



Q3 REPORT (JAN-MAR 2019)

FISCAL YEAR 2018/2019

AUDIO CONFERENCE 15 MAY 2019, AT 10:00 CET

Present from Ascelia Pharma:

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Direct link audiocast:

<https://tv.streamfabriken.com/ascelia-pharma-q3-2018-2019>

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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 ready 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 Q3

Listed on Nasdaq Stockholm main market in March (ticker: ACE)

- Amount raised in IPO: SEK 200 million
- Substantially oversubscribed: strong interest among both institutional and private investors
- More than 6,000 new shareholders in the IPO
- Fully financed Phase III program for Mangoral

KEY EVENTS AFTER THE PERIOD

Summary of key events after Q3



Mangoral®

Supportive feedback from EMA on the phase III program for Mangoral





Oncoral

Encouraging results from the Phase I combination study with Oncoral and oral capecitabine published in the journal Cancer Chemotherapy and Pharmacology



Raised SEK 22 million in utilised IPO overallotment

SELECTED UPCOMING KEY EVENTS IN 2019 AND 2020

	H2-2019	2020
 Mangoral®	Phase III: First Patent First Visit	Phase III: Last Patent Last Visit (H2-2020) Final study results (H2-2020 / early 2021)
 Oncoral	Phase II preparations	Phase II study

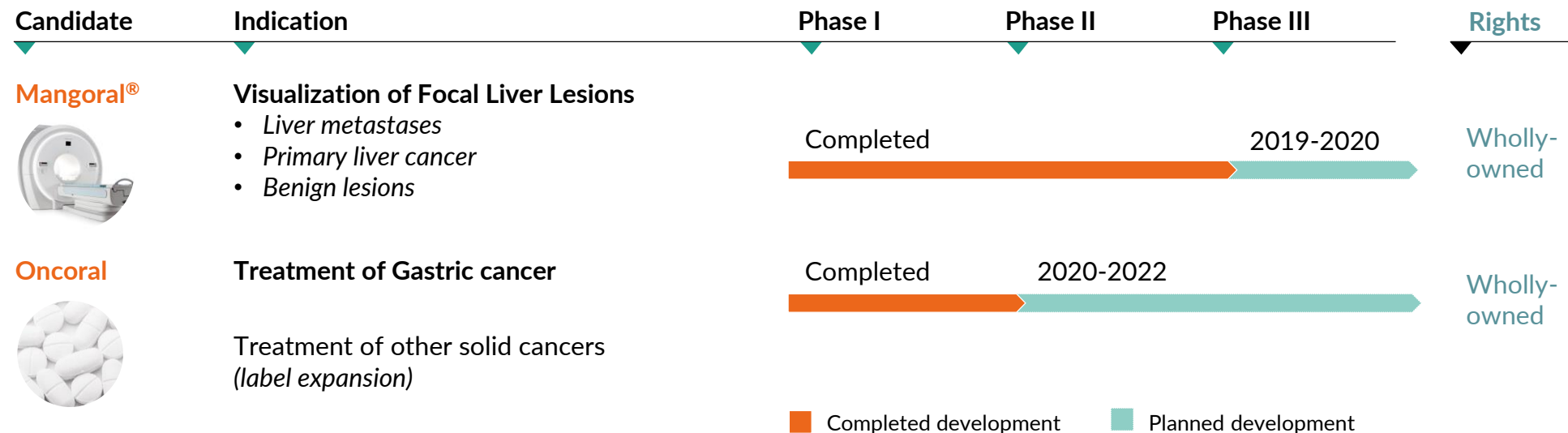
ASCELIA PHARMA HIGHLIGHTS AND PIPELINE

Mangoral®

- Novel imaging drug with Orphan Drug Designation (FDA)
- No competing products
- \$350-500M market with substantial upside potential
- De-risked Phase 3 clinical program starting in 2019

Oncoral

- Novel tablet chemo formulation
- Gastric cancer is an Orphan indication
- Phase I clinical study completed
- Recent acquisition of comparable product >\$1 billion





MANGORAL®

LIVER METASTASES – A MAJOR CHALLENGE IN ONCOLOGY

- 70% of patients with colon cancer will develop liver metastases¹
- Liver metastases are also common in other cancer types such as lung cancer, gastric cancer, metastatic breast cancer^{2,3} etc.
- The liver is the most frequent organ for metastases after lymph node³ and often the first site of metastasis
- Often liver metastases are the cause of death (not primary tumor)⁴



1) Riihimäki, M. et al. Patterns of metastasis in colon and rectal cancer. *Sci. Rep.* 6, 29765; doi: 10.1038/srep29765 (2016); *Journal of Pathology*, 2014, 232:23-31

2) *Oncotarget*, 2016, 7(32):52307; *Lung Cancer*, 2014, 86:78-84 (6):29765

3) Guy diSibio and Samuel W. French (2008) Metastatic Patterns of Cancers: Results From a Large Autopsy Study. *Archives of Pathology & Laboratory Medicine*: June 2008, Vol. 132, No. 6, pp. 931-939

4) Rahbari et al. Metastatic Spread Emerging From Liver Metastases of Colorectal Cancer: Does the Seed Leave the Soil Again? *Annals of Surgery*; February 2016 - Volume 263 - Issue 2 - p 345-352

