication	Phase I	Phase II	Phase III	Registration	Market launch
<b>Mangoral</b> <ul style="list-style-type: none"> <li>Novel non-gadolinium imaging drug</li> <li>Orphan Drug Designation</li> <li>No competing products</li> <li>\$350-500M market with upside potential</li> <li>Fully funded Phase III clinical program</li> </ul>	<b>Visualisation of focal liver lesions</b> <ul style="list-style-type: none"> <li>Liver metastases</li> <li>Primary liver cancer</li> <li>Benign lesions</li> </ul>	Completed	Completed	2020 - Full study report H1 2021	2021	2022
<b>Oncoral</b> <ul style="list-style-type: none"> <li>Novel tablet chemotherapy formulation</li> <li>Phase I clinical study completed with promising results</li> <li>Gastric cancer is an Orphan indication</li> </ul>	<b>Treatment of gastric cancer</b> Treatment of other solid cancers (label expansion)	Completed	2021 - 2023	The clinical development strategy is to partner after Phase II for the further development to market		

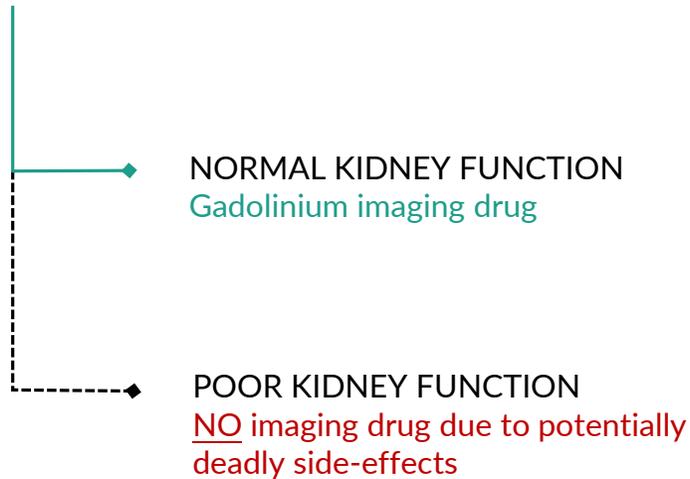


MANGORAL

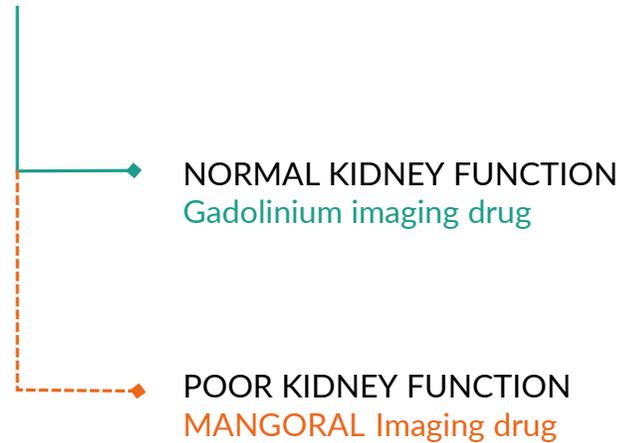


# PATIENTS REFERRED FOR LIVER MRI SCAN

## TODAY



## TOMORROW



**Mangoral** aims to be the new gold standard liver MRI imaging drug for patients with impaired kidney function



280,000

patients with impaired kidney function in major markets

**WARNING: NEPHROGENIC SYSTEMIC FIBROSIS (NSF)**  
See full prescribing information for complete boxed warning.  
Gadolinium-based contrast agents (GBCAs) increase the risk for NSF among patients with impaired elimination of the drugs. Avoid use of GBCAs in these patients unless the diagnostic information is essential and not available with non-contrasted MRI or other modalities.

- The risk for NSF appears highest among patients with:
  - Chronic, severe kidney disease (GFR < 30 mL/min/1.73m<sup>2</sup>), or
  - Acute kidney injury.
- Screen patients for acute kidney injury and other conditions that may reduce renal function.
- For patients at risk for chronically reduced renal function (for example, age >60 years, hypertension or diabetes), estimate the glomerular filtration rate (GFR) through laboratory testing (5.1).

