



# ASCELIA PHARMA

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## ASCELIA PHARMA

ADVANCING ORPHAN ONCOLOGY

### Capital Markets Day

*Magnus Corfitzen, CEO*

*21 October 2020*

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# ASCELIA PHARMA – ADVANCING ORPHAN ONCOLOGY



IMPROVING THE LIFE FOR PEOPLE  
WITH RARE ONCOLOGY-RELATED  
CONDITIONS

## CURRENT ASSETS

### Mangoral

- Global Phase 3 development ongoing
- For liver MRI in subset of overall population (Orphan Drug)

### Oncoral

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

## CAPABILITIES

### People & Partners

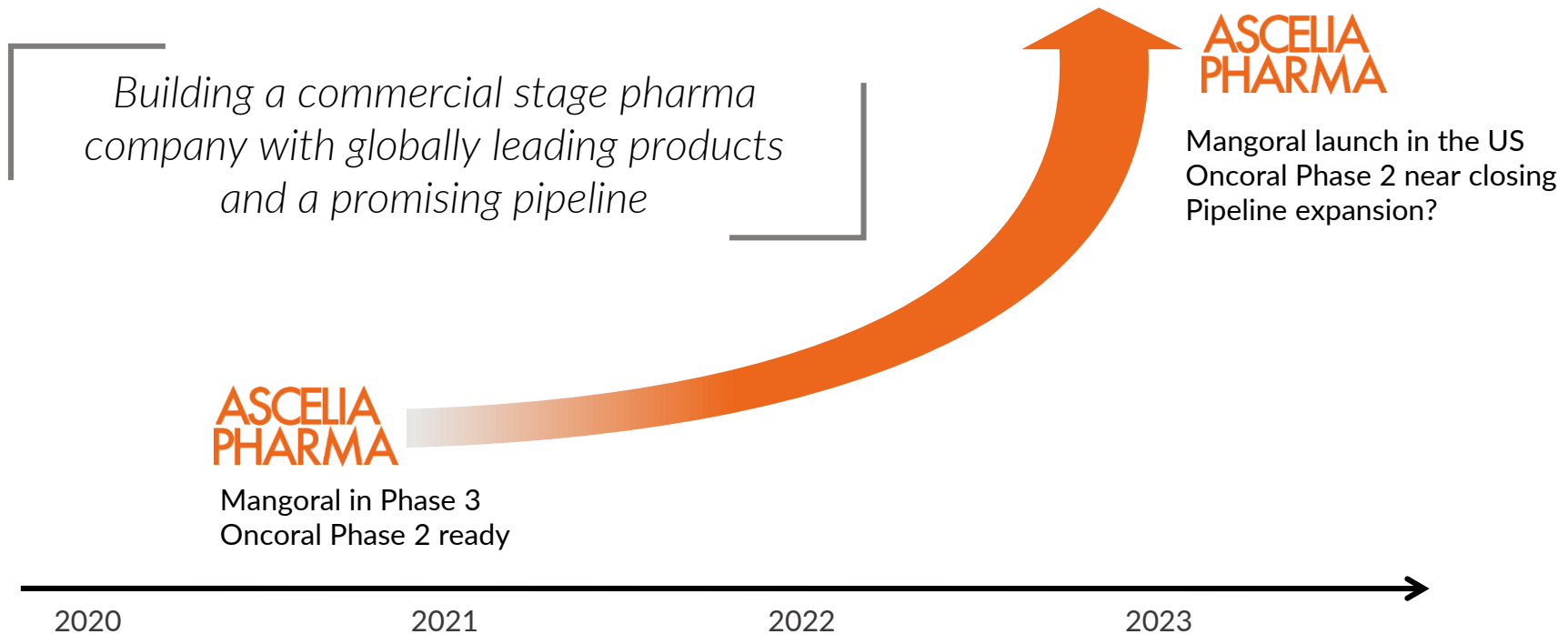
- Strong experienced team
- World class network of global KOLs and advisors
- Supply chain in place

### Financial

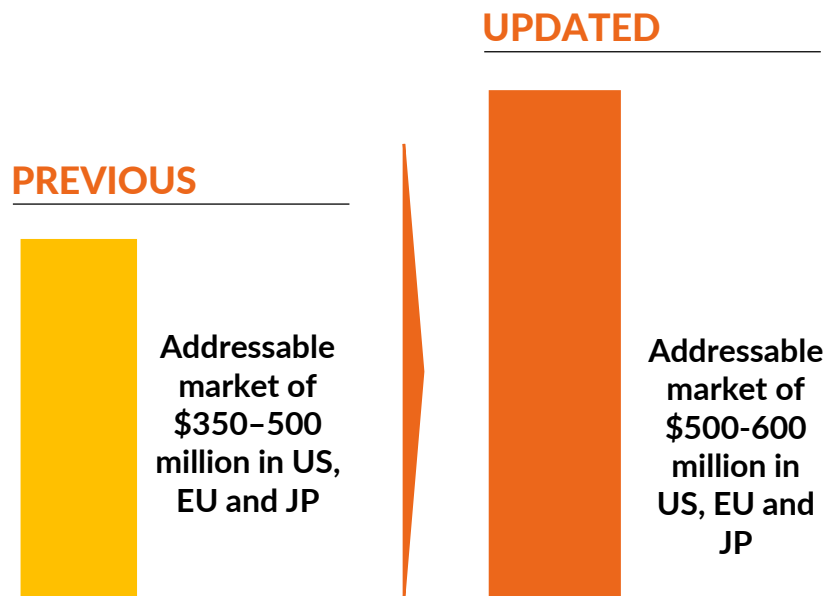
- Fully financed to reach substantial value creating milestones
- Listed on NASDAQ



