



ASCELIA PHARMA

ADVANCING
ORPHAN ONCOLOGY

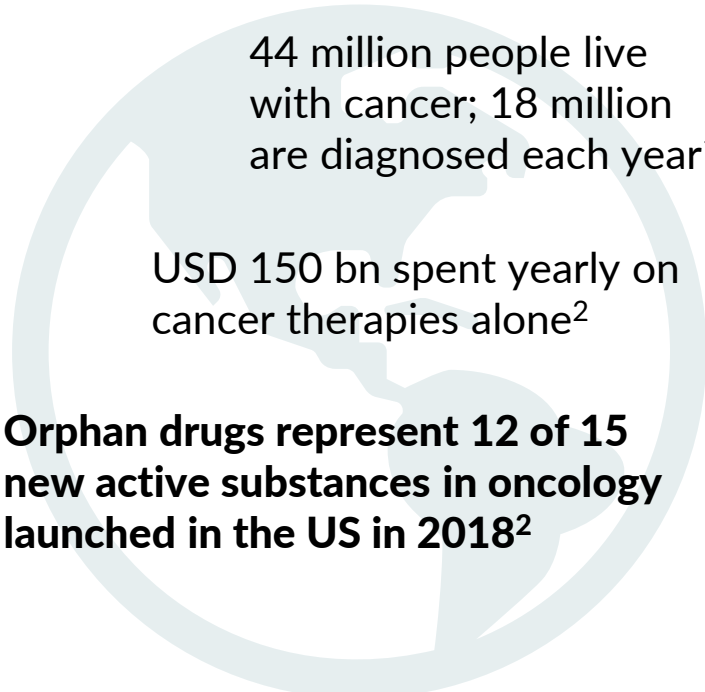
PRESENTATION AT ABGSC
LIFE SCIENCE SUMMIT
26 MAY 2020

ASCELIA
PHARMA

Share ticker: ACE
Nasdaq Stockholm (small cap)

ASCELIA PHARMA: ADVANCING ORPHAN ONCOLOGY

A global health burden



44 million people live with cancer; 18 million are diagnosed each year¹

USD 150 bn spent yearly on cancer therapies alone²

Orphan drugs represent 12 of 15 new active substances in oncology launched in the US in 2018²

Dedicated to unmet needs in orphan oncology

Drugs with a clear development and market pathway

- Advancing liver imaging with orphan MRI contrast agent with no competition (in ongoing Phase 3)
- Advancing chemotherapy with novel tablet for gastric cancer (Phase 2 ready)

Capabilities to bring new compounds to market

- World class cross-functional team
- Headquartered in Malmö, Sweden
- Listed on NASDAQ STOCKHOLM in 2019 (ticker: ACE)
- Solid financial position with SEK 169 million in liquid assets

Sources

- 1) <https://canceratlas.cancer.org/the-burden/the-burden-of-cancer/> (2018 figures)
- 2) Global Oncology Trends 2019, IQVIA (2018 figures)

CLINICAL STAGE PORTFOLIO ADDRESSING CLEAR UNMET NEEDS

Drug candidate	Indication	Phase 1	Phase 2	Phase 3	Filing	Launch
Mangoral <ul style="list-style-type: none">• Only <u>non</u>-gadolinium imaging drug• No competing products• \$350-500M market with upside potential• De-risked Phase 3 clinical program• Orphan Drug Designation	Visualisation of focal liver lesions <ul style="list-style-type: none">• Liver metastases• Primary liver cancer• Benign lesions	✓	✓	2020 – H2 2021	H1 2022	Q4 2022 – H1 2023
Oncoral <ul style="list-style-type: none">• Novel tablet chemotherapy formulation• Phase 1 completed with promising results• Gastric cancer is an Orphan indication	Treatment of gastric cancer	✓	2021 – 2023	Strong case for development and commercialisation partnering after phase 2		

An elderly couple is walking through a sun-dappled park. The woman, on the left, has short brown hair and is wearing a red short-sleeved top and light-colored pants. The man, on the right, has white hair and a mustache, wearing a blue and white checkered short-sleeved shirt and blue jeans. They are holding hands and smiling. In the foreground, a wicker picnic basket filled with food and drinks is visible. The background is filled with large, leafy trees.

