



**CORPORATE PRESENTATION**  
**FEBRUARY 2020**

**MEDIVIR**

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# An oncology-focused development company set for growth

## Proprietary nucleotide-prodrug platform

- Multiple opportunities for breakthrough oncology products
- Spearheaded by MIV-818 and MIV-828

## Advanced clinical programs for partnering/out-licensing

- Remetinostat, Birinapant and MIV-711

## The company

- Experienced leadership team and effective organization
- Focus on clinical development and business development

# Nucleotide-prodrug projects

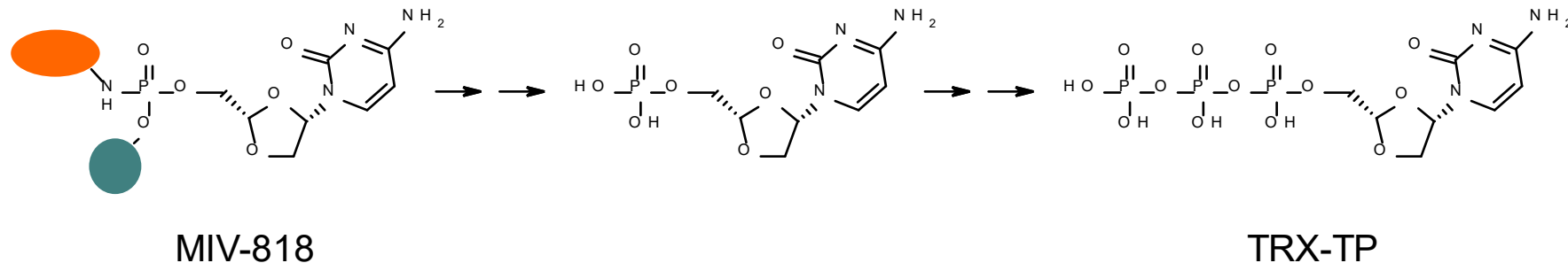
# The nucleotide-prodrug concept: A versatile source of new oncology products

- By combination of “prodrug tail” and a nucleotide, a tunable uptake in target cell/tissue can be achieved.
- Once in the cancer cell, the prodrug is cleaved and an active nucleotide metabolite is formed.
- This concept has the potential to provide oncology products with an improved efficacy/tolerability profile.

Nucleotide prodrug	Indication	Research	Preclinical	Phase I	Exclusivity
MIV-818	HCC				IP : 2035
MIV-828	AML				IP : Est 2039
"MIV-838"	Blood cancer				IP : Est 2040

# MIV-818: A liver-targeted nucleotide

- MIV-818 is an oral prodrug of the clinically effective troxacitabine
- Once absorbed from the GI-tract, MIV-818 is transported to the liver
- The prodrug is taken up by liver cancer cells and converted into troxacitabine triphosphate (TRX-TP)
- TRX-TP is incorporated into DNA and causes double-strand DNA breaks and cell death



# MIV-818: A nucleotide-prodrug for primary liver cancer

Current treatment options for hepatocellular carcinoma (HCC) provide little benefit. Because of the liver targeting and the mechanism of action, MIV-818 may provide an outstanding efficacy and safety profile. May be ideal as stand alone treatment and/or add-on to standard of care.

HCC is the most common form of primary liver cancer:

- Third leading cause of cancer-related deaths globally
- Orphan disease in western markets, high incidence in Asian markets
- Five year survival: 11%
- Genetically heterogeneous; no good molecularly targeted therapy available