# BUILDING ASCELIA PHARMA AND BUILDING VALUE



# INCREASED MANGORAL ADDRESSABLE MARKET TO \$500-600M



SAME PATIENT GROUP

NEW REAL-WORLD PROCEDURE DATA

ADDITIONAL PAYER AND EXPERT INPUT

# BRIGHT FUTURE AHEAD – CREATING VALUE




**Complete Mangoral development**



**Mangoral commercialization**



**Oncoral Phase 2 plan and execution**



**Grow the company**



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ADVANCING ORPHAN ONCOLOGY

### Capital Markets Day

*Julie Waras Brogren, Chief Commercial Officer*

*21 October 2020*



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# COMMERCIAL OUTLOOK

## Ascelia Pharma Capital Markets Day

October 21, 2020

ASCELIA  
PHARMA





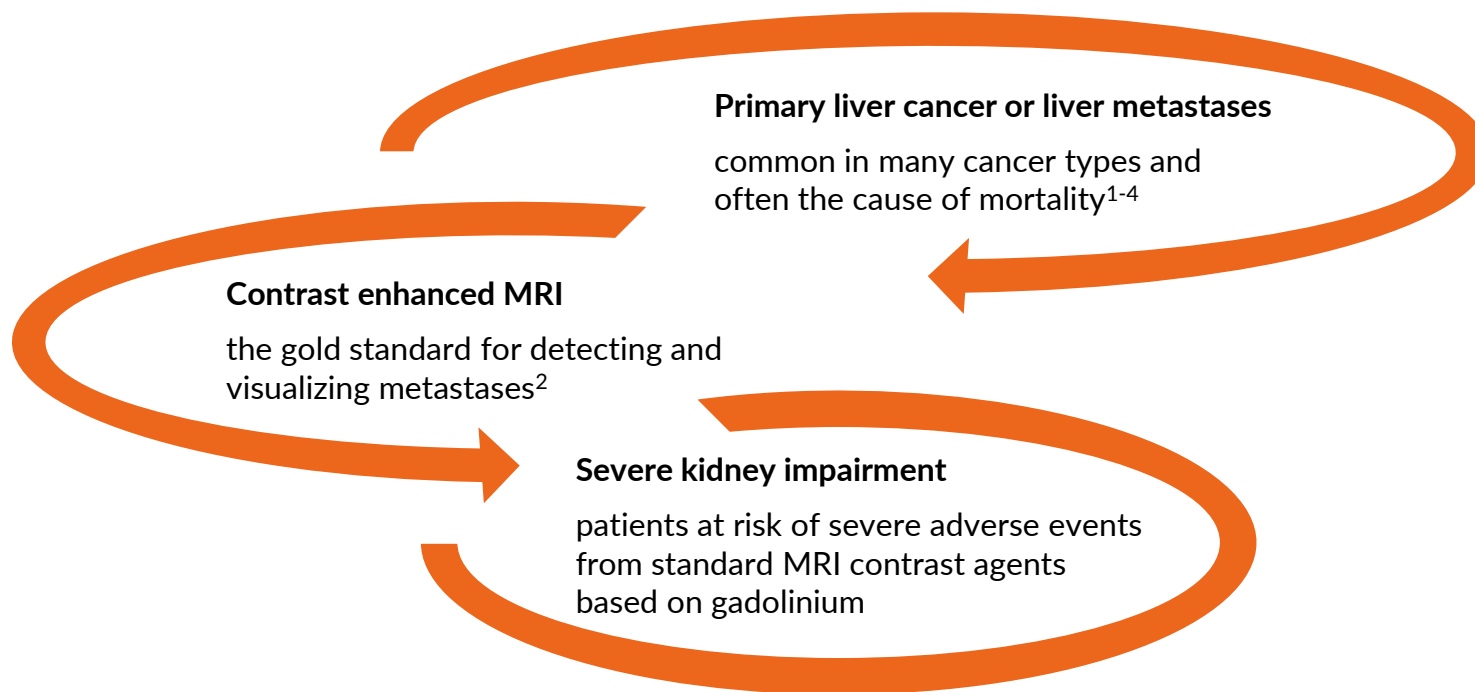
## Ascelia Pharma Capital Markets Day

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## MANGORAL MARKET OPPORTUNITY AND COMMERCIAL PREPARATIONS

- **Upgraded estimate** of addressable market to \$500-600 million (previously \$350-500 million)
- Decision makers **understand the value** that Mangoral provides
- **Launch preparations progress** with a strong case for own commercialization in the US

# WELL-DEFINED PATIENT POPULATION DRIVE POTENTIAL



Sources:

- 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 and ACR (American College of Radiology) Appropriateness Criteria
- 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



# PREVIOUS ADDRESSABLE MARKET ESTIMATE

## PREVALENCE AND MARKET RESEARCH

### **Patient population size based on epidemiology data**

- Suspected liver metastases or primary liver cancer
- Severe kidney impairment
- Estimated scans per patient from guidelines

