

Annual Meeting  
2019

# Ascelia Pharma – building a Medicon Valley based company with global ambitions

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medicon valley alliance

Creating Opportunities

**ASCELIA  
PHARMA**

Share ticker: ACE  
Nasdaq Stockholm (small  
cap)

# ANNUAL MEETING 2019 MEDICON VALLEY ALLIANCE

Copenhagen

4 November 2019

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# INVESTMENT HIGHLIGHTS



## Ascelia Pharma (ticker: ACE) – Orphan oncology company

- Drugs targeting unmet medical needs with known mode of action and low development risk
- Listed on NASDAQ Stockholm - Fully financed Phase III program for Mangoral<sup>®</sup>



## Mangoral<sup>®</sup> – Phase III ready liver imaging drug

- \$350-500 million annual addressable market
- No competing drugs
- Phase III program with high likelihood of success – results end 2020 / early 2021
- Orphan Drug Designation



## Oncoral – Phase II ready chemotherapy tablet for gastric cancer

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

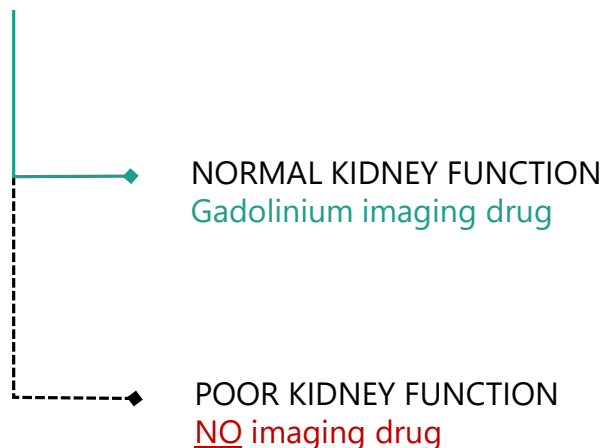


MANGORAL<sup>®</sup>

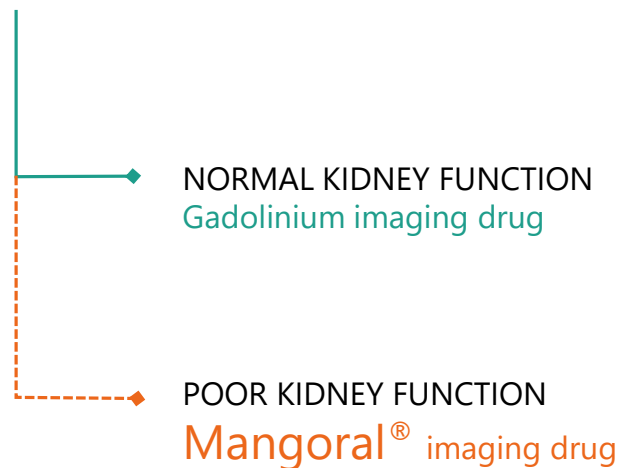


# MANGORAL<sup>®</sup> – PATIENTS REFERRED FOR LIVER MRI SCAN

## TODAY



## TOMORROW



**Mangoral<sup>®</sup>** aims to be the new gold standard liver MRI imaging drug for patients with impaired kidney function



