



# CORPORATE PRESENTATION

## JULY 2019

**MEDIVIR**  
SCIENCE WORKING WONDERS

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# About Medivir

- Founded in 1988
- Listed on Nasdaq Stockholm
- Market cap around 600 million SEK, cash position at Q1-19: 228 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; 14 FTEs

# Medivir – Oncology focused biotech with major upside

## CLINICAL DEVELOPMENT

- Birinapant/Keytruda® combination: Ongoing phase II MSS CRC
  - Interim data in Q4-19
- MIV-818: Ongoing phase I study in liver cancer
  - Early proof of concept in phase Ia (Q2-19)
  - Phase Ib to start in Q4-19
- Remetinostat: Ongoing phase II ISS study for BCC
  - Positive interim data in Q2-19

## BUSINESS DEVELOPMENT

- Out-licensing of phase III-ready MIV-711 for OA
- Partnering of phase III-ready remetinostat for CTCL
- Finding new homes for our preclinical research programs

# Broad and robust pipeline

PROJECT & MECHANISM	DISEASE AREA	RESEARCH	PRECLINICAL	PHASE I	PHASE II	PHASE III	EXCLUSIVITY	
<b>Remetinostat</b> HDAC INHIBITOR (TOPICAL)	Cutaneous T-cell lymphoma (MF)	Completed					IP: 2034	
	Basal cell carcinoma <sup>1)</sup>	Completed		Ongoing			IP: 2034	
<b>Birinapant</b> SMAC MIMETIC (INTRAVENOUS)	Solid tumors (combo with Keytruda®)	Completed			Ongoing		IP: 2034	
<b>MIV-818</b> NUCLEOTIDE DNA POLYMERASE INHIBITOR (ORAL)	Hepatocellular carcinoma	Completed		Ongoing			IP: 2035	
<b>MIV-828</b> NUCLEOTIDE BASED DNA POLYMERASE INHIBITOR (INTRAVENOUS)	Blood cancer (acute myeloid leukemia)	Completed	Ongoing					IP: Est 2039
<b>MIV-711</b> CATHEPSIN K INHIBITOR (ORAL)	Osteoarthritis	Completed					IP: 2034	

<sup>1)</sup> Investigator sponsored study at Stanford U.

Completed
  Ongoing



# Remetinostat for early-stage MF cutaneous T-cell lymphoma

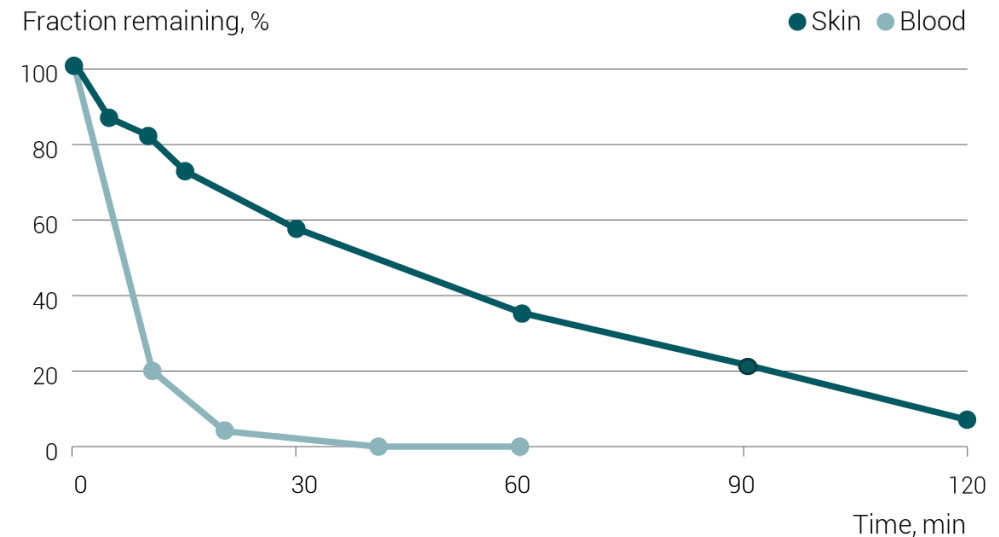
# MF-CTCL: orphan blood cancer indication

Cutaneous T-cell lymphoma (CTCL) affects lymphocytes (cells belonging to the immune defense system) located in the skin and typically has a chronic course.