### **Unmet needs based on literature and survey with 85 US radiologists**

### **Payer value market research based on input from 25 payers in key markets**

## PREVIOUS

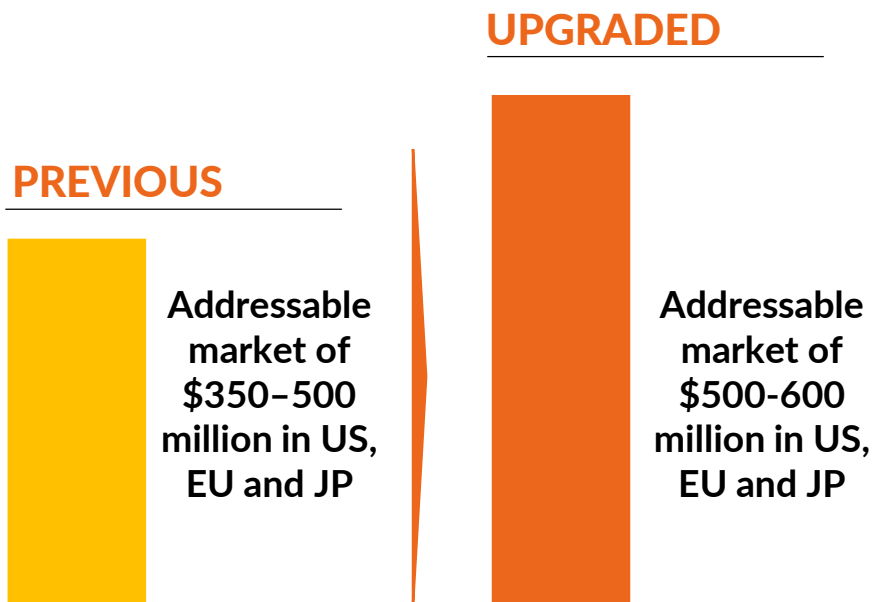
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**Addressable  
market of  
\$350–500  
million in US,  
EU and JP**

# ADDRESSABLE MARKET UPGRADED TO \$500-600 MILLION

*Illustrative*



## SAME PATIENT GROUP

### NEW REAL-WORLD PROCEDURE DATA<sup>1</sup>

- Primary cancer type & liver imaging
- Kidney impairment
- Imaging frequency per patient

### ADDITIONAL PAYER AND EXPERT INPUT<sup>2</sup> (+50 stakeholders)

- Unmet need and clinical guidelines validation
- Value of Mangoral and required evidence
- Pricing potential and reimbursement paths

Sources:

1) Market research with Decision Resources Group, 2020

2) Market research and analyses with Charles River Associates, 2020



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## MANGORAL MARKET OPPORTUNITY AND COMMERCIAL PREPARATIONS

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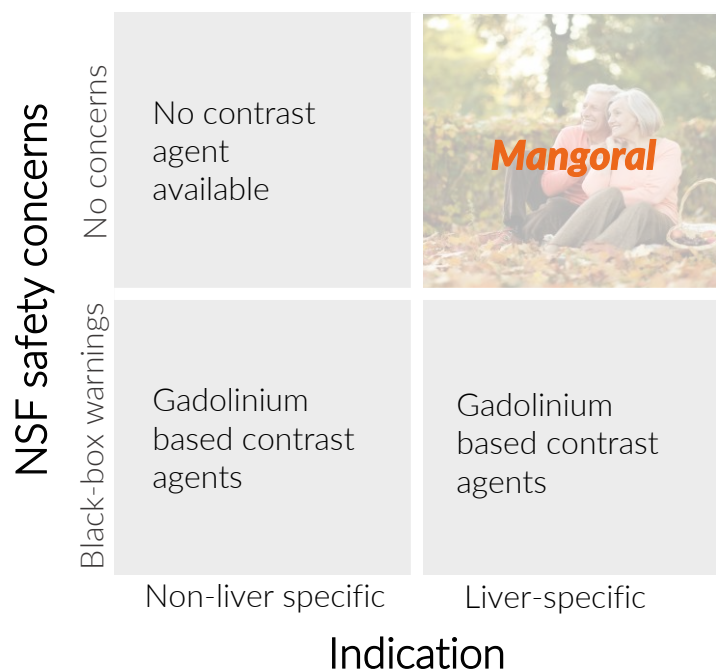
- **Upgraded estimate** of addressable market to \$500-600 million (previously \$350-500 million)
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# AIMING TO BECOME THE PREFERRED OPTION FOR PATIENTS WITH SEVERE KIDNEY IMPAIRMENT