LIVER METASTASES: HOW TO FIND AND WHAT TO DO

DETECT AND LOCALISE

Liver MRI is the **most sensitive** method for detection of liver metastases¹⁾

Gadolinium based imaging drugs are given to maximize accuracy of liver metastasis detection in MRI



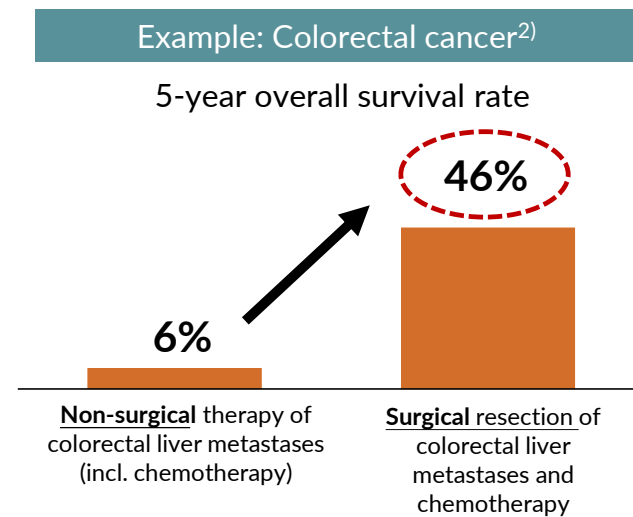
TREAT

Treatment options for liver metastases are:

- Surgical resection (only if detected early)
- Localised therapies (ablation embolization, radiation)
- Drug therapy

IMPROVE SURVIVAL

Accurate, early detection of liver metastases significantly impact treatment decisions and patient survival



1) Albiin N et al. Manganese chloride tetrahydrate (CMC-001) enhanced liver MRI: evaluation of efficacy and safety in healthy volunteers. MAGMA. 2012 Mar 8
2) Clinical Colorectal Cancer, Vol. 15, No. 4, Dec 2016, e183-192

GADOLINIUM CAN CAUSE NEPHROGENIC SYSTEMIC FIBROSIS IN PATIENTS WITH POORLY FUNCTIONING KIDNEYS



FDA



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH



No alternative MRI imaging drug for cancer patients with severely impaired kidneys

WARNING: NEPHROGENIC SYSTEMIC FIBROSIS (NSF)

See full prescribing information for complete boxed warning.

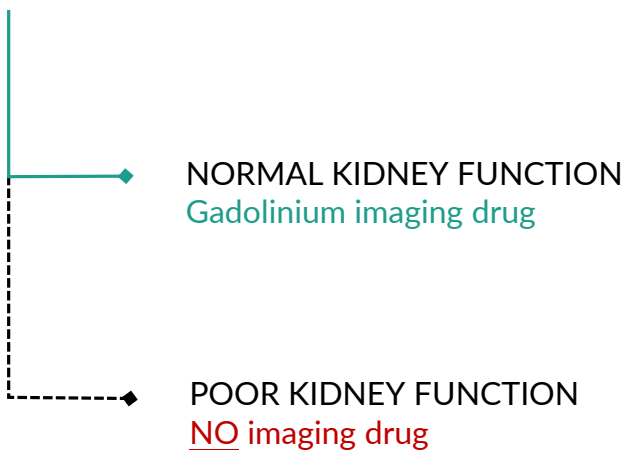
Gadolinium-based contrast agents (GBCAs) increase the risk for NSF among patients with impaired elimination of the drugs. Avoid use of GBCAs in these patients unless the diagnostic information is essential and not available with non-contrasted MRI or other modalities.

- The risk for NSF appears highest among patients with:
 - Chronic, severe kidney disease (GFR < 30 mL/min/1.73m²), 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](#)).

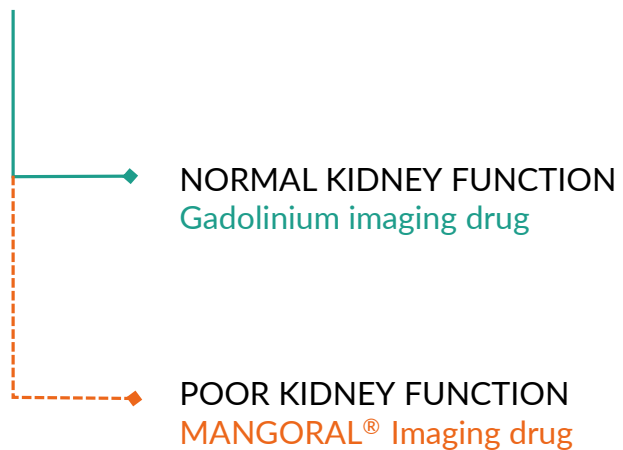


PATIENTS REFERRED FOR LIVER MRI SCAN

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

MANGORAL® STRONG CLINICAL RESULTS AND KEY BENEFITS

MANGORAL PROFILE AND KEY ADVANTAGES



Mangoral is based on manganese – a natural trace element in the body



FDA Orphan Drug Designation



Strong enhancement of liver on MRI – metastases do not take up manganese and appear darker on the MRI