MANGORAL

LIVER MRI CONTRAST AGENT IN
PHASE 3 CLINICAL STUDIES

LIVER METASTASES – A MAJOR CHALLENGE IN ONCOLOGY

LIVER METASTASES COMMON IN MANY CANCER TYPES

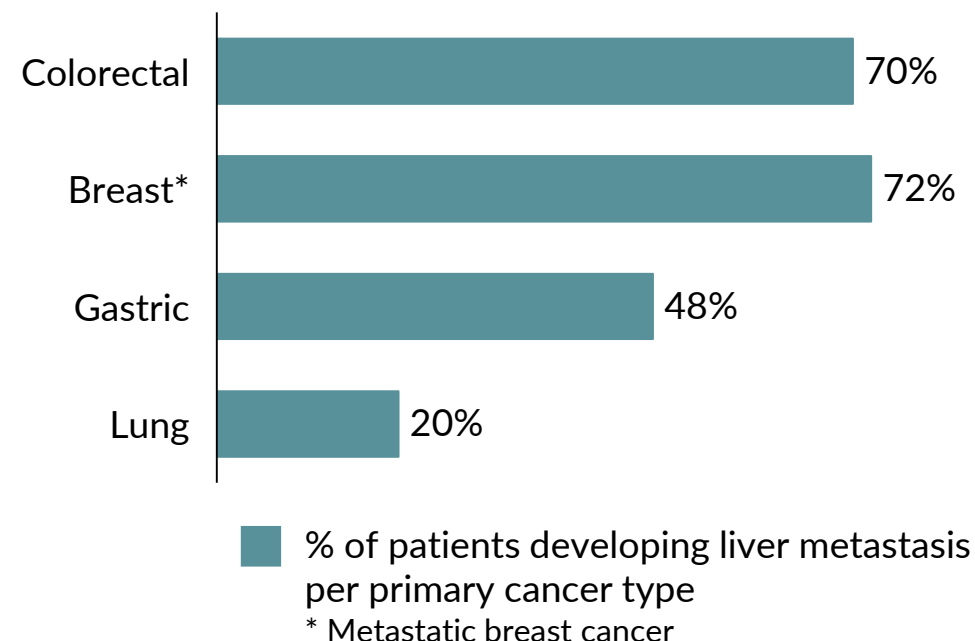
The liver is the **most frequent** organ for metastases after lymph node³ and often the first site of metastasis

- 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.

Liver metastases often the **cause of mortality** (not primary tumour)⁴



Incidence of liver metastasis in various primary cancers ¹⁻⁴



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 metastases²⁾

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



TREAT

Treatment options for liver metastases are:

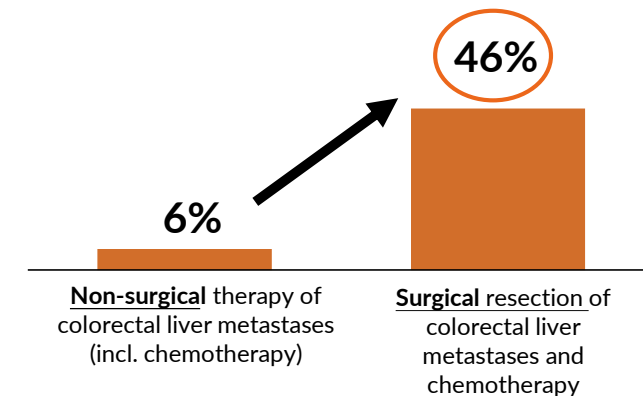
- Surgical resection (only if detected early)
- Localised therapies (ablation embolisation, radiation)
- Drug therapy