**280,000**

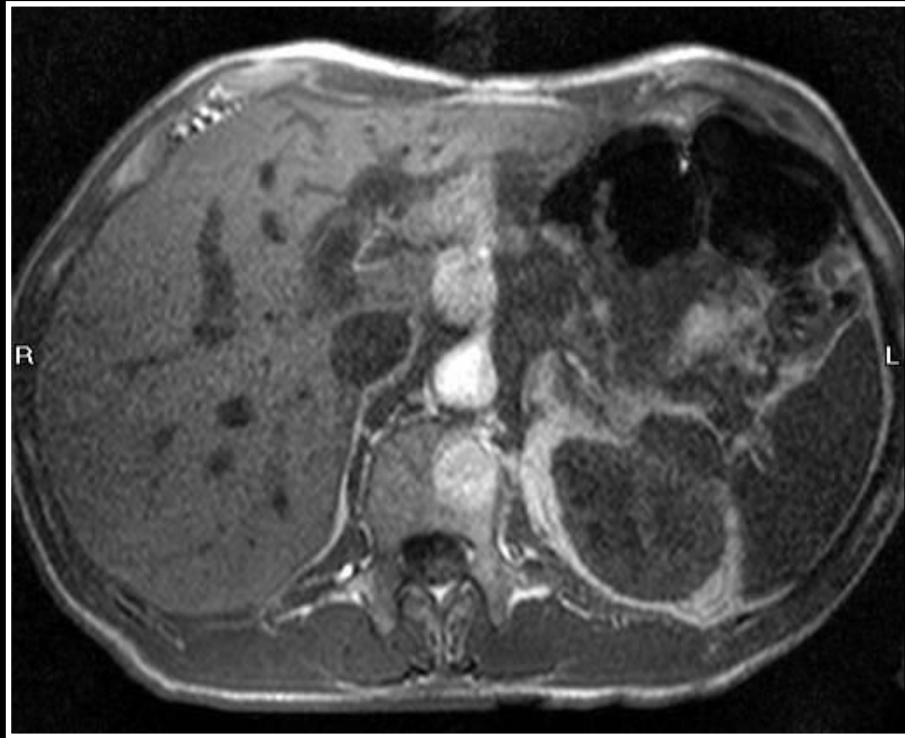
patients with impaired kidney function in major markets

**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-contrast 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).

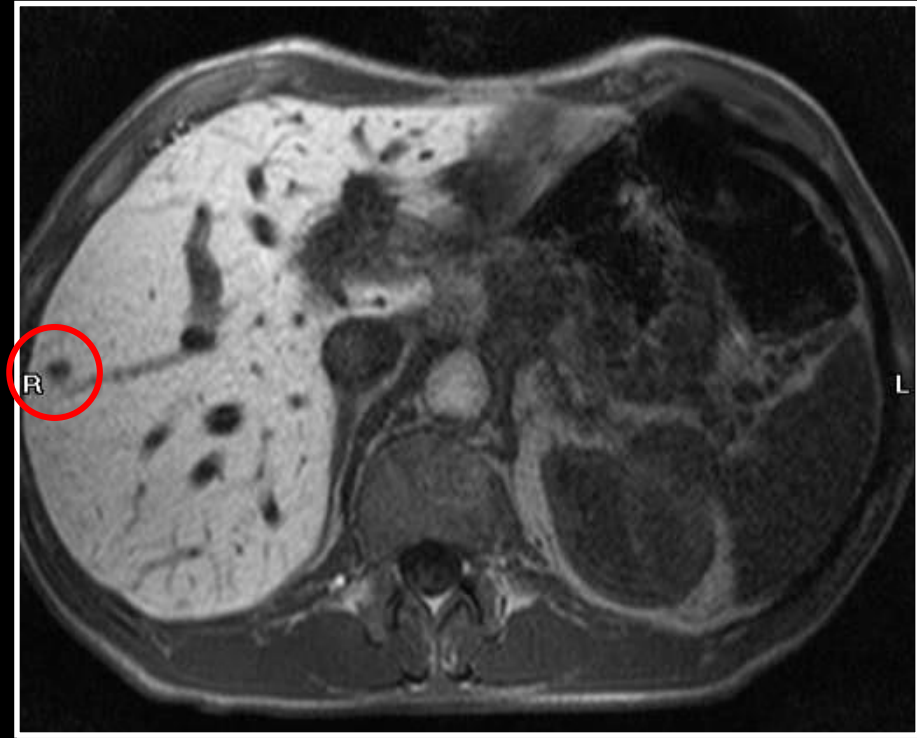
# MANGORAL<sup>®</sup> MAKES A REAL DIFFERENCE