No risk of Nephrogenic Systemic Fibrosis



Limited systemic exposure and good safety profile



No competing drug



Provides ease of use for patients and clinicians alike

STRONG CLINICAL RESULTS

Six Phase I and Phase II trials completed

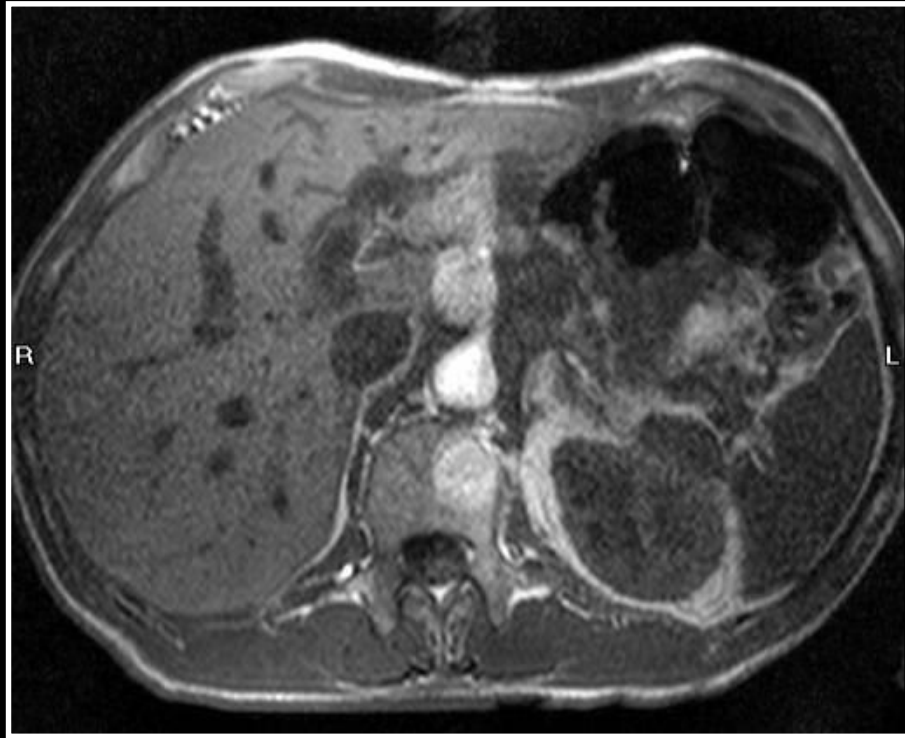
- The clinical trials have shown strong clinical efficacy without any safety concerns

Blinded read study

- The study with 178 subjects further underlined that Mangoral significantly improves MRI performance
- 33% more lesions were detected after Mangoral enhanced MRI
- Parameters incl. lesion visualization (conspicuity) and delineation had $p\text{-value} < 0.0001$ which demonstrates significant improvements in MRI imaging

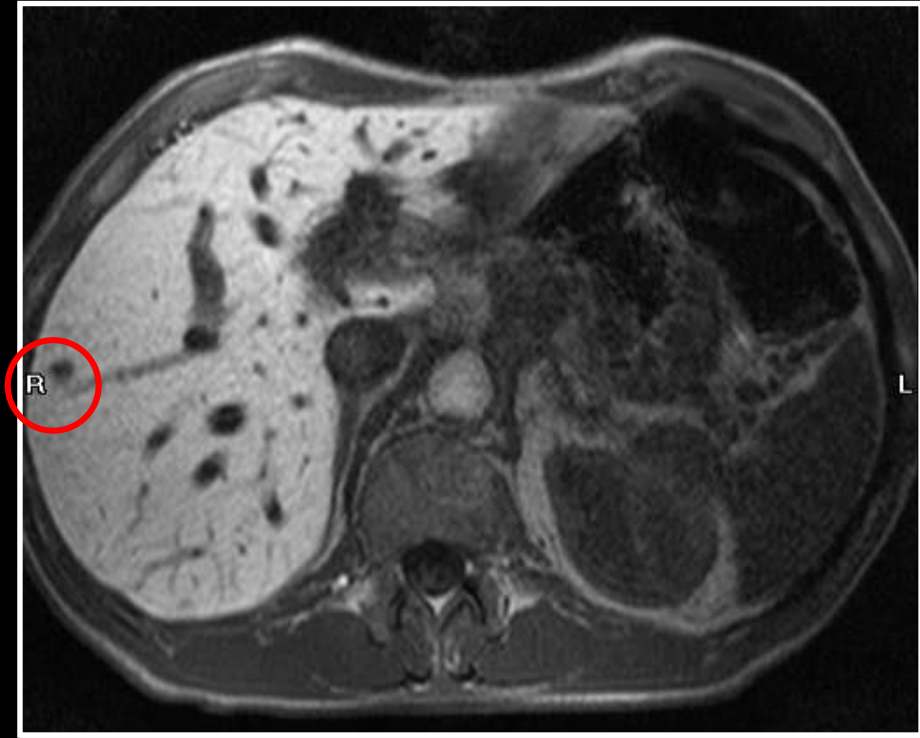
MANGORAL[®] MAKES A REAL DIFFERENCE

PATIENT EXAMPLE FROM PHASE II STUDY



Unenhanced liver MRI

(standard of care today in target patient population)