IMPROVE SURVIVAL

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

Example: Colorectal cancer³⁾

5-year overall survival rate



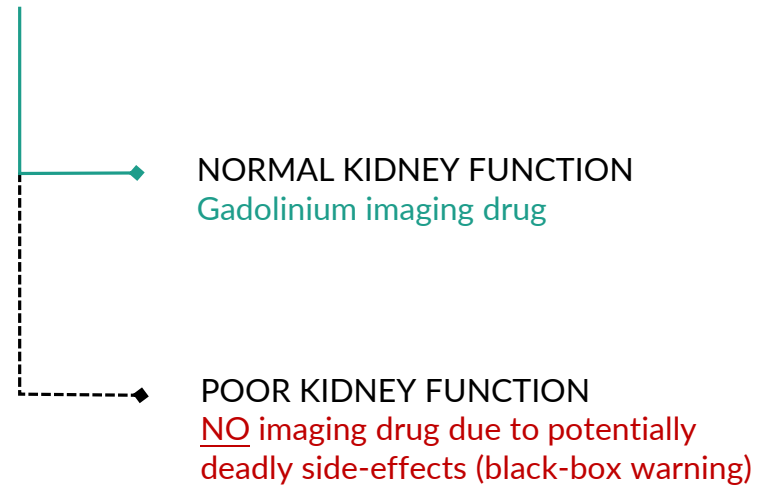
1) Guy diSibio and Samuel W. French (2008) Metastatic Patterns of Cancers: Results From a Large Autopsy Study. Archives of Pathology & Laboratory Medicine: June 2008, Vol. 132, No. 6, pp. 931-939

2) Albin N et al. Manganese chloride tetrahydrate (CMC-001) enhanced liver MRI: evaluation of efficacy and safety in healthy volunteers. MAGMA. 2012 Mar 8

3) Clinical Colorectal Cancer, Vol. 15, No. 4, Dec 2016, e183-192

MANGORAL – MANGANESE BASED LIVER CONTRAST AGENT

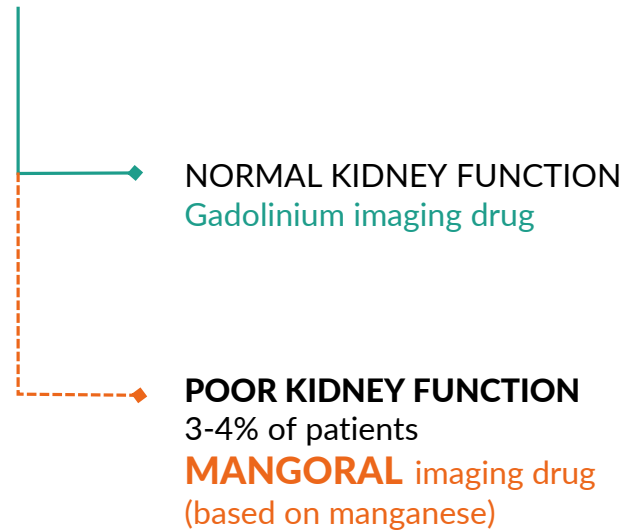
TODAY



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).

TOMORROW



Mangoral aims to be the only standard of care liver MRI imaging drug for patients with impaired kidney function