*Illustrative*

## MRI CONTRAST AGENT LANDSCAPE

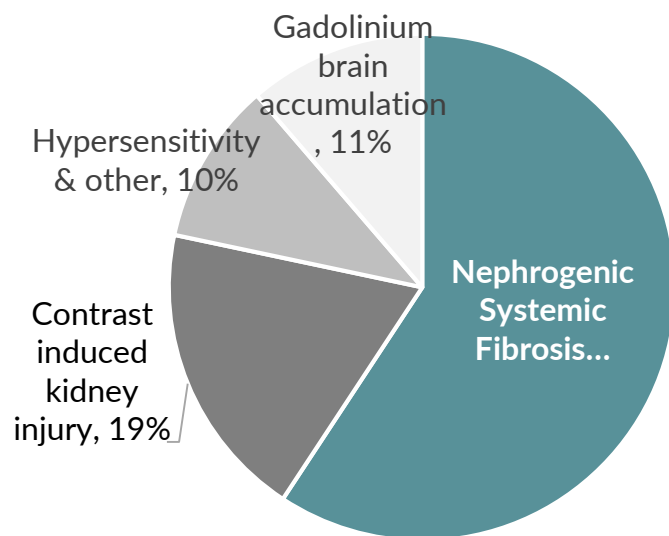


No contrast agent option is currently advised for liver imaging in patients with severe kidney impairment

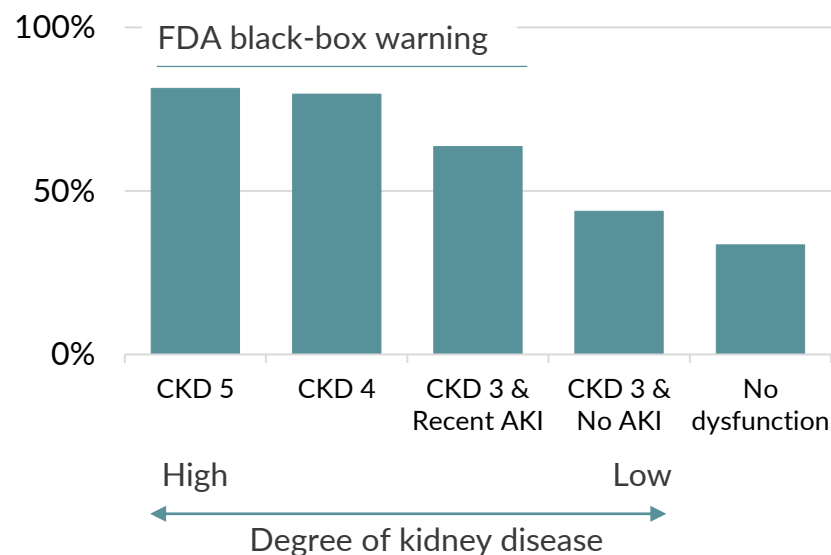
Mangoral aims to be the only safe and effective liver imaging contrast agent for people with suspected primary liver cancer or metastases severely impaired kidney function

# NSF RISK DRIVES CONTRAST AGENT DECISIONS

NSF remains the most concerning aspect of gadolinium-based contrast agents<sup>1</sup>



MRI without gadolinium-based contrast agent is preferred for patients at risk<sup>2</sup>



Source: Back Bay Ascelia market research survey with 84 US radiologists, 2019. Answers to: 1) "Please indicate the most concerning thing about using GBCAs? (100 points distribution)" and 2) "Of the patients with liver metastases seen in the last 12 months what % do NOT receive MRI with contrast?"

# DECISION MAKERS UNDERSTAND THE VALUE

Illustrative

## MANGORAL DECISION STAKEHOLDERS



### Key stakeholders

*Payers, Policy Makers and KOLs*

...are aware of the limitations of gadolinium-based contrast agents (GCBAs)

...and agree that Mangoral addresses important unmet needs

*"There is room and a need for a new product – it could be a very positive market.."*

*– National payer*

*"...budget impact should not be a problem"*

*– Health Technology Assessor*

Agreement Mangoral addresses needs



# ADDRESSING UNMET NEEDS OF PATIENTS AND PROVIDERS

## CLEAR VALUE PROPOSITION TO PATIENTS, PHYSICIANS AND HEALTH CARE SYSTEMS

85% of clinicians in a survey are likely or extremely likely to use Mangoral for target patient population<sup>1</sup>

**No MRI contrast agent advised**  
for patients with severe renal impairment  
or acute kidney failure<sup>2</sup>

Clear target  
patient  
population

**Improved visualisation**  
of focal liver lesions (incl. metastases)  
compared to unenhanced MRI

+33% more  
lesions<sup>3</sup>

**Early detection**  
of focal lesions and metastases allows early  
intervention and higher survival rate

94% clinicians  
confirm<sup>4</sup>

Sources:

1) Market research by Back Bay Life Science Advisors with survey of 84 US radiologists

2) Based on ACR clinical guidelines and regulatory drug class warning for gadolinium-based contrast agents in patients with severe renal impairment (an eGFR <30 ml/min/1.73 m<sup>2</sup>) or acute kidney failure.

3) Outcomes from Phase 1 and 2 studies

4) 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?'