# Preclinical evidence for MIV-818 liver targeting

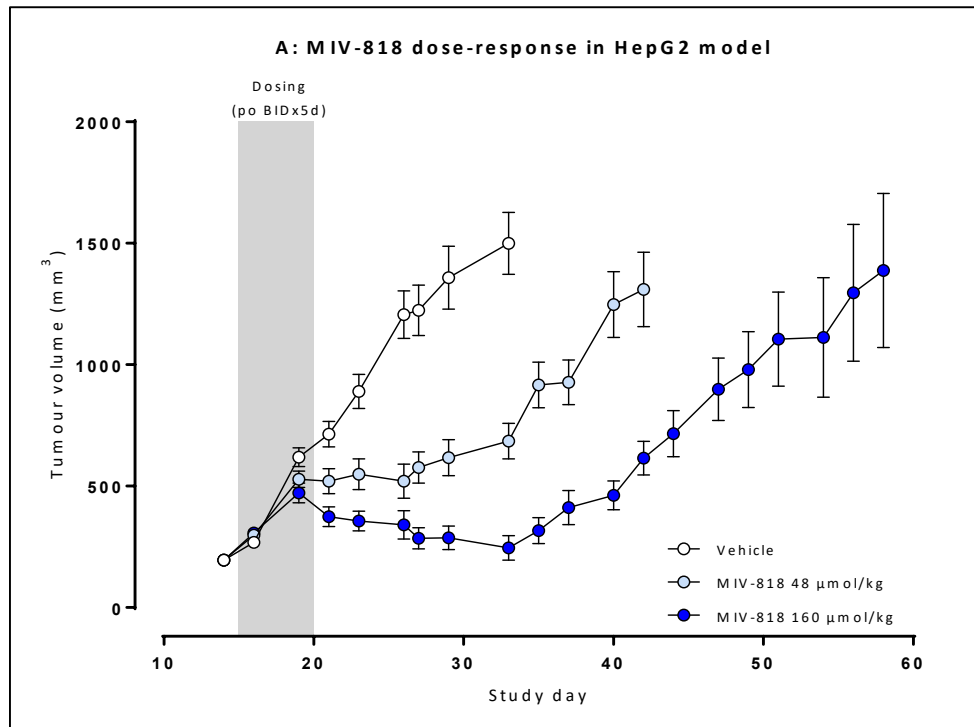
- MIV-818 exhibited substantial liver targeting by preferential formation of the active TRX-TP metabolite in liver of rats
- The liver targeting of the TRX-TP metabolite was 100-fold higher for MIV-818 than for troxacitabine

Compound	Route	Dose (μmol/kg)	Liver TRX-TP/Plasma TRX (AUC ratio)
Troxacitabine (TRX)	<i>iv</i>	80	<0.016
MIV-818	<i>oral</i>	80	1.9