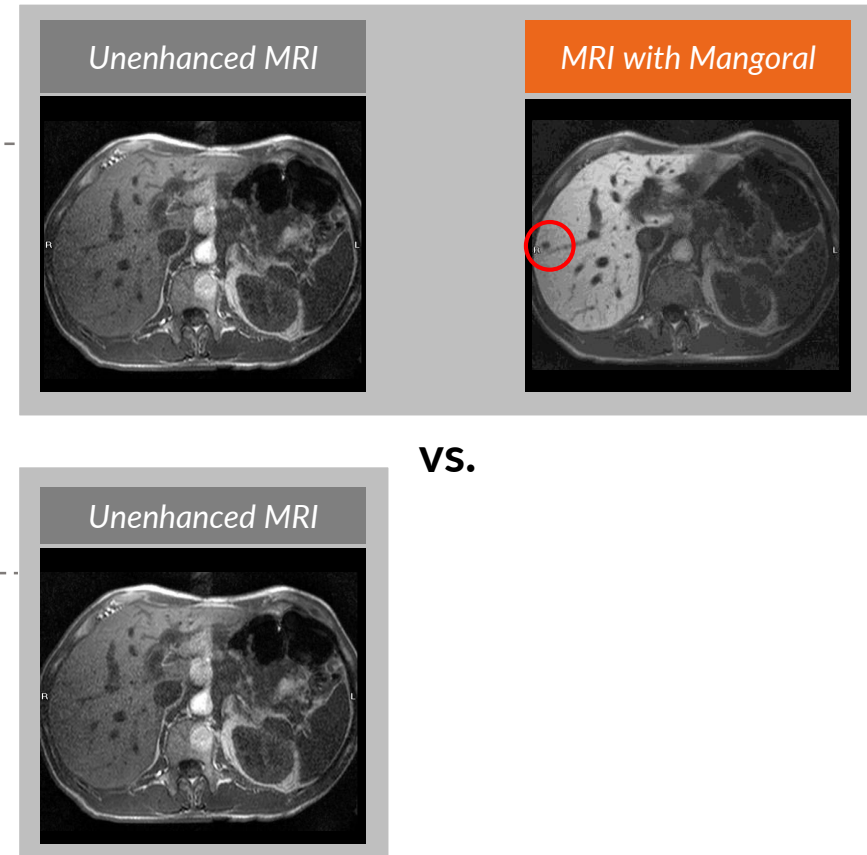
Mangoral enhanced liver MRI

A liver metastasis now appear

MANGORAL® PHASE III CLINICAL STUDY DESIGN IMPLIES INCREASED LIKELIHOOD OF SUCCESS

Mangoral clinical phase III study design – based on Phase III protocol meeting with FDA and EMA

Number of patients	Up to 200 patients
Endpoint 	Lesion visualisation <ul style="list-style-type: none">• No. of lesions visualised• Semi-quantitative and Semi-qualitative parameters
Comparator 	Unenhanced MRI + Mangoral MRI vs. Unenhanced MRI
Evaluation 	Centralised evaluation by 3 radiologists
Randomisation 	No – each patient will be in both study arms
Follow-up 	72 hours



MANGORAL® IS THE ONLY PRODUCT IN A \$350-500M MARKET

Overview of Mangoral's addressable market

280,000 patients having risk of cancer in the liver and poor kidney function

Mangoral useful for diagnosis, monitoring and surveillance

\$1,500 - \$3,000 per dose of Mangoral based on Value-based-pricing

\$350-500 million addressable market for Mangoral

Source

Detailed epidemiology analysis by geography, age groups and primary disease

Use of liver MRI today and clinical guidelines

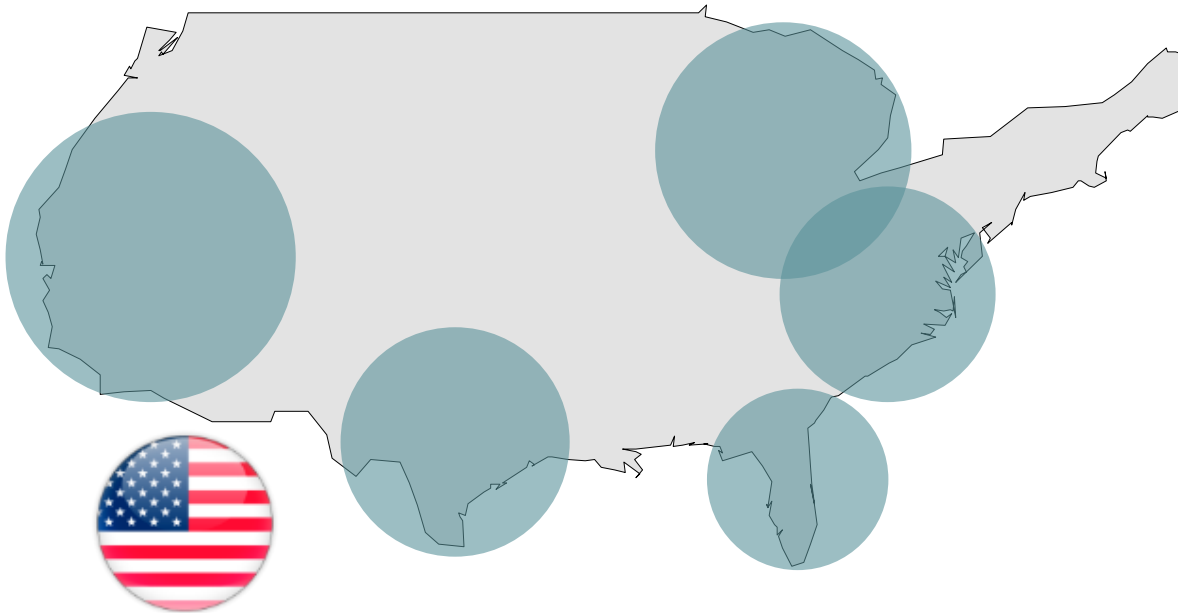
>25 interviews with payors/health insurers in US and EU and analysis of value provided by Mangoral