# MANGORAL HAS STRONG DATA AND A CLEAR PATH TO MARKET

## Strong data package for Mangoral

6 phase I and II clinical studies completed

Consistent strong efficacy readout and safety profile

Blind read study of all imaging data presented at major conferences

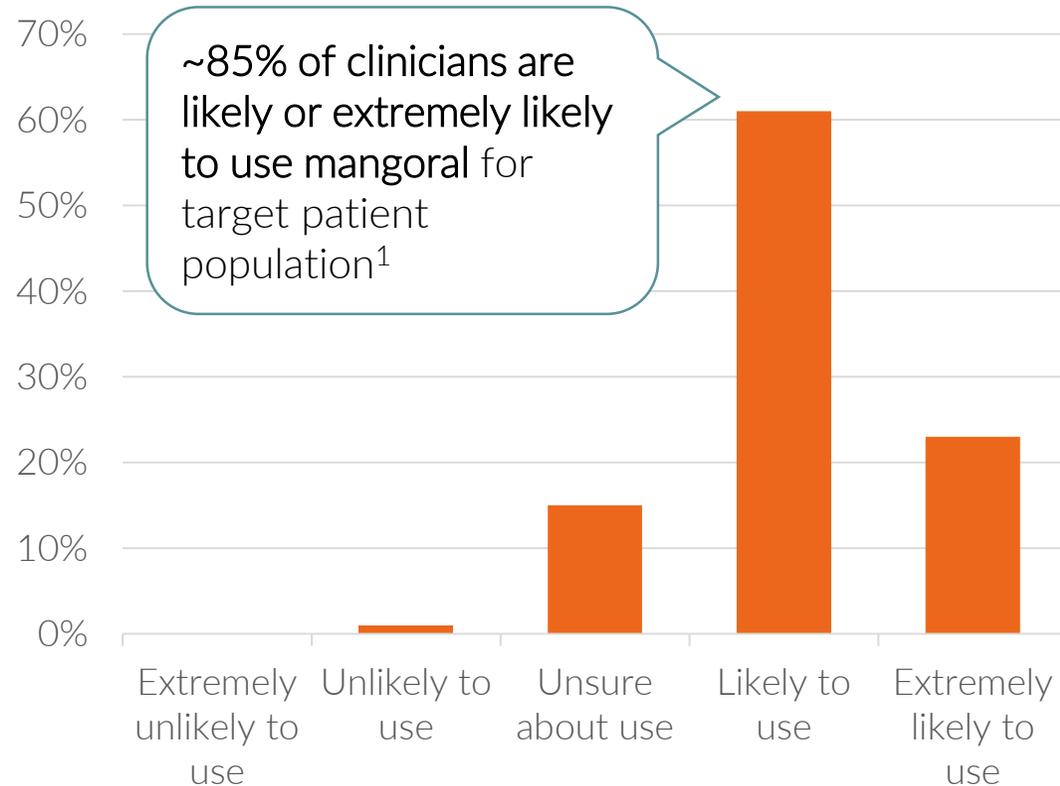
- The study with 178 subjects further underlined that Mangoral significantly improves MRI performance
- 33% more lesions were detected after Mangoral enhanced MRI
- Mangoral significantly improved lesion visualisation (conspicuity; p-value <0.0001) and delineation (p-value <0.0001)

## Phase III registration-enabling study

Number of patients	Global study in up to 200 patients
Endpoint 	<b>Lesion visualisation</b> <ul style="list-style-type: none"><li>• Lesion border delineation</li><li>• Conspicuity (lesion contrast compared to liver background)</li></ul>
Comparator 	Unenhanced MRI + Mangoral MRI vs. Unenhanced MRI
Follow-up 	72 hours
Randomisation 	<b>No</b> - each patient at his/her own control
Validation 	Phase III program has been discussed with FDA and EMA

# CLEAR DEMAND CONFIRMS USD 350-500M MARKET POTENTIAL

## Unmet need confirmed



## Payer and clinician value proposition

**The only liver MRI contrast agent** for patients with severe renal impairment or acute kidney failure<sup>2</sup> (~280,000 patients in major markets)