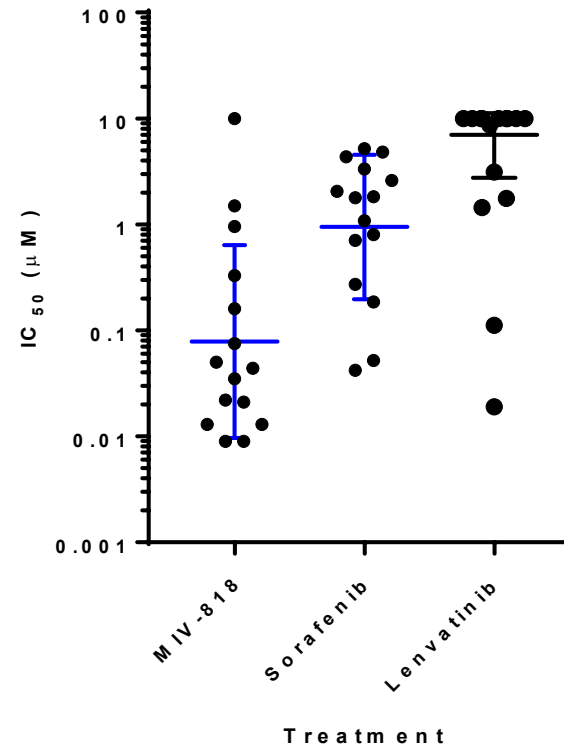


# MIV-818 shows efficacy in preclinical HCC models

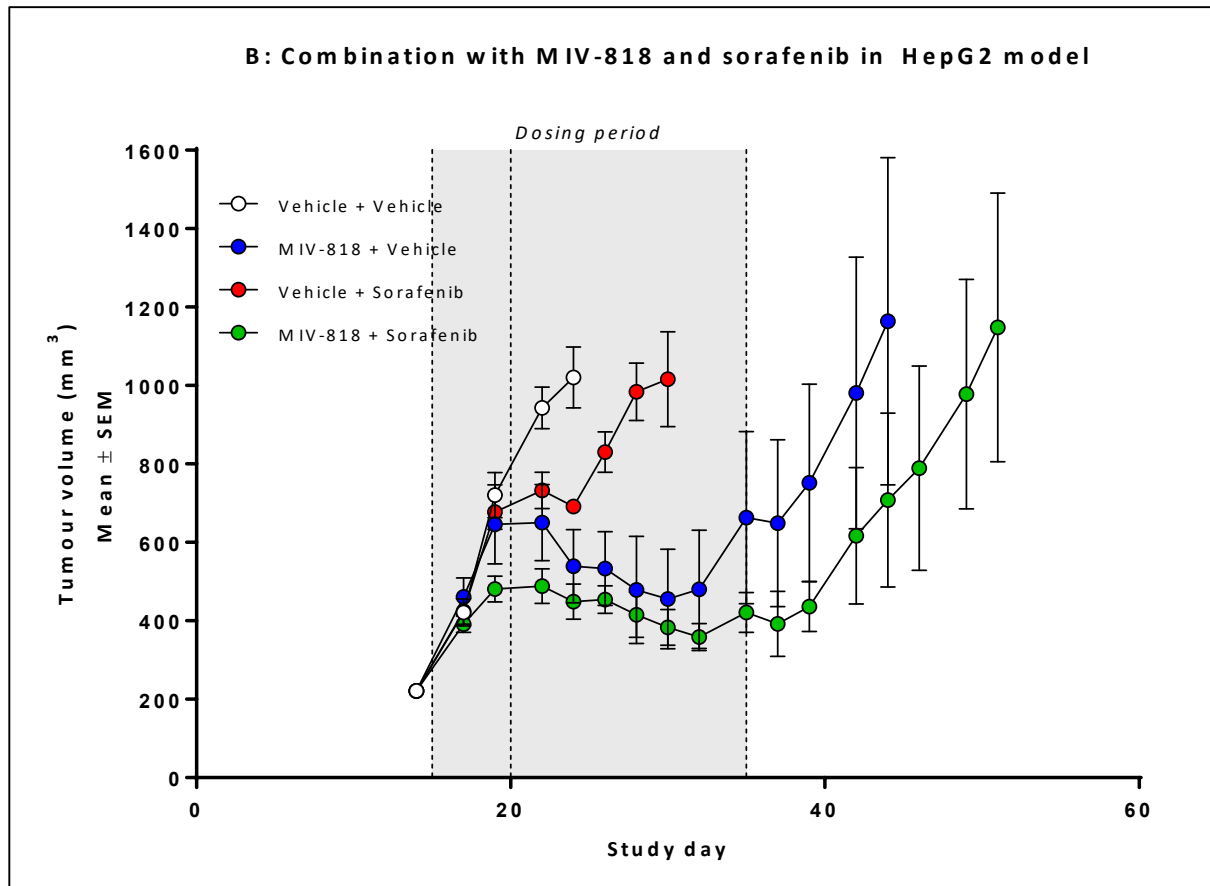
Inhibition of tumour growth in mouse HCC xenograft models in vivo



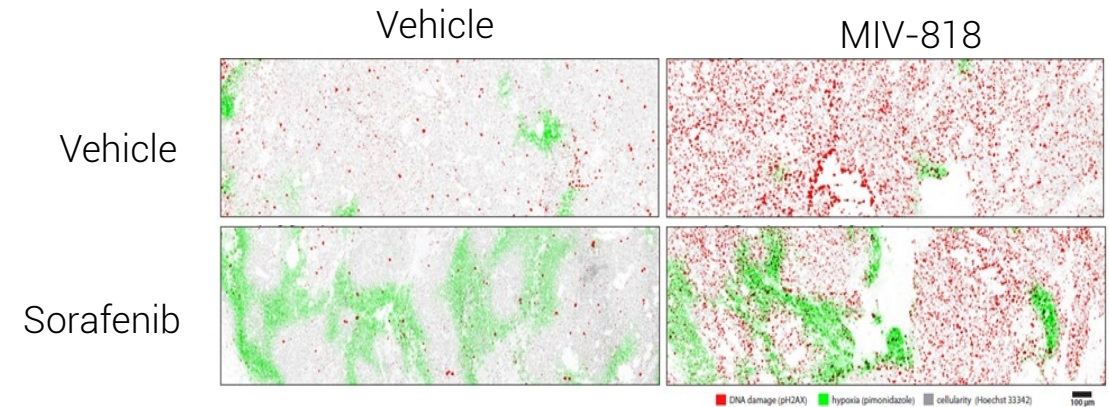
Inhibition of patient-derived HCC cell lines in vitro