Note: 280,000 is estimated relevant patient population with cancer in USA, Europe and Japan

MANGORAL[®]

COMMERCIAL STRATEGY FOR A 2022 SALES LAUNCH

10-20 Sales Reps sufficient for penetration in concentrated regions



- Ascelia's sales force will target major hospitals with nephrology units
- 10-20 sales reps in the US sufficient for significant penetration
- Reimbursement expected shortly after sales launch
- Chief Commercial Officer will be recruited during the Phase 3 clinical study to finalize commercial strategy and prepare launch
- No recent innovation in the MRI space Mangoral has attracted major attention. This will be utilized in the pre marketing phase
- Ascelia sales force in Europe being evaluated
- Find commercial partners in Japan, South Korea and China



ONCORAL

ONCORAL – A NOVEL IRINOTECAN TABLET FOR ANTI-CANCER TREATMENT

NOVEL ORAL FORMULATION



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



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

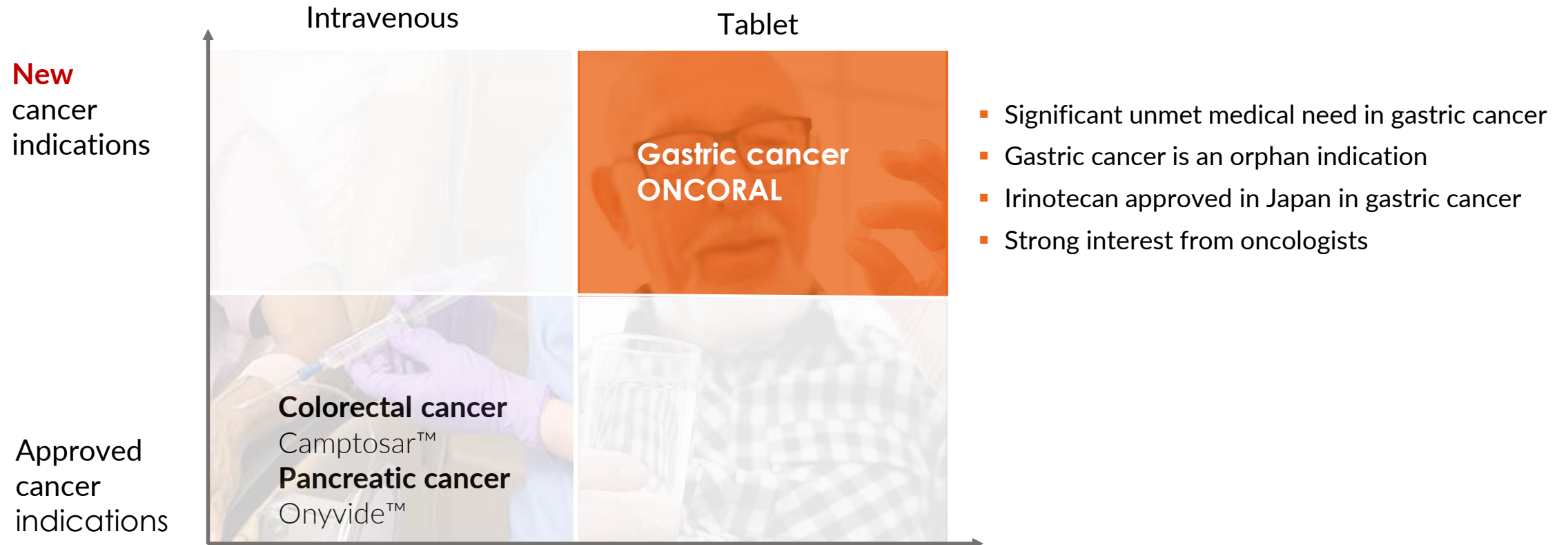


Expected to be efficacious and safe **together** with other well-recognized anti-cancer drugs



Orphan drug indication for gastric cancer by the FDA and EMA

ONCORAL HIGHLY DIFFERENTIATED FROM OTHER IRINOTECAN PRODUCTS



ENCOURAGING ONCORAL PHASE I STUDY RESULTS

Phase 1 [single agent study](#) published in Jan 2019

Results showed that Oncoral was well tolerated; side effects were generally mild to moderate, manageable and similar in type to those observed with intravenous irinotecan

Hematological toxicities were few and all were mild to moderate

Pharmaco-Kinetic (PK) data showed consistent daily exposures during treatment at days 1 and 14 with no drug accumulation

The active metabolite, SN-38, interpatient variability was in the same range as after infusion of irinotecan

In this heavily pre-treated patient population, Oncoral indicated activity even among patients previously treated with irinotecan infusion