## PATIENT EXAMPLE FROM PHASE II STUDY



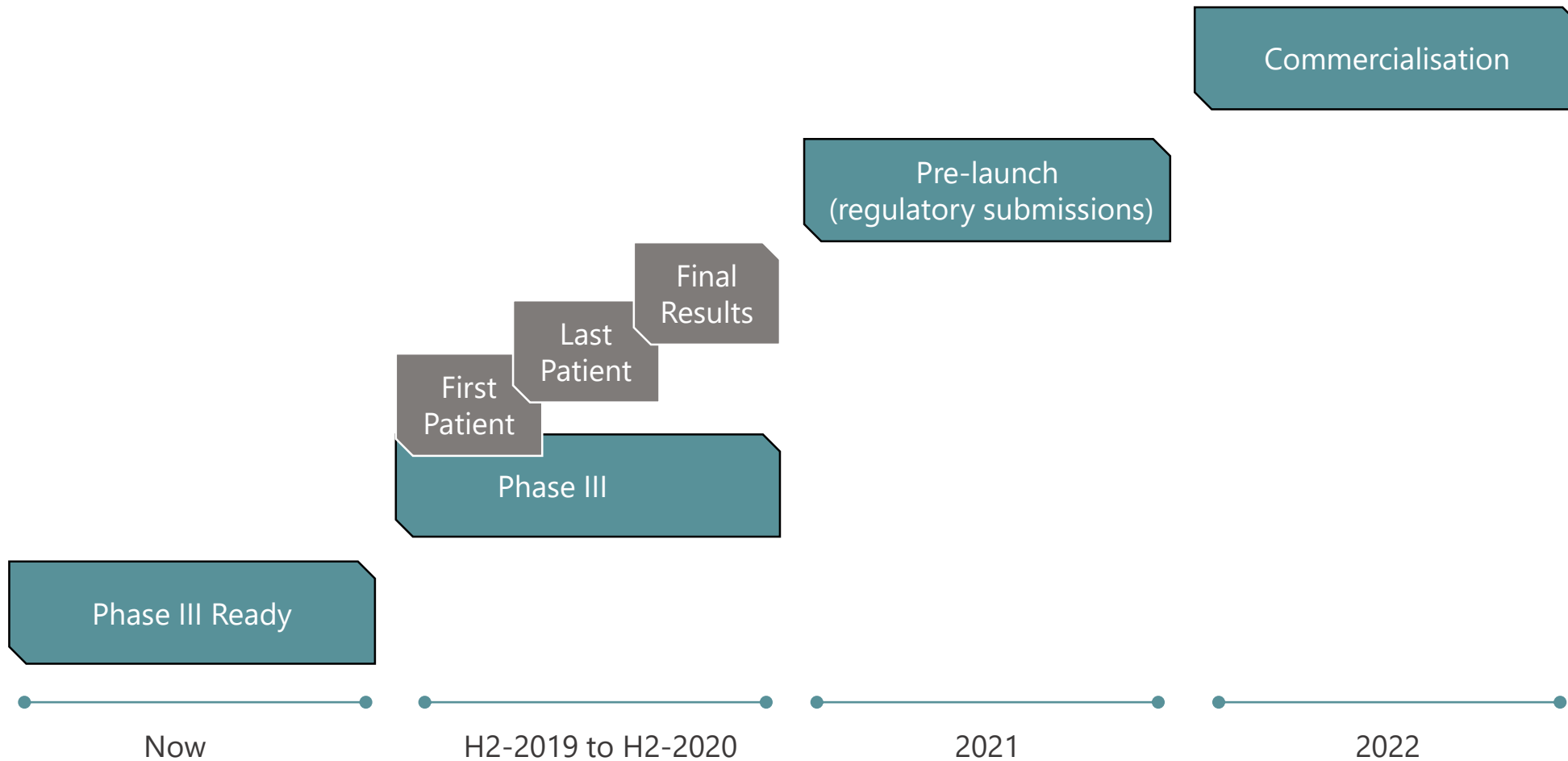
**Unenhanced liver MRI**

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



**Mangoral<sup>®</sup> enhanced liver MRI**






# MANGORAL<sup>®</sup> – RAPIDLY APPROACHING THE MARKET

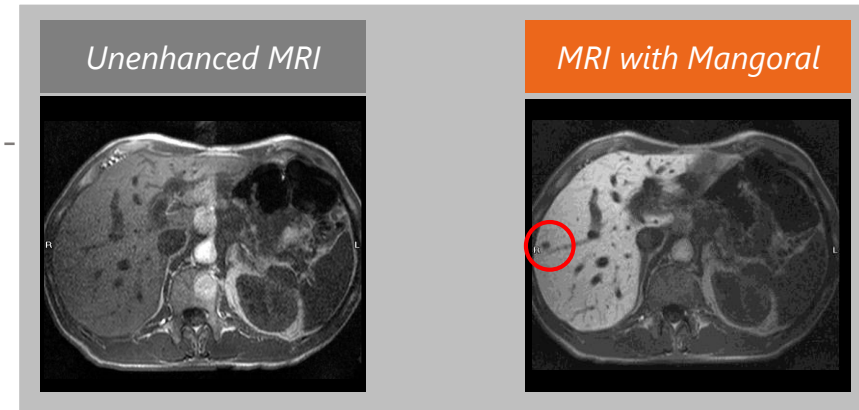




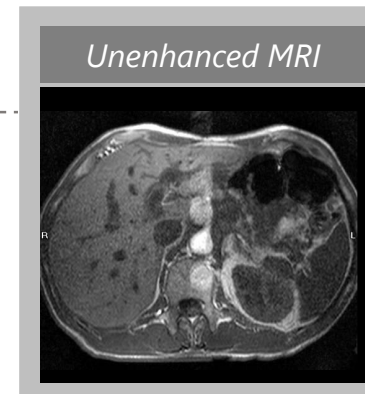
# MANGORAL<sup>®</sup> PHASE III CLINICAL STUDY DESIGN IMPLIES INCREASED LIKELIHOOD OF SUCCESS

Mangoral pivotal registration-enabling study design – based on Phase III protocol meeting with FDA and EMA

<b>Number of patients</b>	Up to 200 patients
<b>Endpoint</b> 	Lesion visualisation <ul style="list-style-type: none"><li>• Lesion border delineation</li><li>• Lesion contrast compared to liver background</li></ul>
<b>Comparator</b> 	Unenhanced MRI + Mangoral MRI vs. Unenhanced MRI
<b>Evaluation</b> 	Centralised evaluation by 3 radiologists
<b>Randomisation</b> 	<b>No</b> – each patient will be in both study arms
<b>Follow-up</b> 	72 hours



**VS.**



# MANGORAL<sup>®</sup> IS THE ONLY PRODUCT IN A \$350-500M MARKET

## Overview of Mangoral<sup>®</sup>'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 annual addressable market for Mangoral<sup>®</sup> and no competing drugs**

## 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<sup>®</sup>*



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 demonstrated in Phase I clinical study



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 **in combination** with other well-recognized anti-cancer drugs

# 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

# ONCORAL TARGETING A >\$4 BILLION MARKET IN GASTRIC CANCER

## DISEASE CHARACTERISTICS

- Gastric cancer is the 6<sup>th</sup> most prevalent cancer in the world<sup>1)</sup>
- Gastric cancer is the 3<sup>rd</sup> most frequent cause of cancer death<sup>1)</sup>
- The 5-year survival of gastric cancer is approximately 20%<sup>2)</sup>

## MARKET OPPORTUNITY

- Market for gastric cancer treatment forecast to increase to >\$ 4 billion in 2024<sup>3)</sup>
- Drug treatment typically combination of 2-3 drugs

### Key growth drivers

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- ① Increase in overall incidence of gastric cancer
- ② Anticipated increase in treatment rates
- ③ Extended treatment duration
- ④ New lines of more expensive therapies
- ⑤ Increased number of patients receiving branded therapy