**Improved visualisation** of focal liver lesions (incl. metastases) compared to unenhanced MRI (+33% more lesions in phase 1&2 studies)

**Early detection** of focal lesions and metastases allows early intervention and higher survival rate (94% of clinicians confirm<sup>3</sup>)

Source: Market research by Back Bay Life Science Advisors with interview of 84 radiologists across the US regarding clinical practices in liver MRI scanning, the use of gadolinium and mangoral product profile. Notes: 1) Survey answers to question: 'What is your overall opinion of this product for its target population of patients with known or suspected liver metastases and severe renal insufficiency or acute kidney injury?' 2) Based on regulatory drug class warning on use of gadolinium-based contrast agents in patients with renal impairment (an eGFR <30 ml/min/1.73 m<sup>2</sup>) or acute kidney failure. 3) Survey answers to 'Using contrast MRI is important for early intervention, to detect small lesions, which if removed can be curative e.g. colorectal cancer metastases?'

# COMMERCIAL PREPARATIONS FOR A 2022 LAUNCH UNDERWAY

## Key success factors

Obtain **ACCESS** and reimbursement at optimal **PRICE**



Prepare **SALES** and **MARKETING** for early adoption



Mobilise **CAPABILITIES** and **RESOURCES** for success



## Achievements

- ✓ No competition supported by regulatory drug class warning for gadolinium-based agents
- ✓ Orphan drug payer pathways
- ✓ Clear unmet needs and clinical benefits
- ✓ Well defined target patient and HCP population
- ✓ Chief Commercial Officer joining in Q1 2020
- ✓ Strong commercial case for launching with own field force in the US

## 2020 focus areas

- ▶ Expand pricing & market access strategy
- ▶ Map key markets and key payers to define first and most attractive pathways
- ▶ Continue early payer discussions
- ▶ Continue pre-launch dialogue with KOLs
- ▶ Build prioritised roll-out plan and projections for a 2022 launch
- ▶ Develop commercialisation team according to milestones
- ▶ Design and mobilise timely US operations



ONCORAL

# ONCORAL – A NOVEL IRINOTECAN TABLET FOR ANTI-CANCER TREATMENT

## NOVEL ORAL FORMULATION

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Formulated as a **tablet** for convenient dosing and health-economic benefits



**Promising safety potential** of oral administration



Potential for **all-tablet chemo-combination**

## PHARMACEUTICAL INGREDIENT HAS PROVEN EFFECT

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Irinotecan shown to be effective in **killing cancer cells**



Expected to be efficacious and safe **together** with other well-recognized anti-cancer drugs



**Orphan drug indication** for gastric cancer by the FDA and EMA

# ONCORAL – PREPARATIONS FOR PHASE II

## Promising Phase I results (published in 2019)

- [Single agent study](#) and [combination study](#) completed with promising results
- Results from **single agent study** showed that:
  - Oncoral was well tolerated; side effects were generally mild to moderate, manageable and similar in type to those observed with intravenous irinotecan
  - Pharmacokinetic (PK) data showed consistent daily exposure and the active metabolite, SN-38, interpatient variability was in the same range as after infusion of irinotecan
- Result from **combination study** showed:
  - Reassuring tolerability of Oncoral together with another oral chemotherapy, capecitabine
  - The combination with capecitabine could become a more convenient and patient friendly treatment option compared to the intravenous formulations compounds

## Preparations for Phase II

Current work comprise developing the:

- Positioning of Oncoral for the treatment patients with gastric cancer
- Clinical development strategy
- Study design

A scientist in a white lab coat and blue gloves is using a pipette to transfer liquid into a small vial. The background shows various laboratory glassware, including a flask with yellow liquid and a flask with blue liquid. The scene is lit with a cool blue light.