The study was presented at ESMO congress in October 2018



Phase 1 [combination study](#) published in April 2019

The combination of Oncoral with another oral chemotherapy, capecitabine, was encouraging which could enable an all-oral chemotherapy combination

The study data demonstrated reassuring tolerability of Oncoral together with capecitabine

The combination with capecitabine could become a more convenient and patient friendly treatment option compared to the intravenous formulations of these compounds

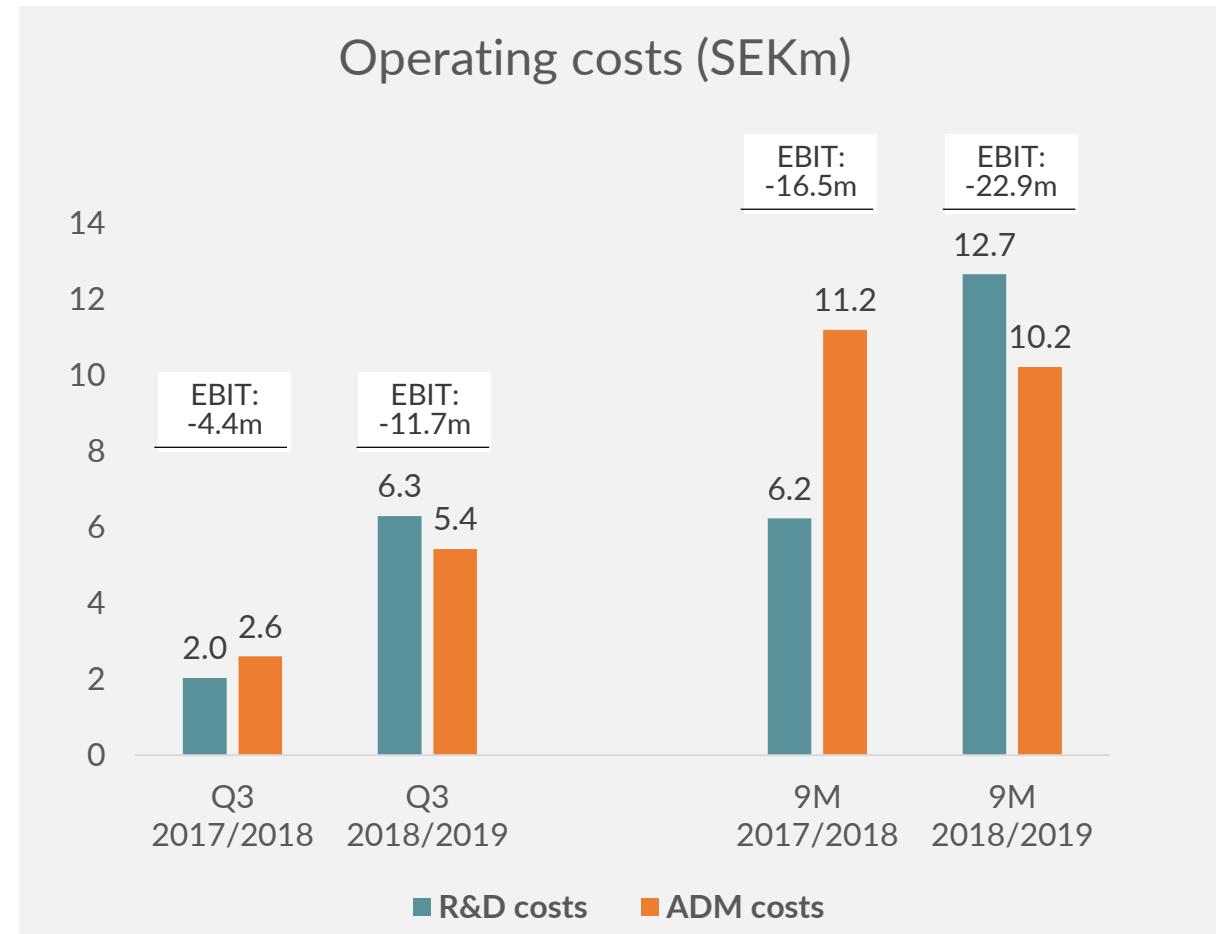
The encouraging tolerability profile justifies further clinical studies to assess the efficacy of this treatment regimen