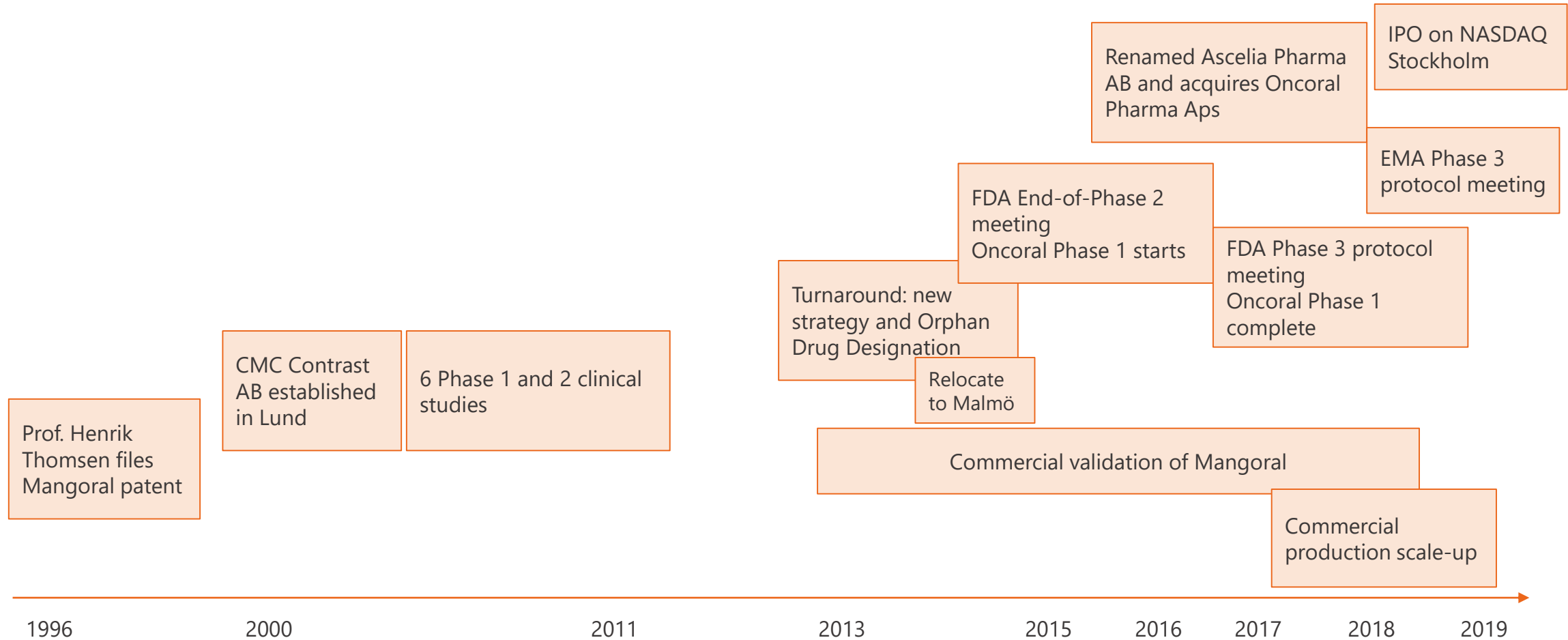
1) IARC (2012)

2) Clinical Colorectal Cancer 2015; 14(4): 239-50

3) GlobalData - Gastric and Gastroesophageal Junction Adenocarcinoma – Global Drug Forecast and Market Analysis to 2024

ASCELIA PHARMA  
THE HISTORY OF A MEDICON  
VALLEY COMPANY

# ASCELIA PHARMA HISTORY





# MEDICON VALLEY IMPACT ON ASCELIA PHARMA DNA



- Excellent people with pharma and biotech backgrounds
- Most people in Ascelia Pharma have worked in both countries
- Copenhagen airport proximity



- Strong science in the region
- Mangoral and Oncoral both invented in Medicon Valley



- Some VC firms in Sweden and Denmark – more are needed
- NASDAQ Stockholm eco-system to facilitate biotech IPO's

# PERSONAL RECOMMENDATION - SWEDEN

- Labor laws more appropriate for large industrial companies - not dynamic start-ups
- Example:
  - Recruitment process similar to Denmark until signing of contract
  - Notice period typically 3 months for the employee
    - 'Loss of motivational momentum' from signing to start
    - Significant impact on ability to grow – 'lost opportunities'



Look to Denmark for inspiration for a more flexible job market – will be better for employees and companies

# PERSONAL RECOMMENDATION - DENMARK

- Essential element for evolving successful start-up companies into commercial successes is capital
- Denmark has 2nd highest tax on capital gains (source: OECD)
- High taxation is not only a tool to limit cigarette use – but also a tool to make capital scarce to build companies, create jobs and create a prosperous society



Look to Sweden for inspiration to make capital available to companies by rethinking taxation on capital gains tax

# ASCELIA PHARMA

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