- CTCL is a rare form of non-Hodgkin lymphoma primarily present in the skin. Mycosis fungoides (MF) is the most common form of CTCL
- Annual new cases; US ~ 2,000; EU ~ 3,000; Sweden ~ 25
- Five-year survival: ~ 85%; more than 16,000 US patients live with MF-CTCL
- Skin lesions and severe itching are common and affect patients quality of life
- Early stage disease lasts for long periods and requires well tolerated therapy
- Available treatments, such as Vorinostat and other systemic HDAC inhibitors, bexarotene, and Valchlor, have severe side effects

# Remetinostat: for treatment of early stage MF-CTCL

- Remetinostat is a histone deacetylase (HDAC) inhibitor
- Remetinostat's unique chemistry and topical formulation provides for activity in skin and rapid degradation in blood
- Approved HDAC inhibitors not used in early-stage MF-CTCL patients
- US orphan drug designation





# Remetinostat: clinical proof-of-concept phase II MF-CTCL study

Twelve months phase II data shows reduction in both lesions and severe itch

Dose	1% 1x/day n=20	0.5% 2x/day n=20	1% 2x/day n=20
Lesion responses <sup>1</sup>	20%	25%	40%
Patients with clinically significant pruritus	(40%) n=8/20	(30%) n=6/20	(50%) n=10/20
Pruritus responses	38%	50%	80%

Well tolerated:

- No HDAC inhibitor-associated systemic adverse events
- Median time on treatment: 336 days (1% 2x/day dose)

1) Confirmed responses based on CAILS, the Composite Assessment of Index Lesion Severity

# Remetinostat: next steps

- Medivir has defined a phase III design based on the requirements clarified by the FDA
- One phase III study expected to be sufficient for FDA
- Phase III study will enroll treatment-experienced patients
- Medivir seeks to identify a business partner for the further development of remetinostat

# Remetinostat for basal cell carcinoma

# Remetinostat: interim phase II BCC data presented at SID 2019\*

## Basal cell carcinoma

- The most common form of cancer in humans occurring in the skin
- Surgery is standard of care, but there is a need for efficacious and safe treatments when surgery is impractical, e.g. multiple lesions and/or difficult treatment sites

## Interim phase II data

- Fifteen patients recruited in open-label study
- Treatment: retinostat gel 1% (with occlusion) 3 times/day for six weeks
- ORR ( $\geq 30\%$  in longest diameter): 64%
- 43% of tumors fully cleared
- No systemic toxicities
- Grade 2 reversible eczematous reaction in 71% of patients

\* Urman et al., An open label phase 2 clinical trial of topical retinostat for basal cell carcinoma





# Birinapant: Uniquely potent against selected solid tumors



# Solid tumors: large unmet medical needs

Many patients with solid tumors have few or no options and are in need of effective medicines to extend life. The immuno-oncology medicine Keytruda<sup>®</sup> on its own is not sufficiently effective in treatment of certain solid tumors.