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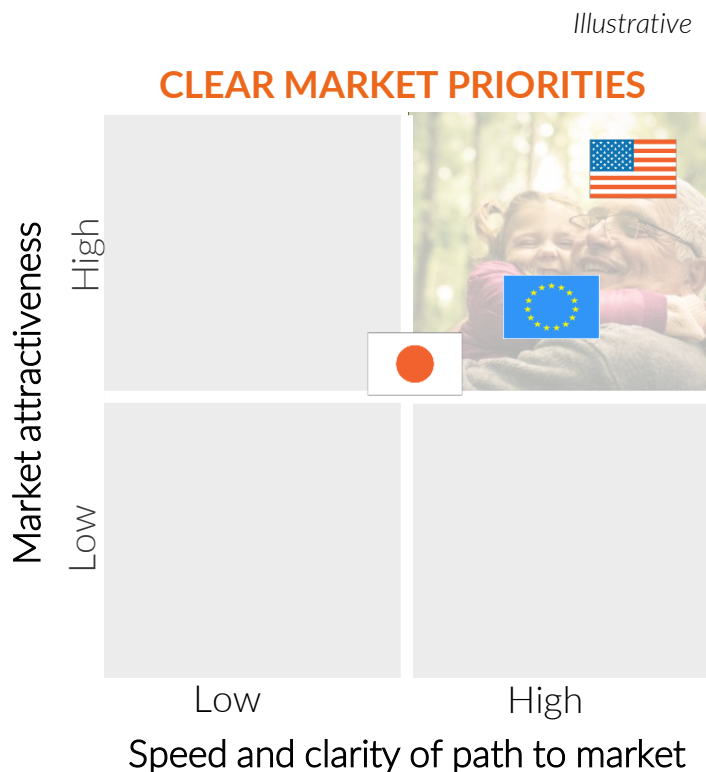
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## MANGORAL MARKET OPPORTUNITY AND COMMERCIAL PREPARATIONS

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# THE US IS THE MOST IMPORTANT MARKET OPPORTUNITY



**US**  
represents the most attractive market for Mangoral launch and has a clear regulatory and market access pathway

**EU and Japan**  
represent the next priority markets

**Other markets**  
under further evaluation, incl. China

# MARKET SPECIFIC VALUE MAXIMIZING COMMERCIALIZATION

*Illustrative*



Ascelia Pharma to drive commercialization



Commercial  
partner



Ascelia Pharma global synergies

Commercial  
partner



Commercial  
partner



# STRONG CASE FOR OWN COMMERCIALIZATION IN THE US



## STRONG RELATIONSHIPS IN THE US

SPARKLE Phase 3 Study sites with leading US radiologists at approximately 15 sites, incl.

- Yale University School of Medicine
- Massachusetts General Hospital
- Standard School of Medicine
- Harvard Medical School

Manufacturing partner, New Jersey



Imaging experts  
New York

Hepatic Impairment Study  
Clinical research site  
Texas Liver Institute



## STRONG CASE FOR OWN US TEAM

### Attractive potential

- Strong payer value and market access path
- Sizeable target patient population
- Clear clinical decision makers

### US capability

- Sales team of ~20 FTEs reach priority physicians
- Commercial and cross-functional support team
- Local logistics and distribution partnerships
- Leverage global synergies

**Building an attractive top-line and retaining profit in Ascelia Pharma**

# WELL DEFINED DECISION MAKERS IN THE US



## DECISION MAKERS TO REACH

### Payers

Coverage expected by Public and Commercial plans

- Medicare/Medicaid cover ~70% of Mangoral patient population<sup>1</sup>
- Value evidence requirements identified for formulary assessment and procurement

### Hospitals/ clinics

Reachable hospitals/clinics

- ~750 hospitals/clinics perform ~75% of liver MRI procedures<sup>1</sup>
- ~400 hospitals/clinics serve ~75% of patients with kidney impairment<sup>1</sup>

### Physicians

Radiologists main clinical decision makers

- Radiologists drive 95% of contrast agent decisions; nephrologist involved<sup>2</sup>
- ~2,000 radiologists perform regular liver MRIs for patients with kidney impairment<sup>1</sup>

Notes:

1) Ascelia analysis based on market research for Ascelia Pharma by Decision Resources Group, 2020