FINANCIALS

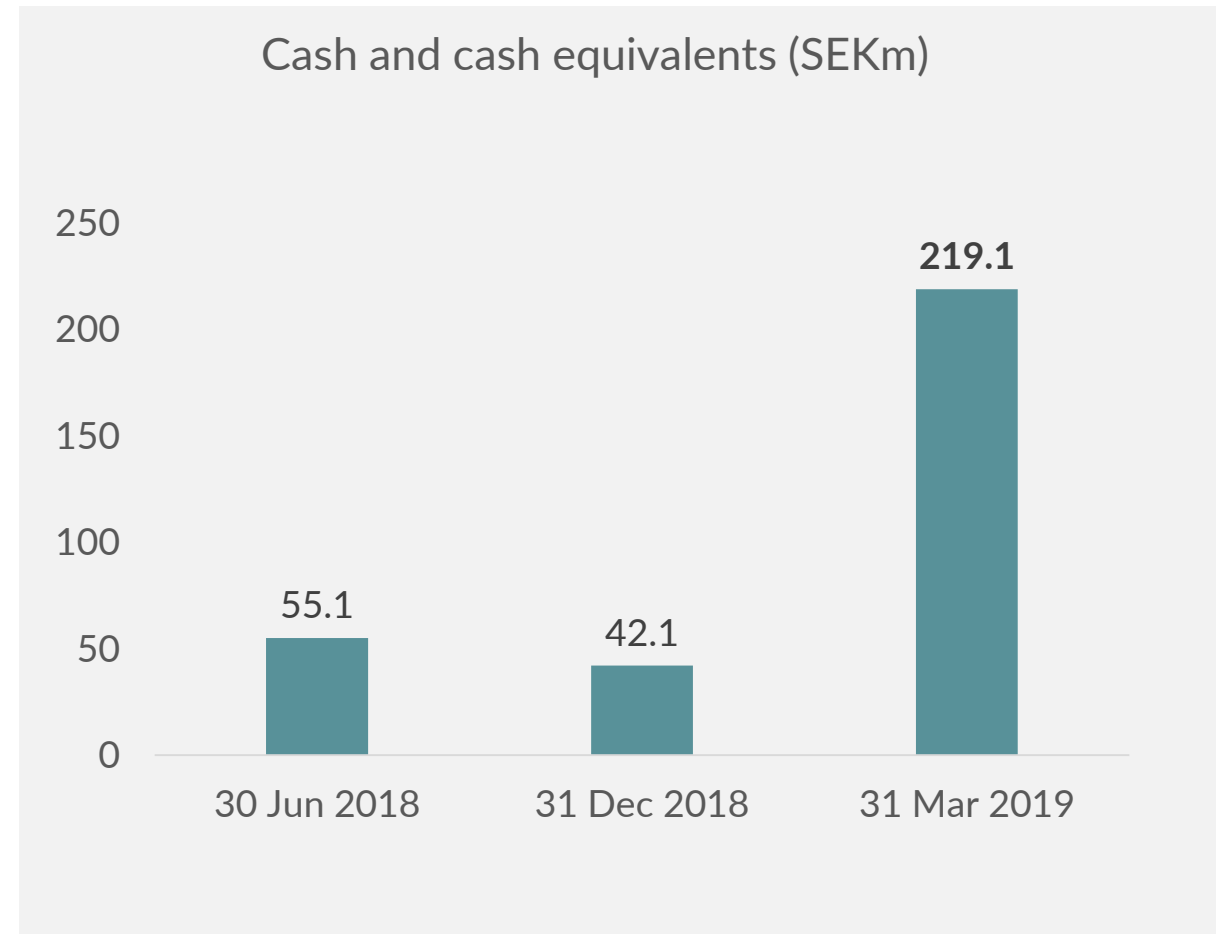
Q3 2018/2019 FINANCIAL HIGHLIGHTS – OPERATING RESULTS

- Increased operating loss y/y driven primarily by higher R&D activity for Mangoral Phase III
 - Protocol finalisation
 - Site selection
 - Upscale of manufacturing



Q3 2018/2019 FINANCIAL HIGHLIGHTS – LIQUIDITY POSITION

- **Strong liquidity following the IPO**
 - Liquidity position of SEK 219 million per 31 March 2019
 - Overallotment utilised in April 2019 raising additional SEK 22 million
 - The funds available provide a fully financed Phase III program for Mangoral including commercial preparations as well as financing to prepare the Phase II program for Oncoral