## Colorectal cancer indication (CRC)

- The second most common cancer in women and the third in men
- Estimated new cases 2018: US: ~ 140,000; EU: ~ 490,000; Sweden: ~ 6,200
- Five-year survival when metastatic: 14%


## Other cancer indications

- Ovarian cancer, the leading cause of mortality due a gynecologic tumor
  - Estimated new cases 2018: US: ~ 22,000; EU: ~ 23,000 Sweden: ~ 700
  - Five-year survival: 47%
- Cervical cancer, the third most common cancer in women world-wide
  - Estimated new cases 2018: US: ~ 13,000; EU: ~ 60,000; Sweden: ~ 450
  - Five-year survival: 62.5%

# Birinapant/Keytruda<sup>®</sup> studied in cancer patients with solid tumors

- Birinapant, a bivalent SMAC mimetic, enables tumor cell death and augments the immune system
- Great potential to improve treatment of cancers when combined with immuno-therapy
- Ongoing collaboration with Merck for a phase I/II combination study of birinapant/Keytruda<sup>®</sup> in solid tumors
  - Joint development committee oversees the study
  - Keytruda<sup>®</sup> provided at no cost by Merck
  - Medivir retains full global rights to birinapant and data
- Phase I
  - 19 patients with solid tumors were evaluated. Highest dose tested recommended for phase II
  - 1 MSS colorectal cancer patient has partial response. Continued treatment after 80 weeks\*
- Phase II
  - 15 patients with MSS colorectal cancer have been recruited
  - Interim/futility analysis in Q4 2019

\* ASCO 2019 oral presentation: Schilder et al., Determination of the Recommended phase 2 dose of birinapant in combination with pembrolizumab: Results from the dose escalation phase of BPT-201.



# MIV-818: Nucleotide prodrug for the treatment of liver cancer

# Primary liver cancer : hepatocellular carcinoma and intrahepatic cholangiocarcinoma

- Hepatocellular carcinoma (HCC) is the third leading cause of cancer-related deaths worldwide
  - Estimated new cases 2018: Asia: ~ 610,000; US: ~ 42,000; EU: ~ 82,000; Sweden: ~ 550
  - Orphan disease in Western markets, but one of fastest growing and most deadly cancers in US
  - High incidence in Asia including China - Hepatitis B & C very common
  - Five-year survival: 18%
  - Genetically heterogeneous leading to limited effect of molecularly targeted therapies
- Intrahepatic cholangiocarcinoma is the second most common primary liver tumor
  - Median survival is only twelve months
- Existing treatment options provide very little survival benefit