2) Back Bay Ascelia market research survey with 84 US radiologists, 2019

# OUTLOOK FOR MARKET OPTIMAL LAUNCH STRATEGY

## REST OF WORLD PARTNERING



## OPTIMAL VALUE

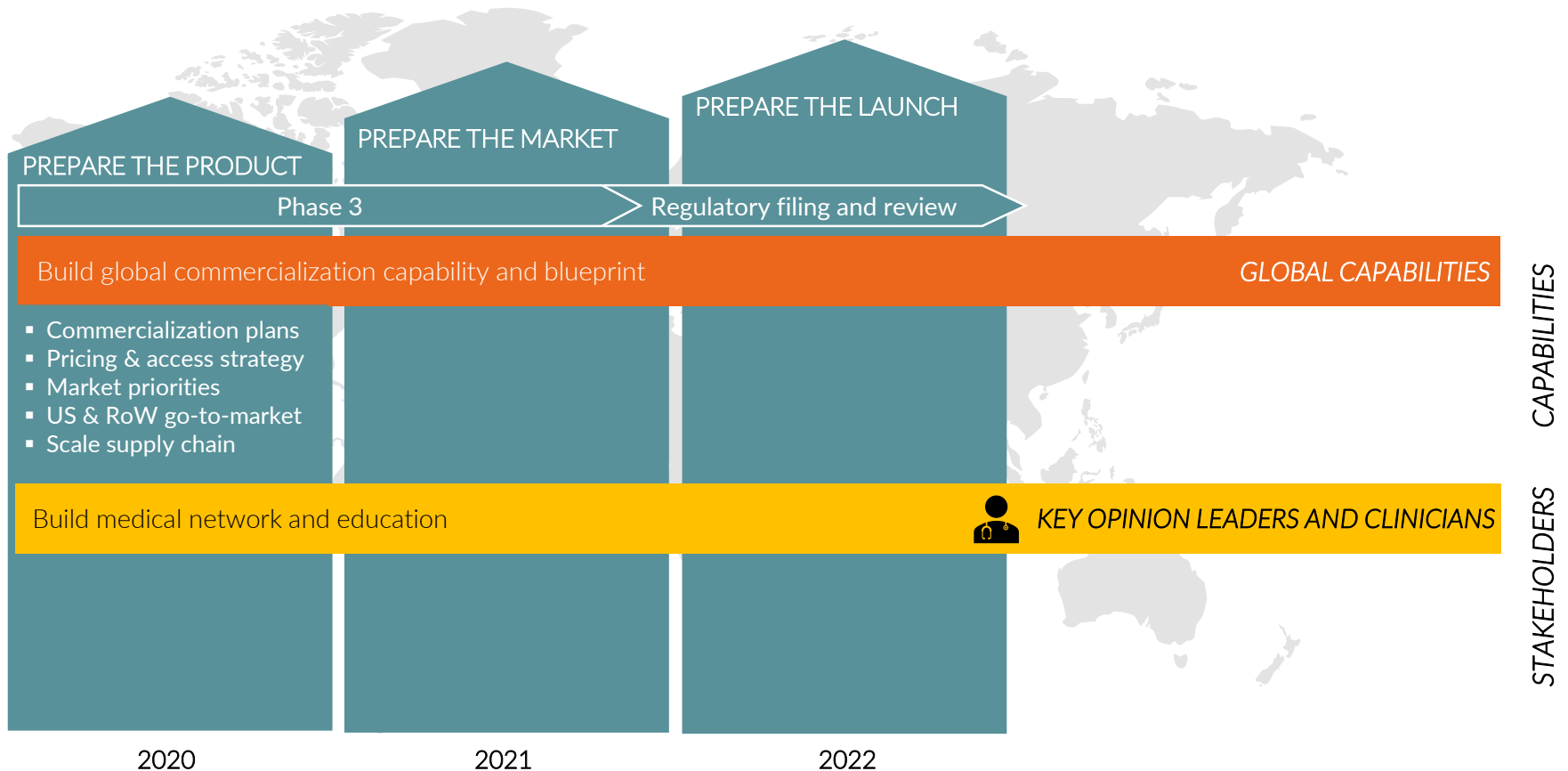
### Focus on Europe and Japan

- Sizeable target patient population
- Maturity of MRI practice
- Pricing and reimbursement opportunity defined
- Regulatory pathway clear
- Other markets under assessment, e.g. China

### Partnering opportunity

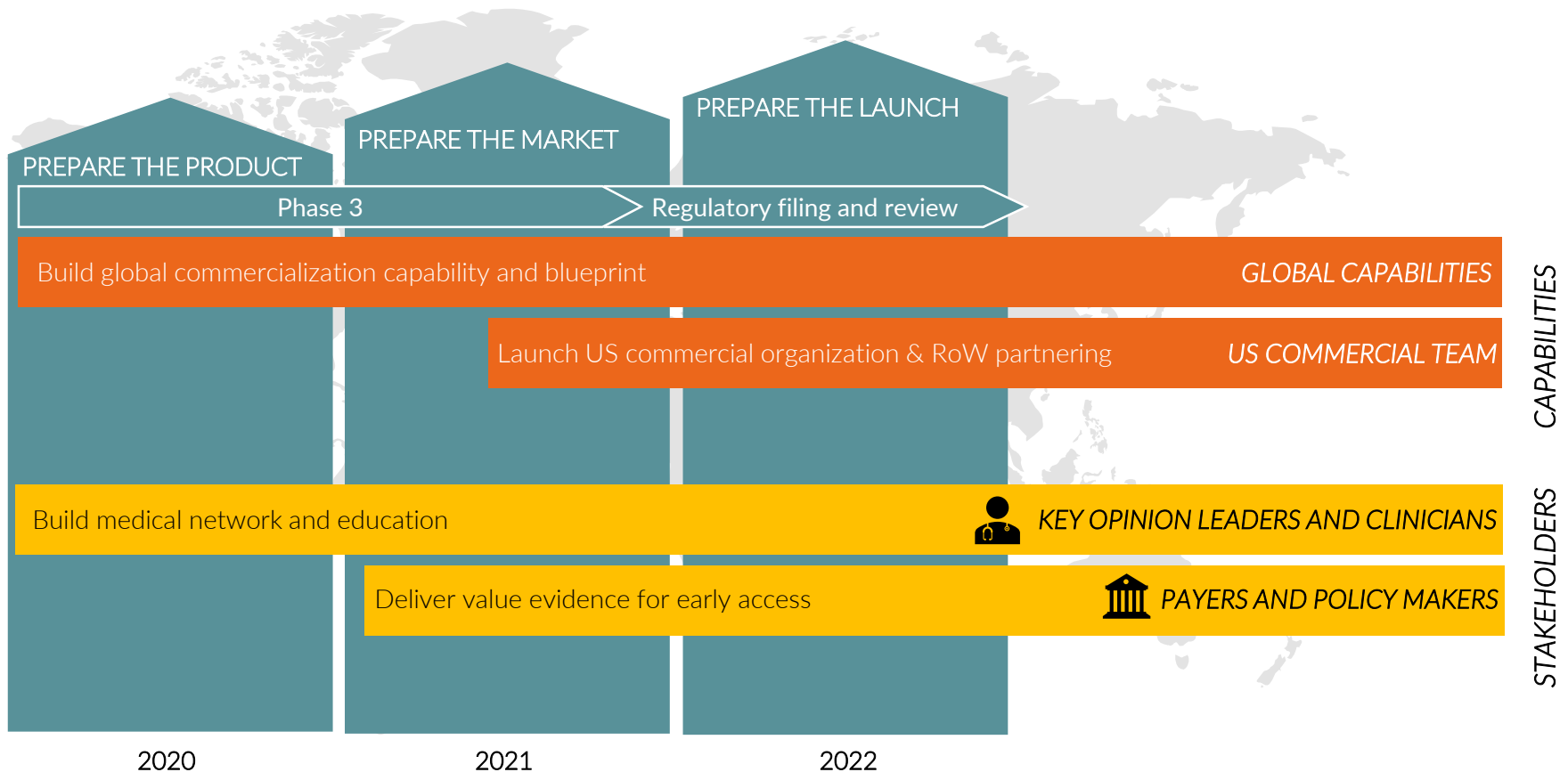
- Focused launches to reach target clinicians and patient population
- Ideal partnership based on capabilities and strategic match
- Leverage global synergies to maximize value