# MIV-818: enhanced anti-tumor effect in combination with sorafenib in preclinical HCC models



DNA damage (pH2AX) and hypoxia in mouse HCC tumor model HepG2

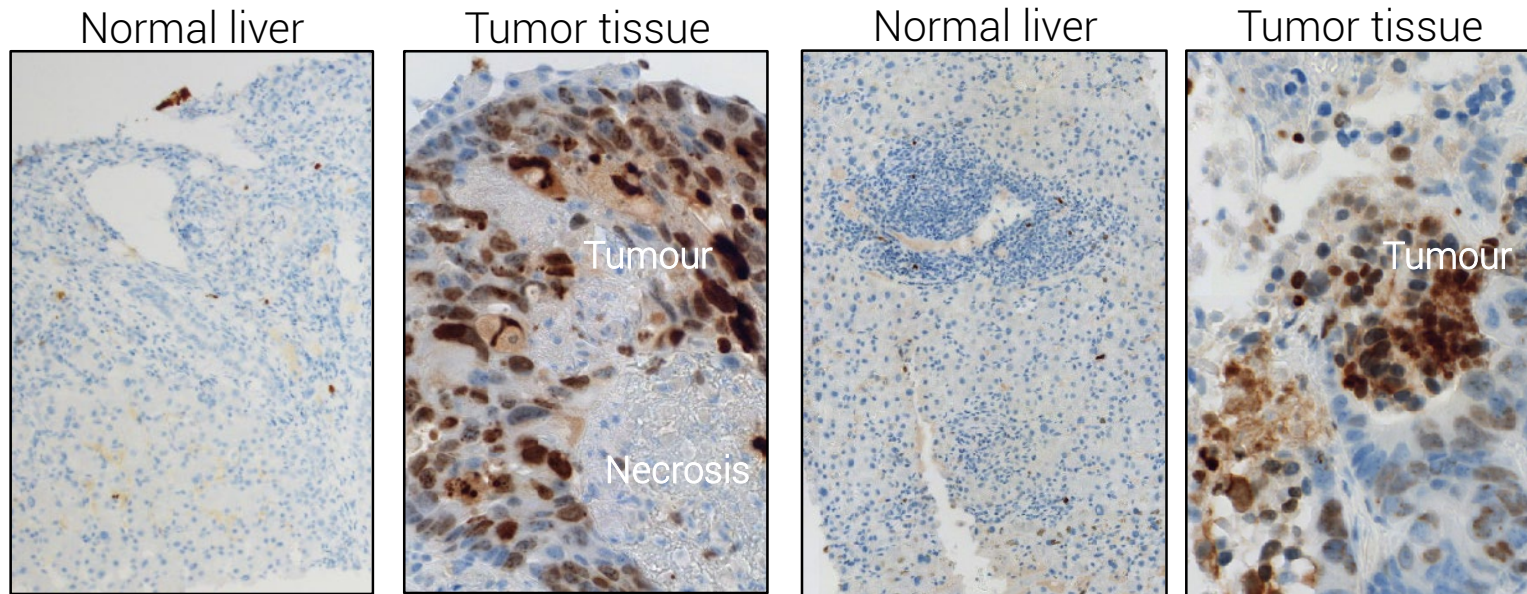


MIV-818 also shows favourable combination effect in preclinical models in vitro:

- With check-point (anti-PD1) inhibition
- With multiple DNA damage repair inhibitors

# MIV-818: Selective effect signal in liver cancer in phase Ia

- Clear signs of effect, measured as DNA damage, observed in liver biopsies from tumor tissue in MIV-818 treated patients. Normal liver tissue does not appear to have been affected
- This tumor selective effect was observed at low levels of MIV-818 in plasma and is an early proof-of-concept of the intended liver-directed effect in patients
- DNA damage also observed in hypoxic liver cancer regions
- Signs of effect on the size of the liver tumors in several patients



Data from Patient 2

Data from Patient 4

*Clear evidence of pH2AX induction (brown nuclear stain) resulting from DNA damage in tumor but not in normal liver tissue*