# MIV-818: prodrug for enhanced efficacy and safety in liver cancer therapy

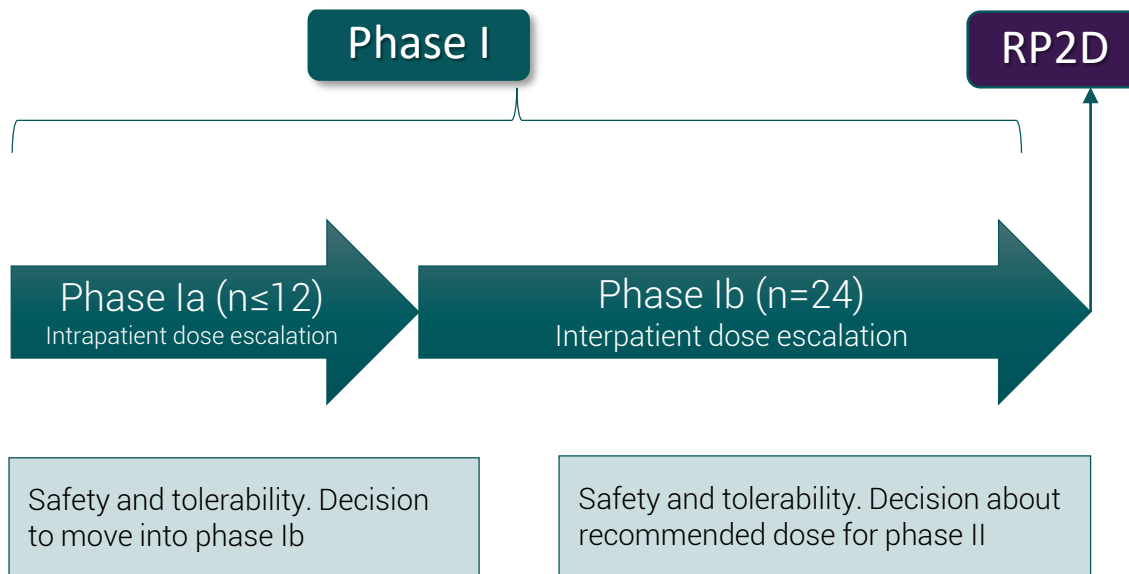
## Troxacitabine

- Clinically active but failed due to systemic dose-limiting toxicities



## MIV-818

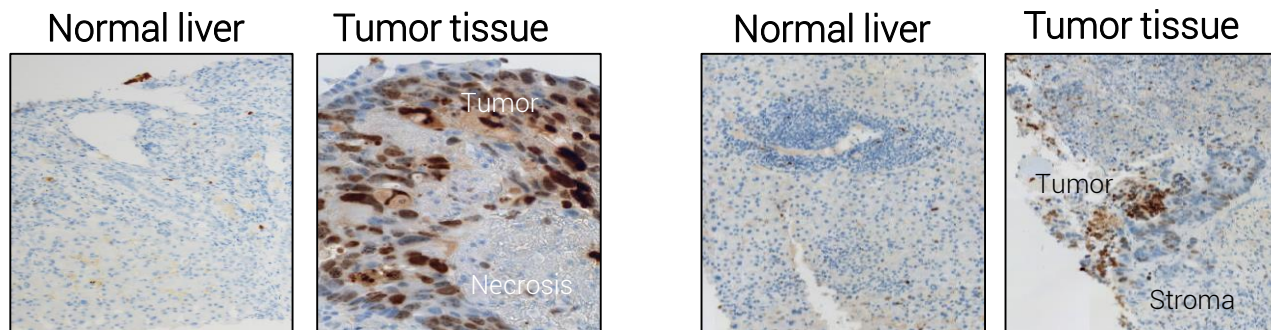
- Enhanced activity
- Selectivity for cancer
- Improved delivery to the liver
- Oral administration
- Limited systemic side effects





# MIV-818: Proof-of-concept phase Ia data

- Data from six patients with advanced liver cancer and treated with escalated doses were analyzed
- MIV-818 was well tolerated. Lowering of blood counts in patient six suggests that highest dose is close to starting dose for phase Ib
- Clear signs of effect, measured as DNA damage, in biopsies from liver cancer tissue. No DNA damage seen in normal liver tissue
- Tumor selective effect observed at low MIV-818 levels in plasma



Data from Patient 2

Data from Patient 4

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

# MIV-828: For acute myeloid leukemia

# MIV-828: Summary

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

## Opportunity in hematological cancers

- Better tolerated and more effective agent in patients with AML and other hematological cancers
- Initial development in relapsed/refractory AML patients

## Profile of MIV-828

- Nucleotide prodrug based on one of Medivir's proprietary areas of expertise
- Active metabolite shown to have potent anti-leukemic activity in preclinical in vivo models
- Increased activity and reduced susceptibility to pharmacologic resistance mechanisms of prodrugs
- Preclinical activity also demonstrated against T-cell lymphoma