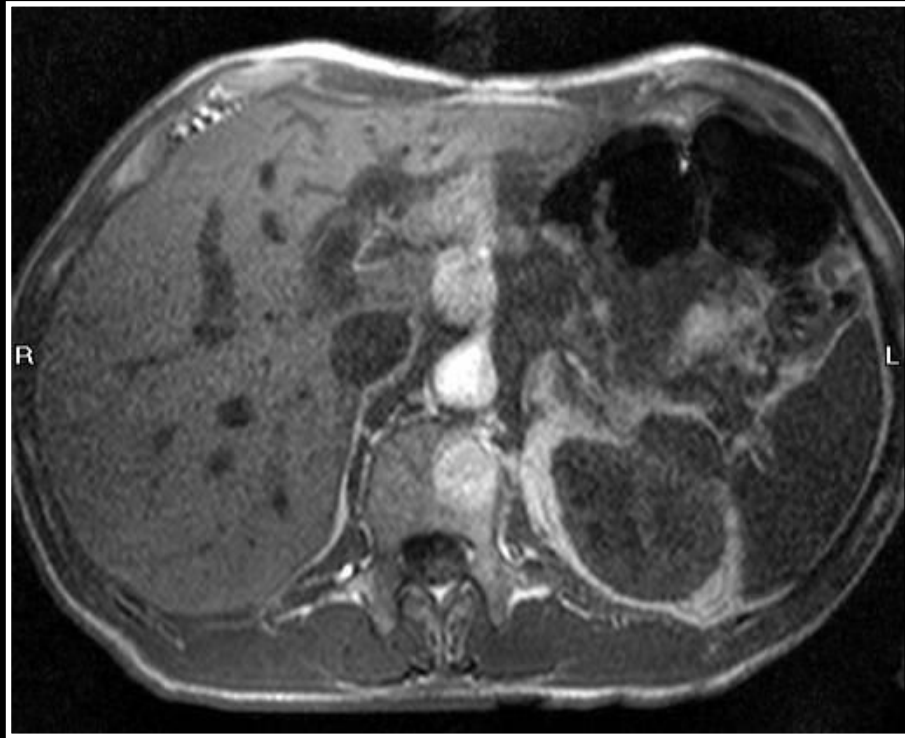


280,000

patients with impaired kidney function in major markets

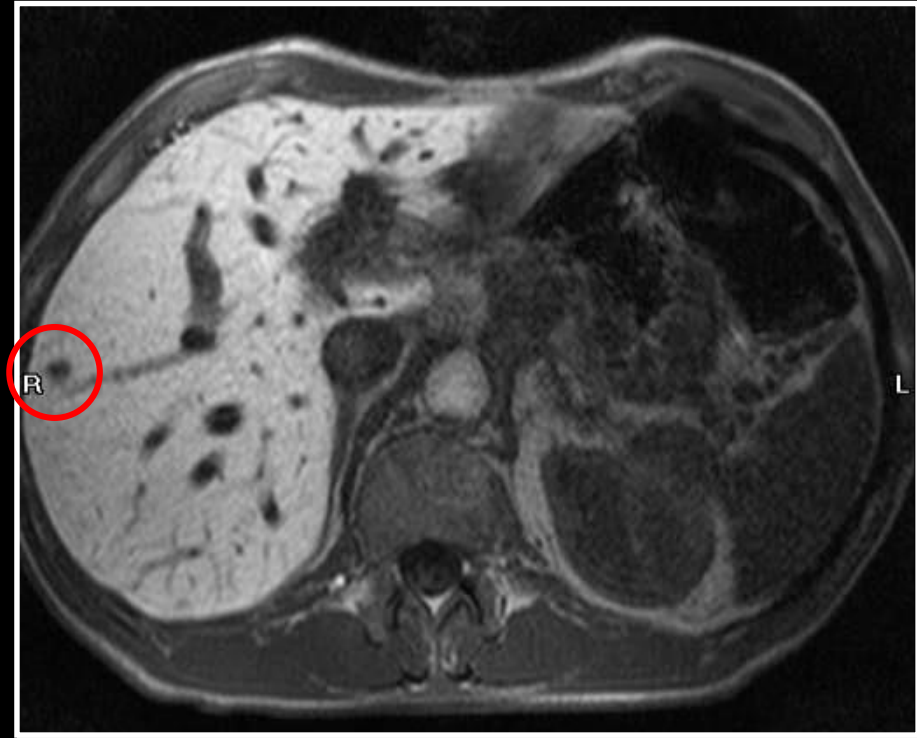
MANGORAL MAKES A REAL DIFFERENCE

PATIENT EXAMPLE FROM PHASE II STUDY



Unenhanced liver MRI

(standard of care today in target patient population)



Mangoral enhanced liver MRI

Liver metastasis appear with Mangoral

MANGORAL CLINICAL ACTIVITIES

Study	Objective of the study	Site location and no. of patients	Time schedule
Pivotal Phase 3 study ("SPARKLE")	Assess efficacy and safety of Mangoral in patients with severely reduced kidney function and with known or suspected liver lesions	Global multicentre study in up to 200 patients	<ul style="list-style-type: none"> • Study ongoing • Study results expected in H2-2021
Hepatic study	Assess the influence of hepatic impairment on the safety, pharmacokinetics and pharmacodynamics of Mangoral	Open-label study on 24 healthy and hepatically impaired participants at the Texas Liver Institute, San Antonio, US	<ul style="list-style-type: none"> • Study ongoing • Study expected to be completed in 2020
Food effect study	Assess the effect of food intake on Mangoral uptake	Study contract to be awarded	<ul style="list-style-type: none"> • Study preparations ongoing • Short study, expected to be completed in 2020