# FINANCIALS

# FINANCIAL HIGHLIGHTS – OPERATING RESULTS

Increased operating loss y/y driven by higher R&D activity for Mangoral Phase III preparations:

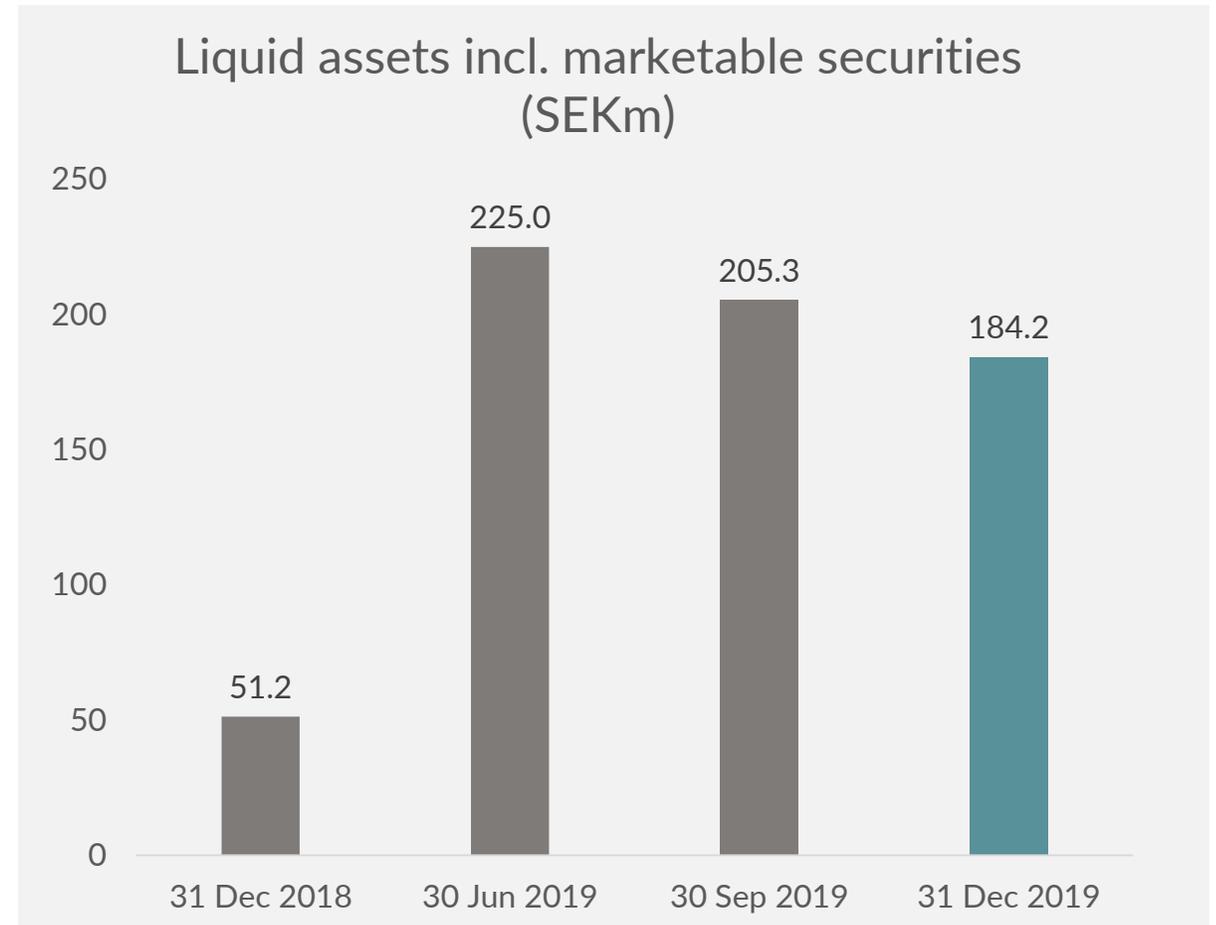
- Preparing and opening of clinical study sites
- Upscale of manufacturing
- Regulatory work



# FINANCIAL HIGHLIGHTS – LIQUIDITY POSITION

## Continued strong liquidity:

- Liquid assets incl. marketable securities of SEK 184.2 million
- The liquidity position provides a fully financed Phase III program for Mangoral including some commercial preparations as well as financing to prepare the Phase II program for Oncoral



# ASCELIA PHARMA

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