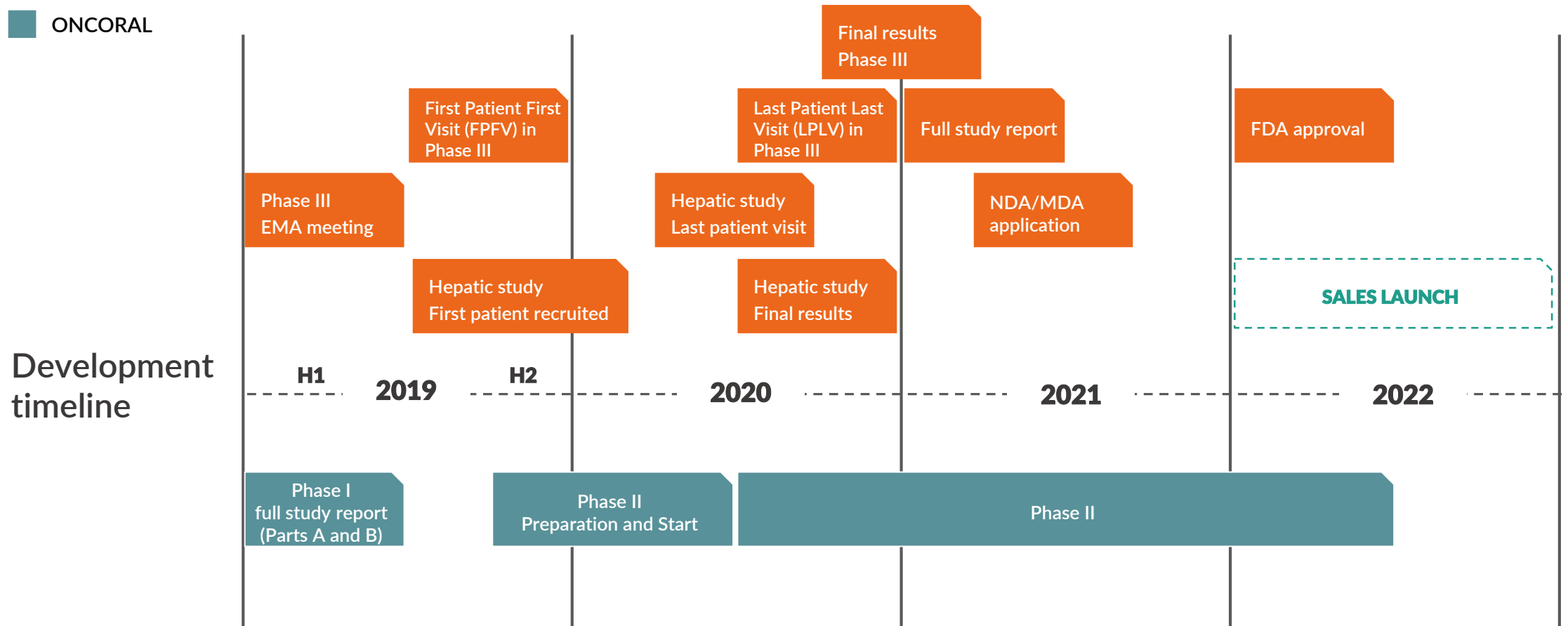




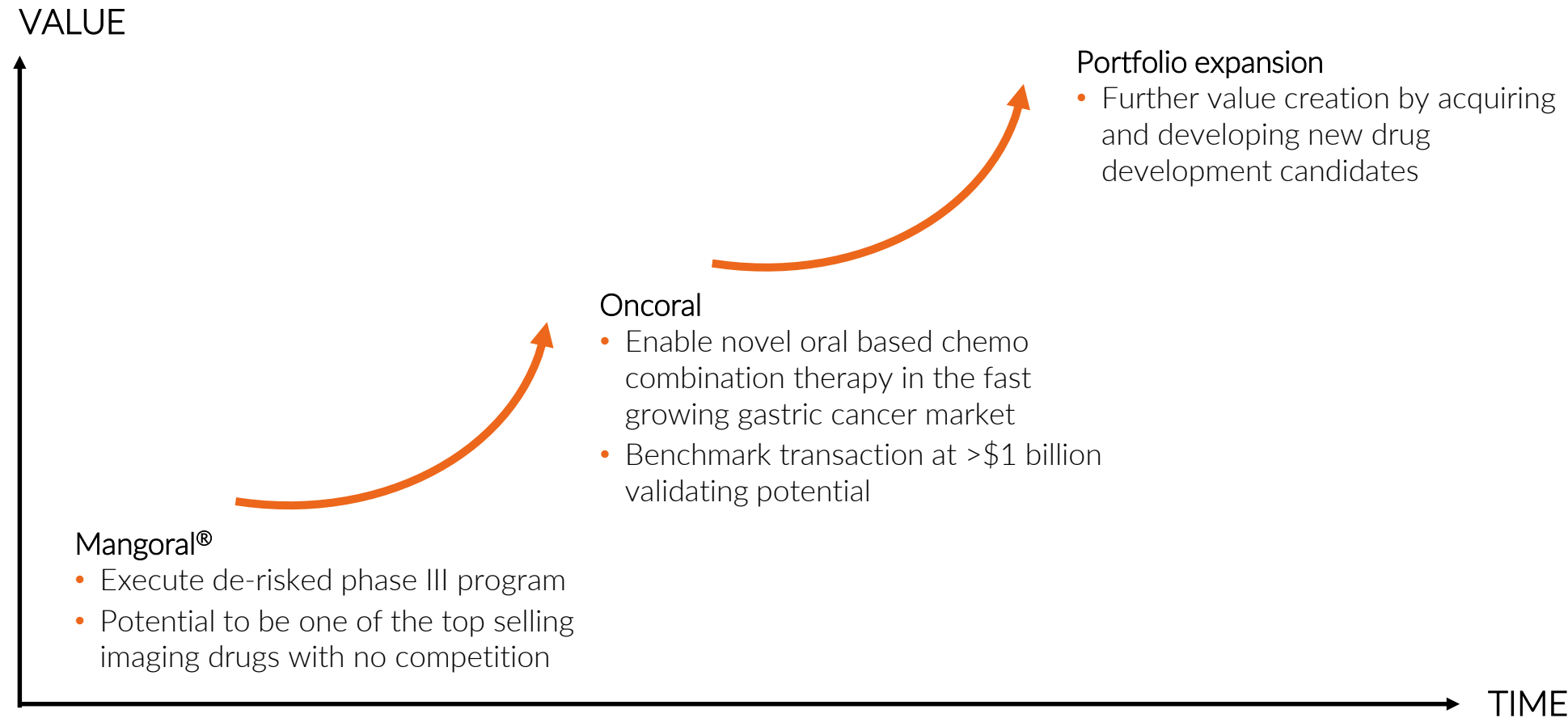
► MILESTONES AND STRATEGIC OUTLOOK

SIGNIFICANT VALUE DRIVERS AHEAD

 MANGORAL®
 ONCORAL



ASCELIA PHARMA STRATEGIC OUTLOOK



ASCELIA PHARMA

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