# MANGORAL GO-TO-MARKET: BLUEPRINT READY IN 2020

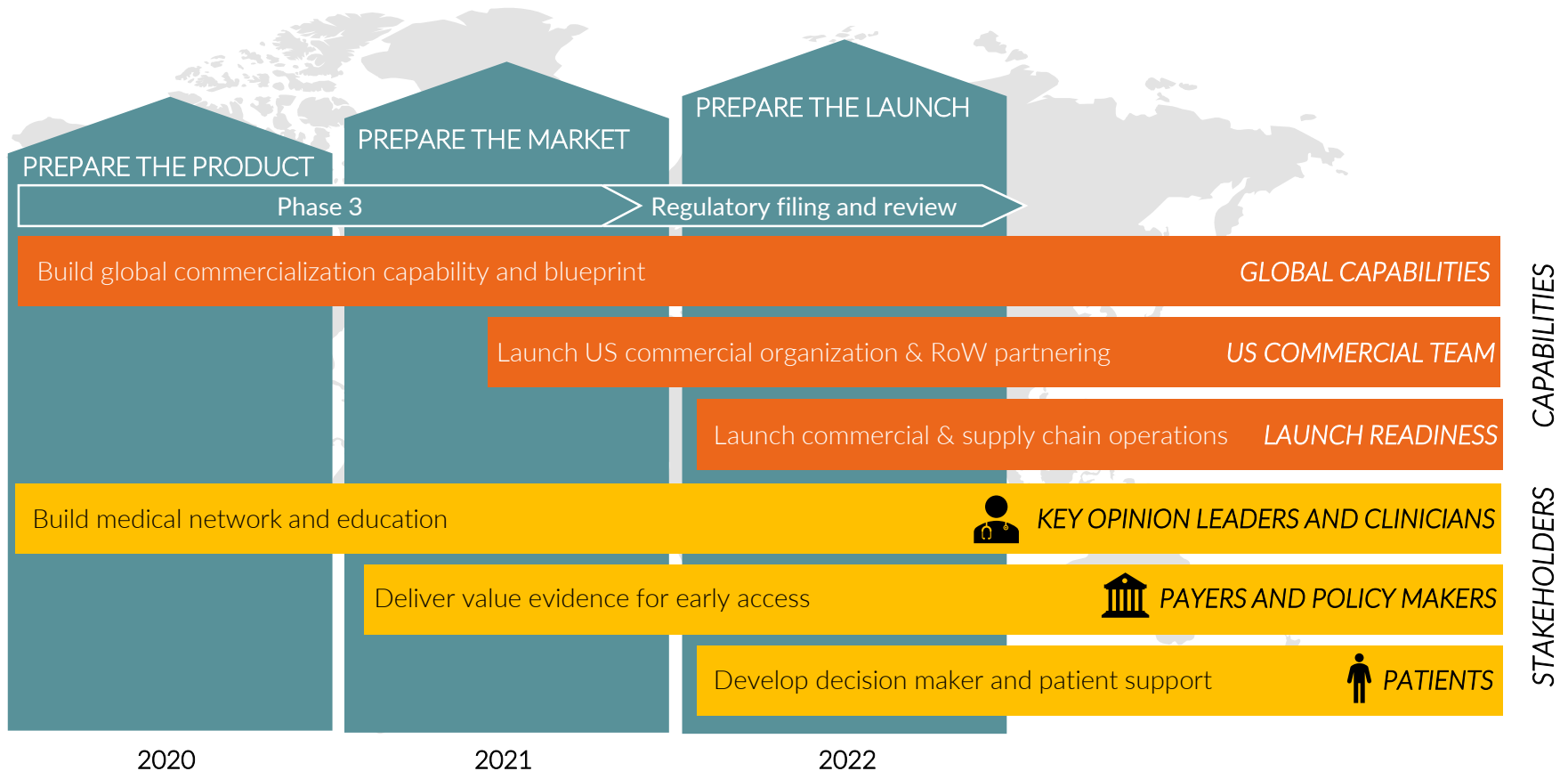




# MANGORAL GO-TO-MARKET: LAUNCH US TEAM IN 2021



# MANGORAL GO-TO-MARKET: LAUNCH READY END OF 2022





## Ascelia Pharma Capital Markets Day

October 21, 2020

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*Carl Bjartmar, Chief Medical Officer*