# MIV-818 study design

## Phase Ia

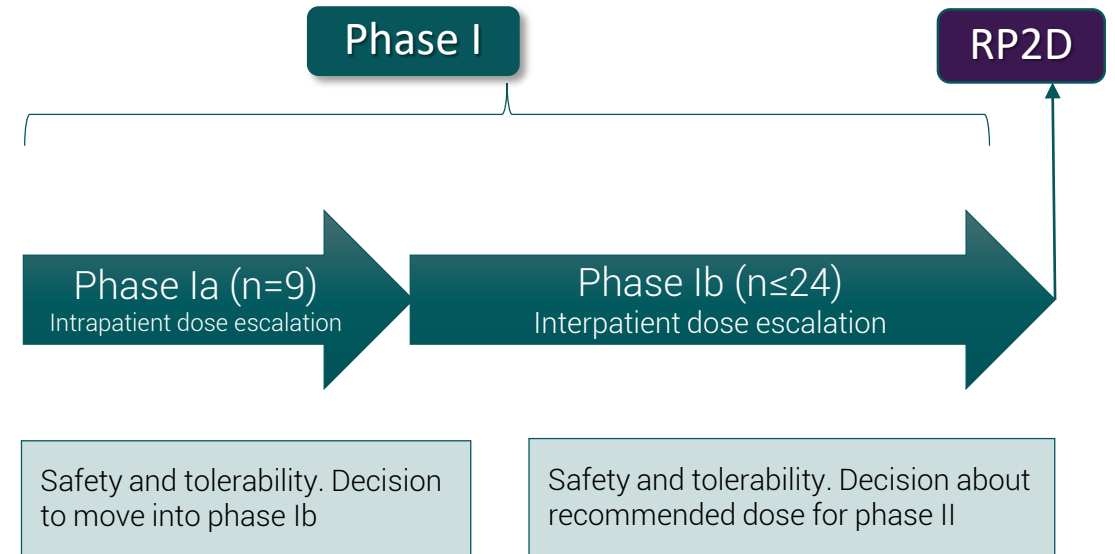
- Inpatient dose escalation
- Objective: Establish the start-dose for phase Ib
- A total of nine patients included
- Start-dose for phase Ib determined

## Phase Ib

- Classic 3+3 dose escalation
- Start-dose: 40mg 5 days/week
- Objective: Establish the phase II dose by evaluating the safety and tolerability of MIV-818

## Phase II

Planning ongoing for placebo-controlled add-on study to standard-of-care



# Potential role for MIV-818 in HCC treatment

Early stage disease currently treated with surgery, transplantation or chemo embolization

Drug therapy in advanced disease provides 2-3 months of extended life expectancy:

- 1<sup>st</sup> line therapy; sorafenib or lenvatinib
- 2<sup>nd</sup> line therapy; several immuno-oncology drugs

Role of MIV-818:

- May fill major unmet medical need by providing better efficacy in HCC therapy.
- Provides opportunity for monotherapy and/or add-on to standard-of-care (both 1<sup>st</sup> and 2<sup>nd</sup> line therapy) in patients with advanced stage HCC.

# MIV-828 for acute myeloid leukemia

## Profile of MIV-828

- Nucleotide prodrug given intravenously
- Active metabolite shown to have potent anti-leukemic activity in preclinical in vivo AML models
- Increased activity and reduced susceptibility to pharmacologic resistance mechanisms of prodrugs
- Preclinical activity also demonstrated against T-cell lymphoma

## Opportunity in hematological cancers

- Better tolerated and more effective agent in patients with relapsed/refractory AML and other hematological cancers
- Overcomes multiple resistance mechanisms and shows synergy with most approved AML therapeutics
- Shows efficacy in targeting AML cancer stem cells

*Acute myeloid leukemia (AML) leads to an overproduction of abnormal immature cells in the bone marrow and prevents development of normal blood cells, which could lead to anemia, bleeding episodes and a high susceptibility for severe infections.*

- Expected new cases 2018: US: ~ 19,500; EU: ~ 43,000; Sweden: ~ 350
- Affects all ages, median age at diagnosis 68 years in the US
- Five-year survival: 50% in younger patients (<60y) and less than 10% in patients > 70y