These studies, together with the already completed Phase 1 and 2 studies, ensure a comprehensive data package for the regulatory submissions in key markets

DE-RISKED PHASE 3 STUDY UNDERPINNED BY STRONG DATA FROM COMPLETED STUDIES AND STUDY DESIGN

Strong data package for Mangoral

Six phase 1 and 2 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 persons further underlined that Mangoral significantly improves MRI performance
- 33% more lesions were detected after Mangoral enhanced MRI
- **Mangoral significantly improved lesion visualisation**
Delineation: p-value <0.0001
Conspicuity: p-value <0.0001

Phase 3 registration-enabling study (study ongoing)

Number of patients

Global study in up to 200 patients

Endpoint



Lesion visualisation

- Lesion border delineation (border sharpness of lesions)
- Conspicuity (lesion contrast compared to liver background)

Comparator



Unenhanced MRI + Mangoral MRI
vs.
Unenhanced MRI

Follow-up



72 hours

Randomisation



No – each patient at his/her own control

Validation



Phase 3 program has been discussed with FDA and EMA

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

Addressable market

8.5 million people

... in key markets have suspected liver metastases or primary liver cancer (based on relevant primary cancers, colorectal, breast, lung and gastric)

280,000 of these

... equivalent to 3-4% have poor kidney function (stage 5, 4 or 3 worsening or acute kidney injury – with higher prevalence in elderly and cancer populations)

need MRI

... for diagnosis, monitoring and surveillance

at \$1,500 - 3,000 per dose

... based on unmet needs in patient population and value of contrast enhanced MRI

\$350–500 million

... addressable market annually with no competing drug

OUTLOOK FOR MARKET OPTIMAL LAUNCH STRATEGY

Strong case for own US commercialisation



US operations

- Field team of 10-20 FTEs can target 3,500-5,000 key accounts
- Target major hospitals with nephrology departments and independent specialist clinics
- US capability to include commercial and cross-functional support team

Optimal RoW uptake with partnering



Europe, Japan and RoW

- Market roll-out according to market potential, pricing and access
- Leverage global synergies in pre-launch and launch
- Ascelia vs. partner roles evaluated to maximise value

ADVANCING
ORAL CHEMOTHERAPY

ONCORAL

CHEMOTHERAPY TABLET FOR GASTRIC CANCER
READY FOR PHASE 2

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ONCORAL – NOVEL IRINOTECAN TABLET READY FOR PHASE 2

NOVEL ORAL PATENTED 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

With promising Phase 1 results, we are now preparing for Phase 2

A man and a young child are walking through a field of tall, golden-brown grass. The man, on the right, is wearing a red and white checkered shirt and dark trousers, and is holding the child's hand. The child, on the left, is wearing a blue jacket. The background is filled with trees with yellow and orange leaves, suggesting an autumn setting. A semi-transparent white banner is overlaid on the left side of the image, containing the text.

PRIORITIES 2020 AND INVESTMENT HIGHLIGHTS

Priorities in 2020



First patient in the Phase 3 SPARKLE study



First participant in the hepatic study



Work diligently with study sites during Covid-19 and enrol additional patients




Pre-launch activities and preparations for Mangoral (market launch planned for Q4 2022 – H1 2023)



Prepare Phase 2 study for Oncoral (planned start in 2021)

INVESTMENT HIGHLIGHTS



Ascelia Pharma (ticker: ACE) – Advancing orphan oncology

- Drugs targeting unmet medical needs with known mode of action and low development risk
- Solid financial position: SEK 169 million in liquid assets per 31 Mar 2020



Mangoral – Phase 3 non-gadolinium liver imaging drug

- \$350-500 million annual addressable market
- No competing drugs
- Ongoing Phase 3 program with high likelihood of success – study results expected in H2 2021
- Orphan Drug Designation



Oncoral – Phase 2 ready oral chemotherapy for gastric cancer

- Novel tablet formulation with significant patient and hospital benefits
- Effective molecule for killing cancer
- Promising Phase 1 results and preparing for Phase 2

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