*21 October 2020*



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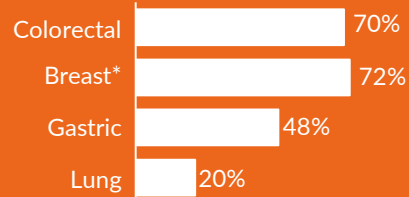
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# CLEAR UNMET MEDICAL NEED

## Liver metastases

- ... are the most common malignant liver lesions
- Develops in many cancer types and often the cause of mortality

Incidence rate of liver metastasis in various primary cancers <sup>1-4</sup>



■ % of patients developing liver metastasis per primary cancer type

\* Metastatic breast cancer

## Contrast-enhanced MRI

- ...is the gold standard for early and accurate detection of liver metastases
- Guideline recommended method for:
  - Detection and visualization of liver metastases
  - Planning of surgery or treatment (localised therapies or drugs)
  - Monitoring and surveillance post-treatment

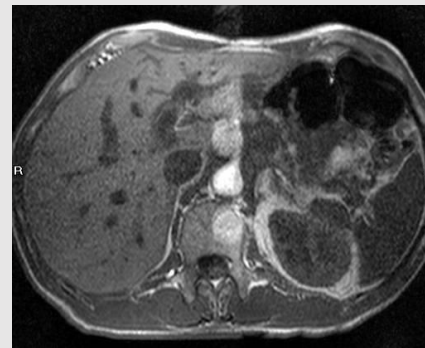
## Patients with impaired kidneys

- ...are recommended not to receive the standard gadolinium-based MRI contrast agents
- Risk of serious side-effects
- Gadolinium-based contrast agents carry regulatory black box warnings (FDA, EMA, PMDA etc)
- These patients lack an MRI contrast agent – a clear unmet medical need

# MANGORAL – ADVANCING LIVER IMAGING

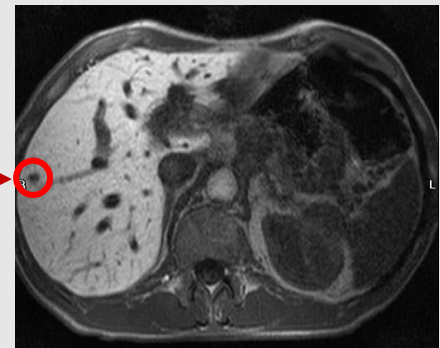
## MANGORAL

- Oral manganese-based diagnostic drug for liver MRI
- Targeting patients with severe renal insufficiency
- These patients are at risk of potentially fatal side effects from the products on the market based on gadolinium
- Orphan Drug Designation from FDA
- Ongoing Phase 3 study SPARKLE



**Unenhanced** liver MRI

*(standard of care today in target patient population)*



**Mangoral** enhanced liver MRI

*Liver metastasis visualized with Mangoral*

# MANGORAL – ADDRESSING THE UNMET MEDICAL NEED

## TODAY

→ NORMAL KIDNEY FUNCTION  
Gadolinium imaging drug

→ POOR KIDNEY FUNCTION  
NO imaging drug due to  
potentially fatal side-effects  
(black-box warning)

## TOMORROW

→ NORMAL KIDNEY FUNCTION  
Gadolinium imaging drug

→ POOR KIDNEY FUNCTION  
~4% of patients  
**MANGORAL** imaging drug  
(based on manganese)

**Mangoral aims** to be the standard of care liver MRI imaging drug for patients where gadolinium-based contrast agents may be medically inadvisable or cannot be administered



# ONGOING PHASE 3 STUDY – SPARKLE

## 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 	<b>Lesion visualisation</b> <ul style="list-style-type: none"><li>• Lesion border delineation (border sharpness of lesions)</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 3 program has been discussed with FDA and EMA

# WORLD-CLASS CLINICS PARTICIPATING IN SPARKLE

## Global Phase 3 study (SPARKLE)

- 40–50 clinical sites including:
  - Yale University School of Medicine, USA
  - Harvard Medical School, USA
  - Stanford School of Medicine, USA
  - Charité Universitätsmedizin, Germany
  - Karolinska University Hospital, Sweden

## Leading expert radiologists participating

- Strong relationship and support
- Awareness of Mangoral
- Validates the unmet medical need

# MANGORAL PHASE 3 DEVELOPMENT PROGRESSING

Mangoral indication	Phase 1	Phase 2	Phase 3	Filing	Market
<b>Visualization of focal liver lesions:</b> <ul style="list-style-type: none"><li>• Liver metastases</li><li>• Primary liver tumors</li><li>• Malignant or benign</li></ul>	Completed	Completed	Ongoing 2020 – 2H 2021	1H 2022	Q4 2022- 1H 2023