# Assets for partnering

# Three advanced clinical-stage assets for partnering

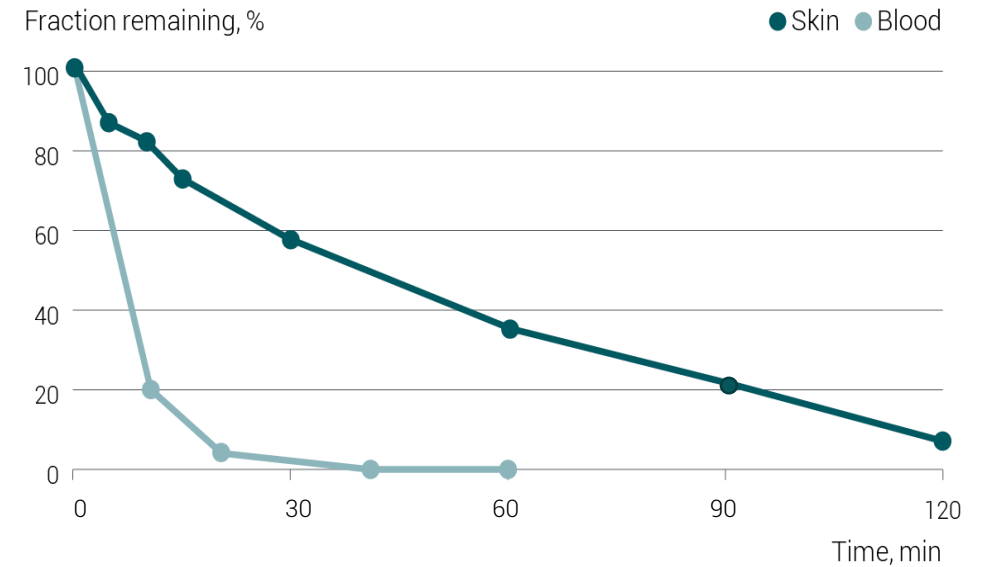
- The phase III ready remetinostat – a topical HDAC inhibitor for cutaneous T-cell lymphoma (MF-CTCL) and potentially basal cell carcinoma (BCC) and squamous cell carcinoma (SCC).
- The bivalent SMAC mimetic birinapant – currently in one combination study: head and neck cancer (HNC) with radiation.
- The cathepsin K inhibitor MIV-711 for osteoarthritis (OA) - has the potential to be the first disease modifying OA medicine.

Nucleotide prodrug	Indication	Phase I	Phase II	Phase III	Exclusivity
Remetinostat	MF-CTCL				IP : 2034
	BCC				
	SCC				
Birinapant	HNC				IP : 2034
MIV-711	Osteoarthritis				IP : 2034



# Remetinostat for MF-CTCL

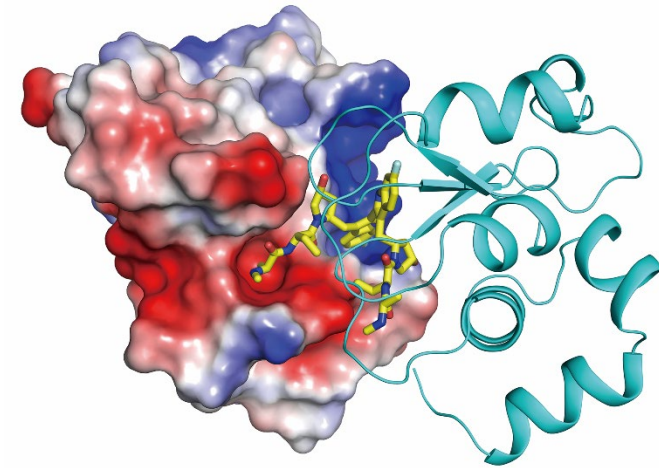
- Formulated as gel for topical administration
- Strong phase II efficacy and safety data
- US-orphan drug designation for MF-CTCL
- EOP2 discussions with FDA clarified that:
  - One placebo-controlled phase III study sufficient for approval
  - Co-primary endpoint required to define lesion effect
  - Pruritus as key secondary endpoint
- Interim analysis of ongoing phase II BCC study reported (at SID 2019) to proceed very well
- A phase II study in SCC initiated in December 2019



Remetinostat is much more stable in skin compared to blood.