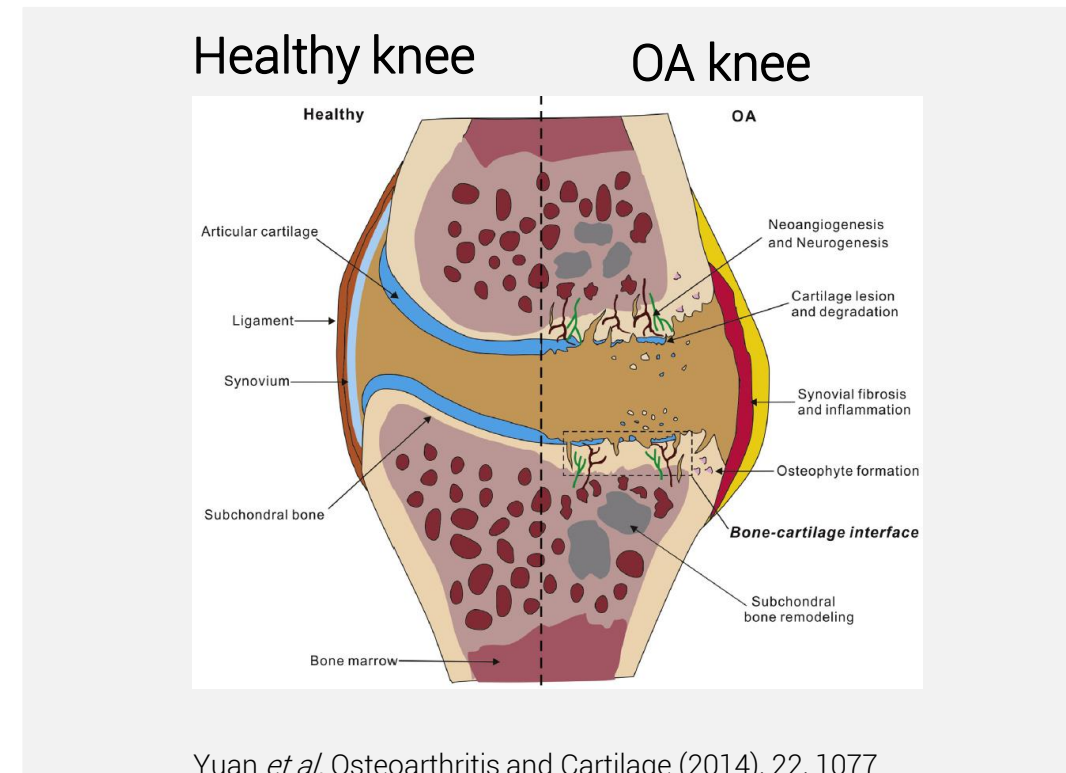


# MIV-711: Cathepsin K inhibitor with FDA fast track status

# Osteoarthritis (OA): the most common form of joint disease

- Affects ~240m adults worldwide
- Prevalence increasing due to aging population and obesity epidemic
- Current treatments focus only on pain relief
- Large unmet medical need for a disease-modifying drug (DMOAD) with potential to slow, halt or reverse the progression of OA
- New US FDA guidelines in OA may enable pathway for accelerated regulatory approval
- New draft FDA Guidance published August 2018, focused on structural endpoints in OA development

Cathepsin K protease is involved in the breakdown of collagen I in both bone and collagen II in cartilage





# MIV-711: positive effects on joint structure and signals of benefit on clinical symptoms

- Study design (MIV-711-201): 3 arms, 26 weeks treatment
- Study design (MIV-711-202): 200 mg MIV-711, 26 additional weeks

## MIV-711-201: Change from baseline vs week 26

	Placebo n=80	MIV-711 100 mg QD n=80	MIV-711 200 mg QD n=80
Femur bone area (mm <sup>2</sup> )	23.2	8.1	8.2
Cartilage thickness (mm)	-0.066	0.008	-0.017

- A trend consistently favoring MIV-711 arms in all predefined analyses of clinical outcomes, e.g. knee pain and knee function
- Safety and tolerability profile supporting advancement of MIV-711 as a disease-modifying OA drug candidate

# Milestones

# Medivir - recent and upcoming milestones

MIV-828: CD nomination	Q4 2018
Birinapant/Keytruda <sup>®</sup> : completion of phase I	Q4 2018
MIV-818: Start of phase Ia	Q4 2018
Remetinostat: EoP2 meeting with FDA	Q4 2018
Birinapant/Keytruda <sup>®</sup> : start of phase II	Q4 2018
MIV-818: POC in phase Ia	Q2 2019
New organization in place	Q3 2019
Birinapant/Keytruda <sup>®</sup> : phase II futility analysis	Q4 2019
MIV-818: Planned start of phase Ib	Q4 2019