# Birinapant for solid tumors

- Birinapant enables tumor cell death and augments the immune system. Has great potential to improve cancer therapy in combination with other treatments
- Ongoing phase I study in head and neck cancer in combination with radiation
- Phase II combination study with Merck's Keytruda® in MSS colorectal cancer was discontinued in December 2019 because of futility



Birinapant antagonises cIAP-1 and cIAP-2

# MIV-711 for osteoarthritis (OA), the most common form of joint disease

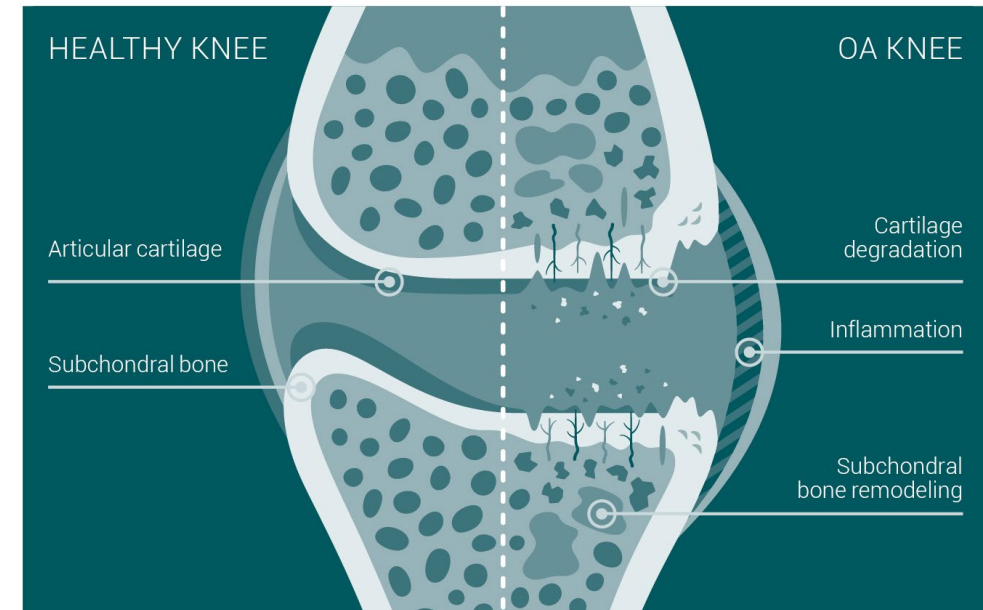
Successful placebo controlled phase II study of MIV-711 in OA:

- MIV-711 showed significant effects on joint structure (bone and cartilage) after 26 weeks.
- Trends favoured MIV-711 over placebo on knee pain and function.
- Safety and tolerability profile supportive of further development.

OA affects around 240 million worldwide

No disease-modifying medicine approved for OA

The FDA open to consider data on structural endpoints – correlation with pain will be required



# Corporate information and milestones

# Corporate information

- Founded in 1988
- Listed on Nasdaq Stockholm
- Market cap around 300 million SEK, cash position at Q3-19: 159 million SEK
- Has developed two drugs from idea to market: Xerclear and Olysio
- Has established over 20 partnerships that generated over 400 million USD
- Current focus: oncology drug development and business development
- Highly competent and effective organization; 13 FTEs

# Experienced leadership



Uli Hacksell, PhD; CEO  
*Astra, ACADIA, Cerecor*



Christina Herder, PhD; COO  
*Pharmacia, Biovitrum*



Magnus Christensen, MBA; CFO  
*O'Learys Trademark, ICA Sverige, HKScan*



Linda Basse, MD; PhD; CMO  
*Abbott, Topo Target, Genmab, Zealand*



Rikard Höse, MD; Med Dir.  
*Karolinska University Hospital, Novartis*



Fredrik Öberg, PhD; CSO  
*Uppsala University*



Linda Palmér, Sr Dir Clin Ops  
*Pfizer*

# Achieved milestones 2019

MIV-818: POC in phase Ia

Q2 2019 ✓

New organization in place

Q3 2019 ✓

Birinapant Head & Neck cancer phase I study started

Q4 2019 ✓

Birinapant/Keytruda<sup>®</sup>: phase II futility analysis

Q4 2019 ✓